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Filed pursuant to Rule 424(b)(4)
Registration No. 333-284352

10,588,233 Shares

Common Stock

This is the initial public offering of shares of common stock of Sionna Therapeutics, Inc. We are offering 10,588,233 shares of our common stock.

Prior to this offering, there has been no public market for our common stock. The initial public offering price per share is $18.00. Our common stock has been approved for listing on The Nasdaq Global Market ("Nasdaq") under the symbol "SION."

We are an "emerging growth company" and "smaller reporting company" as defined under the United States ("U.S.") federal securities laws and, as such, we have elected to comply with certain reduced reporting requirements.

See the section titled "Risk Factors" beginning on page 17 to read about factors that you should consider before deciding to invest in shares of our common stock.

	Per Share	Total
Initial public offering price	$ 18.00	$190,588,194
Underwriting discounts and commissions(1)	$ 1.26	$ 13,341,174
Proceeds, before expenses, to us	$ 16.74	$177,247,020

(1) See the section titled "*Underwriting*" for additional information regarding compensation payable to the underwriters.

We have granted the underwriters the option to purchase up to an additional 1,588,234 shares of common stock from us, at the initial public offering price, less the underwriting discounts and commissions.

The underwriters expect to deliver the shares of common stock on or about February 10, 2025.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

Goldman Sachs & Co. LLC **TD Cowen** **Stifel** **Guggenheim Securities**

February 6, 2025

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Neither we nor the underwriters have authorized anyone to provide you any information or make any representations other than those contained in this prospectus or in any free writing prospectuses prepared by or on behalf of us or to which we have referred you. We and the underwriters take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. We and the underwriters are not making an offer to sell these securities in any jurisdiction where the offer or sale is not permitted. You should assume that the information appearing in this prospectus or in any applicable free writing prospectus is current only as of its date, regardless of its time of delivery or any sale of shares of our common stock. Our business, financial condition, results of operations and prospects may have changed since that date.

For investors outside of the U.S.: we have not, and the underwriters have not, done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than the U.S. Persons outside of the U.S. who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of our common stock and the distribution of this prospectus outside of the U.S.

All trademarks, trade names and service marks appearing in this prospectus are the property of their respective owners. Solely for convenience, the trademarks and trade names in this prospectus may be referred to without the ® and ™ symbols, but such references should not be construed as any indicator that their respective owners will not assert their rights thereto.

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Market, Industry and Other Data

The market data and certain other statistical information used throughout this prospectus are based on independent industry publications, governmental publications, reports by market research firms or other independent sources that we believe to be reliable sources. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. We are responsible for all of the disclosure contained in this prospectus, and we believe that these sources are reliable; however, we have not independently verified the information contained in such publications. While we are not aware of any misstatements regarding any third-party information presented in this prospectus, their estimates, in particular, as they relate to projections, involve numerous assumptions, are subject to risks and uncertainties, and are subject to change based on various factors, including those discussed under the section titled "*Risk Factors*" and elsewhere in this prospectus. Some data are also based on our good faith estimates.

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PROSPECTUS SUMMARY

This summary highlights selected information contained elsewhere in this prospectus. This summary does not contain all of the information you should consider before investing in our common stock. You should read this entire prospectus carefully, including the sections of this prospectus titled "Risk Factors," "Cautionary Note Regarding Forward-Looking Statements," "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our audited consolidated financial statements and the related notes included elsewhere in this prospectus, before making an investment decision. Unless otherwise indicated, all references in this prospectus to "Sionna," the "company," "we," "our," "us" or similar terms refer to Sionna Therapeutics, Inc. and its wholly owned subsidiary, or either or both of them as the context may require.

Overview

We are a clinical-stage biopharmaceutical company on a mission to revolutionize the current treatment paradigm for cystic fibrosis ("CF") patients by developing novel medicines that normalize the function of the cystic fibrosis transmembrane conductance regulator ("CFTR") protein to deliver clinically meaningful benefit to CF patients. Our goal is to deliver differentiated medicines for people living with CF that can restore their CFTR function to as close to normal as possible by directly stabilizing CFTR's nucleotide-binding domain 1 ("NBD1"). Despite having long been identified as a critical component for proper CFTR function, NBD1 has been considered "undruggable," and none of the currently approved CF therapies directly stabilizes NBD1. Worldwide revenue for approved CFTR modulators was approximately $10 billion in 2023 and is expected to grow to $15 billion by 2029. Leveraging more than a decade of our co-founders' research on NBD1, we are advancing a pipeline of small molecules engineered to correct the defects caused by the F508del genetic mutation, which resides in the NBD1 domain. Approximately 90% of people with CF carry at least one copy of the F508del genetic mutation. We believe stabilizing NBD1 is central to unlocking dramatic improvements in clinical outcomes and quality of life for CF patients. We have employed biophysical, cell-based and virtual screening campaigns and extensive use of structural biology to guide the optimization of novel small molecule NBD1 stabilizers.

We are conducting ongoing Phase 1 trials of our two highly potent NBD1 stabilizers, SION-719 and SION-451, evaluating the safety, tolerability and pharmacokinetic ("PK") profile of single and multiple ascending doses in healthy subjects. These trials are randomized (3:1 active:placebo), doubled-blinded, placebo-controlled and are being conducted in Australia. As of January 14, 2025, five single ascending dose ("SAD") cohorts and three multiple ascending dose ("MAD") cohorts of SION-719 have been completed, with over 60 healthy subjects dosed, and six SAD cohorts and three MAD cohorts of SION-451 have been completed, with over 70 healthy subjects dosed. Both SION-719 and SION-451 have been generally well tolerated based on interim Phase 1 clinical data as of the data cutoff date of January 14, 2025. We have established target exposure levels for SION-719 and SION-451 to potentially provide clinically meaningful benefit, if administered as part of a dual combination or as an add-on to the current standard of care ("SOC"), based on our preclinical cystic fibrosis human bronchial epithelial ("CFHBE") model. In these trials, we have achieved the target concentrations for SION-719 and SION-451 with single and multiple doses. We plan to continue enrolling healthy subjects in additional MAD cohorts.

We are also developing a portfolio of complementary CFTR modulators designed to work synergistically with our NBD1 stabilizers to improve CFTR function, as seen in preclinical models. In July 2024, we in-licensed three clinical-stage compounds from AbbVie Global Enterprises Ltd. ("AbbVie") to expand our portfolio of combination product opportunities, including galicaftor (SION-2222), which targets CFTR's transmembrane domain 1 ("TMD1"), and has completed Phase 2 clinical

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trials. In addition, we recently completed a Phase 1 clinical trial evaluating SION-109, which targets CFTR's intracellular loop 4 ("ICL4") region. We plan to evaluate multiple NBD1 stabilizer candidates and complementary modulator candidates and select the most promising candidates to advance into later-stage development. Initially, we intend to evaluate the lead NBD1 stabilizer candidate in combination with the current standard of care, Trikafta, in a proof-of-concept trial. In parallel, we will determine the proprietary dual combination that we believe is optimal to advance into a later-stage clinical trial in CF patients, as illustrated in Figure 1.

Figure 1. Our NBD1 Stabilizers Have Multiple Potential Pathways to Deliver Clinically Meaningful Benefit to CF Patients

CF Background and Unmet Need

An estimated 106,000 people have been diagnosed with CF across 94 countries, including approximately 33,000 adults and children living with CF in the U.S., according to the Cystic Fibrosis Foundation ("CFF"). CF is caused by mutations to the CFTR gene that result in reduced or no function of the CFTR protein. Approximately 90% of people with CF carry at least one copy of the F508del mutation. The F508del mutation (a deletion of the amino acid phenylalanine at position 508 in NBD1) is considered a severe CF mutation, and individuals with this mutation tend to fall at the worst end of the CF severity spectrum because they have little or no CFTR function in epithelial cells.

The current standard of care for people with the F508del mutation is a triple combination product marketed by Vertex Pharmaceuticals, Inc. ("Vertex") as Trikafta (elexacaftor, tezacaftor, ivacaftor and ivacaftor). In addition, in December 2024, Vertex received approval from the U.S Food and Drug Administration (the "FDA") for a second-generation, triple modulator combination, Alyftrek, for the treatment of CF in patients aged six years and older who have at least one F508del mutation or another responsive mutation in the CFTR gene. Vertex also markets three other approved CFTR modulators. None of the approved modulators directly stabilize NBD1.

While advances in the treatment of patients with CF have improved the lives of patients, the median predicted survival age for individuals with CF born in the U.S. between 2019 and 2023 is still just 61 years, according to the 2023 CFF patient registry. We believe significant opportunity remains to provide clinically meaningful benefit to CF patients through the development of NBD1-anchored treatments. NBD1 has long been considered an important target to normalize CFTR function because it is the site where the F508del mutation—the most common mutation that causes CF—resides. At least two-thirds of patients on Trikafta do not have normal CFTR function, as measured by sweat chloride levels below 30 mmol/L. Even treated CF patients can continue to experience the ongoing effects of reduced CFTR function over time, including respiratory infections, pulmonary exacerbations, or "lung attacks," and continued lung function decline. More than 6,000 patients have discontinued use of approved CFTR modulators, none of which target NBD1. Such patients have limited or no alternative treatments available to improve their clinical outcomes or quality of life. Additionally, some patients on Trikafta reduce dosages due to tolerability issues. Our research with key opinion leaders has indicated the desire for more treatment options for CF patients, support for a new mechanism of

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action that could provide clinically meaningful benefit for people living with CF, and need for an alternative for those patients who experience tolerability issues on Trikafta. We aim to expand the current treatment paradigm through a proprietary dual combination or as an add-on to the standard of care.

Our Pipeline

Our proprietary portfolio includes NBD1 stabilizers and complementary modulators. There are two types of complementary modulators: correctors, which partially improve CFTR protein folding to aid its trafficking to the cell surface, and potentiators, which increase CFTR channel function by enabling chloride flow through the cell membrane. We believe the synergistic approach of combining NBD1 stabilizers with complementary modulators provides the highest probability of normalizing CFTR function for CF patients. Our portfolio includes:

- SION-719 and SION-451, our highly potent NBD1 stabilizers, are both in Phase 1 healthy volunteer trials in Australia to evaluate their PK profile, safety and tolerability. We have completed SAD dosing and three MAD dosing cohorts in each trial. Both NBD1 stabilizers have been generally well tolerated in these ongoing trials based on interim data to date and we achieved our target concentrations for SION-719 and SION-451 with single and multiple doses. Topline results from our SION-719 and SION-451 Phase 1 clinical trials are expected in the first half of 2025.
- Galicaftor (SION-2222) and SION-2851 are TMD1-directed CFTR correctors. Galicaftor was generally well-tolerated in Phase 1 and Phase 2 trials, and improvement in sweat chloride as a monotherapy and improvements in sweat chloride and lung function as a combination therapy with navocaftor were observed. SION-2851 has completed a Phase 1 SAD trial in healthy volunteers.
- SION-109, an ICL4-directed CFTR corrector, has been evaluated in a recently completed Phase 1 clinical trial in healthy volunteers. SION-109 was generally well tolerated at all dose levels administered in all parts of this Phase 1 trial. The target exposure for SION-109 as part of a dual combination with SION-451 or SION-719 was achieved with single and multiple doses.
- Navocaftor (SION-3067), a potentiator, has been evaluated in Phase 2 trials, in which it demonstrated potential as a combination therapy.

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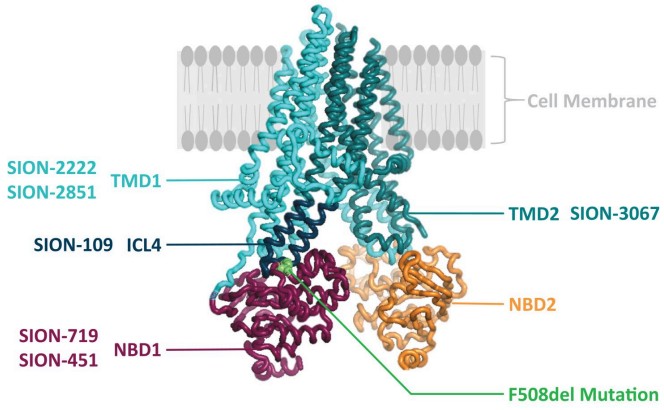
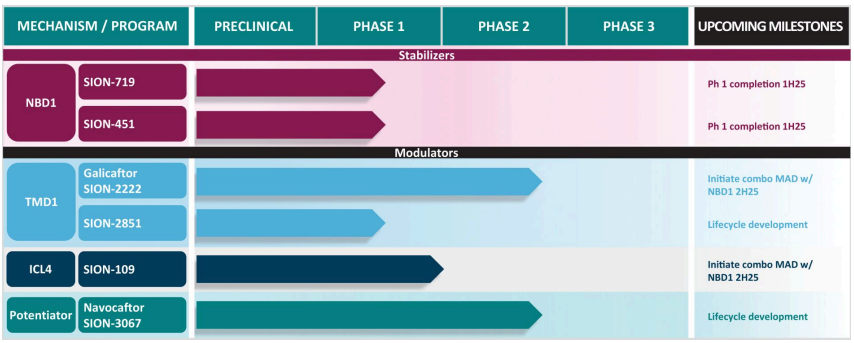
Figure 2 below captures the targeted binding locations within the CFTR structure for our pipeline of NBD1 stabilizers and complementary modulators.

Figure 2. Our Multi-Prong Approach to Potentially Improving CFTR Function



Our current portfolio of programs is summarized in Figure 3 below:

Figure 3. Our Proprietary Pipeline of Product Candidates for the Treatment of CF



Clinical trials for galicaftor, SION-2851, and navocaftor were conducted by AbbVie or Galapagos NV ("Galapagos"). MAD: Multiple Ascending Dose.

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We have not obtained regulatory approval for any product candidate, and it is possible that we may never obtain regulatory approval for any product candidates we may seek to develop in the future.

Our Approach and Leveraging the CFHBE Model

Central to our development strategy is our use of the industry standard, clinically predictive CFHBE model to measure CFTR protein function *in vitro*. Activity in this model has been shown to be correlated to chloride transport activity, which in turn, has been shown to be correlated to improved lung function in clinical trials designed to evaluate product candidates in CF patients. We have used, and plan to continue to use, insights from the CFHBE model to inform pipeline prioritization and development decisions. For example, we selected SION-719 and SION-451 to advance based on their preclinical profiles, including potency in the CFHBE model. We assessed SION-719 and SION-451 in our CFHBE model in direct, head-to-head comparison to elexacaftor/tezacaftor/ivacaftor ("ETI"), the components of Trikafta, which we synthesized using methods described in publicly available sources. When evaluated in our CFHBE model at their respective highest effective dose ("E_{max}") concentrations, both SION-719 and SION-451, in dual combination with one of our complementary modulators, improved *in vitro* CFTR protein activity to wild-type, or normal, levels. This was a more than 1.5-fold improvement in CFTR protein activity compared to the improvement in such activity observed with ETI at E_{max} in the same experiment.

Our Clinical Results and Next Steps

SION-719 and SION-451

We initiated Phase 1 SAD and MAD clinical trials of SION-719 and SION-451 in healthy subjects in July 2024 and August 2024, respectively. These trials are randomized, doubled-blinded, placebo-controlled trials designed to evaluate safety, tolerability and PK of each product candidate. Both trials are being conducted in Australia. In a Part C of each Phase 1 trial, we plan to evaluate the effect of food on the PK of each product candidate and the bioequivalence of a tablet formation compared to the oral suspension administered in the Phase 1 SAD and MAD trials. We intend to enroll up to 120 healthy volunteers in each trial. We expect topline data for these trials in the first half of 2025.

Interim Phase 1 Trial Data for SION-719

SION-719 was generally well tolerated at all dose levels administered based on interim Phase 1 clinical data as of the data cutoff date of January 14, 2025. There were no serious adverse events ("SAEs"). Most treatment-emergent adverse events ("TEAEs") were mild to moderate (Grade 1 or Grade 2). No TEAEs led to the discontinuation of trial drug. No dose-limiting TEAEs or safety trends of concern have been observed.

Increasing exposure was observed with increasing single and multiple doses. The concentration targets for SION-719 as an add-on to standard of care and as part of a dual combination with SION-2222 or SION-109 were achieved with single and multiple doses. A PK summary of SION-719 in the MAD portion of the trial is shown in Figure 4 below. The observed PK was consistent with twice daily dosing ("BID").

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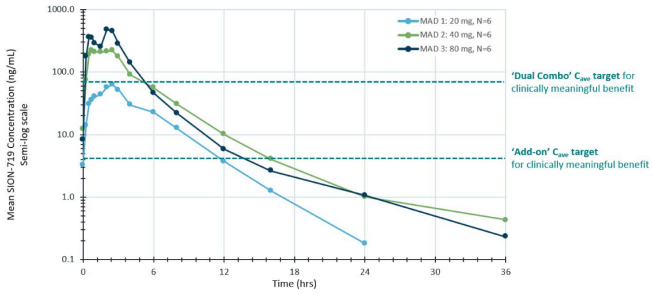
Figure 4. Preliminary Phase 1 PK Summary for SION-719 in the MAD Portion of the Trial



(Each solid line shows mean concentration data from a dosing cohort on Day 10. Dotted lines represent average PK concentration exposure targets that have the potential, based on our preclinical CFHBE model, to provide clinically meaningful benefit, if SION-719 is administered in a proprietary dual combination with either SION-2222 or SION-109, or as an add-on to SOC.)

Interim Phase 1 Trial Data for SION-451

SION-451 was generally well tolerated at all dose levels administered based on interim Phase 1 clinical data as of the data cutoff date of January 14, 2025. SION-451 was generally well tolerated at all dose levels administered. There were no SAEs, and most TEAEs were mild to moderate (Grade 1 or Grade 2). No TEAEs led to the discontinuation of trial drug. No dose limiting AEs or safety trends of concern have been observed.

Increasing exposure was observed with increasing single and multiple doses. The concentration targets for SION-451 as both an add-on to SOC and as part of a dual combination with SION-2222 or SION-109 were achieved with single and multiple doses. A PK summary of SION-451 in the MAD portion of the trial is shown in Figure 5 below. The observed PK was consistent with BID dosing.

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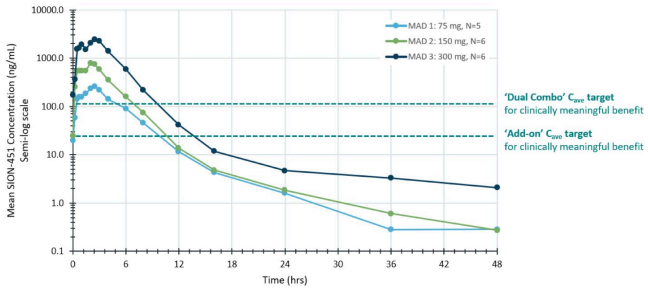
Figure 5. Preliminary Phase 1 PK Summary for SION-451 in the MAD Portion of the Trial



(Each solid line shows mean concentration data from a dosing cohort on Day 10. Dotted lines represent average PK concentration exposure targets that have the potential, based on our preclinical CFHBE model, to provide clinically meaningful benefit, if SION-451 is administered in a proprietary dual combination with either SION-2222 or SION-109, or as an add-on to SOC.)

Next Steps

Following completion of our ongoing Phase 1 clinical trials of SION-719 and SION-451, we plan to select a lead NBD1 stabilizer and conduct a drug-drug interaction trial before initiating a Phase 2a proof-of-concept trial in CF patients. We expect the Phase 2a trial to be a two-way crossover trial in which we enroll up to 20 trial subjects with CF who are stable on physician-prescribed Trikafta. We expect to select safety as the primary endpoint, and PK and improvements to sweat chloride levels as the secondary endpoints. We expect to initiate the Phase 2a clinical trial in the second half of 2025.

Galicaftor and SION-109

Galicaftor has completed Phase 1 and Phase 2 trials in approximately 400 subjects and was well-tolerated in CF subjects and healthy volunteers, with improvements in sweat chloride levels observed as a single agent and improvements in sweat chloride and lung function in combination with navocaftor (SION-3067), a clinical-stage potentiator of CFTR gating activity that we have licensed from AbbVie. We have also recently completed a Phase 1 clinical trial evaluating SION-109 to assess the safety, tolerability and PK of single and multiple ascending doses in healthy volunteers.

Our Strategy

Our mission is to revolutionize the current treatment paradigm for CF patients by developing novel medicines that normalize the function of the CFTR protein to deliver clinically meaningful benefit to CF patients. The key pillars of our strategy are:

- Advance our novel NBD1 stabilizers.
- Develop and advance our pipeline of complementary modulators for proprietary combination product development.
- Build upon our NBD1-centric CF franchise through a data-driven dual combination path.
- Fortify our CF franchise through continued research efforts and utilization of the translational CFHBE model.

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Our Company's History and Our Team

Sionna was founded in 2019 to continue to explore novel approaches to treating CF by targeting NBD1. Our co-founders, Greg Hurlbut, Ph.D., and Mark Munson, Ph.D., spent over a decade extensively researching the NBD1 target as research leaders at Sanofi SA (f/k/a Sanofi Genzyme) ("Sanofi"). We have assembled a leadership team with deep expertise in drug discovery and developing CF and other rare disease therapies, launching and commercializing therapeutics globally, and building successful public pharmaceutical companies.

- ***Mike Cloonan***, our President and Chief Executive Officer, has more than 20 years of leadership experience at global organizations, most recently as Chief Operating Officer at Sage Therapeutics, Inc. and prior to that as Senior Vice President of U.S. Commercial at Biogen, Inc.
- ***Charlotte McKee, M.D.***, our Chief Medical Officer, is a pulmonologist with more than 20 years of drug development experience who, while serving as Vice President of CF and Alpha-1 Antitrypsin Deficiency Clinical Development at Vertex, was instrumental in the development and regulatory approvals of three of Vertex's five approved CFTR modulators, including Trikafta.
- ***Elena Ridloff, C.F.A.***, our Chief Financial Officer and Head of Corporate Development, has more than 20 years of experience in finance in the life sciences industry, most recently as Chief Financial Officer at Acadia Pharmaceuticals Inc.

Since our inception, we have raised approximately $330 million from investors including RA Capital, TPG's the Rise Fund, Atlas Venture, OrbiMed and Enavate Sciences. We also received founding support from the CFF, which has been a committed investor and supporter of our research and development work. Prior to our inception, the CFF spent more than a decade funding early-stage F508del corrector discovery work at Sanofi that contributed to our pipeline. Prospective investors should not rely on the past investment decisions of our investors, as our investors may have different risk tolerances and have received their shares in prior offerings at prices lower than the price offered to the public in this offering. Please see the section titled "*Certain Relationships and Related Person Transactions*" included elsewhere in this prospectus for a description of the financings we have conducted to date.

Preliminary Balance of Cash and Cash Equivalents

We estimate that we had cash, cash equivalents and marketable securities of approximately $168.0 million as of December 31, 2024.

Our actual consolidated financial results as of and for the year ended December 31, 2024 are not yet available. Our financial closing procedures for the year ended December 31, 2024 are not yet complete and, as a result, our final results upon completion of those procedures may differ materially from this preliminary estimate. The preliminary consolidated financial data presented above as of December 31, 2024 is not a comprehensive statement of our financial position or operating results; reflects our preliminary estimate based on information available as of the date of this prospectus; and is subject to change, and those changes may be material. Accordingly, you should not place undue reliance upon this preliminary estimate. This estimate should not be viewed as a substitute for full financial statements prepared in accordance with generally accepted accounting principles in the U.S. and these estimates are not necessarily indicative of the results to be achieved for the stated period, or any other period. We do not undertake any obligation to publicly update or revise this preliminary estimate, except as required by law. See "*Risk Factors*" and "*Management's Discussion and Analysis of Financial Condition and Results of Operations*" in this prospectus for a discussion of

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certain factors that could result in differences between this preliminary unaudited estimate and the actual results.

The preliminary consolidated financial data presented above has been prepared by, and is the responsibility of, our management. Our independent registered public accounting firm, Deloitte & Touche LLP, has not audited, reviewed, compiled or performed any procedures, and does not express an opinion or any other form of assurance, with respect to any of such data. This information should be read in conjunction with our consolidated financial statements and the related notes and "*Management's Discussion and Analysis of Financial Condition and Results of Operations*" for prior periods in this prospectus.

Summary of Material Risks Associated With Our Business

Our business is subject to a number of risks of which you should be aware before making an investment decision. These risks include, but are not limited to, the following:

- We are a clinical-stage biopharmaceutical company and have incurred significant operating losses since inception and anticipate that we will continue to incur significant operating losses for the foreseeable future. Our net losses were $47.3 million and $40.2 million for the years ended December 31, 2023 and 2022, respectively. We had an accumulated deficit of $165.2 million and $119.4 million as of September 30, 2024 and December 31, 2023, respectively. We may never achieve or maintain profitability.
- Even if this offering is successful, we will need substantial additional funding. We may be unable to raise capital on acceptable terms, if at all, and, as a result, we may be required to delay, reduce or eliminate our product development programs or commercialization efforts.
- We are substantially dependent on the success of our NBD1 stabilizers. If we are unable to advance a lead NBD1 stabilizer product candidate into later-stage clinical development or unable to obtain regulatory approval and commercialize an NBD1 stabilizer-anchored therapy for the treatment of CF, or experience significant delays in doing so, our business will be materially harmed.
- We intend to develop our lead NBD1 stabilizer product candidate to be administered in combination with one of our complementary modulators or as an add-on to the current standard of care. Developing combination treatments increases complexity and risk, including risks of drug-drug interactions, unforeseen side effects or failures in our clinical trials that could delay or prevent their regulatory approval or limit the commercial profile of an approved label.
- The regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time-consuming and inherently unpredictable. If we are not able to obtain the required regulatory approval for any product candidate, our business will be substantially harmed.
- We have not yet completed all testing of any product candidate in clinical trials. Preclinical, interim, topline and preliminary results from our preclinical studies or clinical trials are not necessarily predictive of the results or analyses of such results of later clinical trials. If we cannot replicate the positive results from any preclinical studies or clinical trials of our current or potential future product candidates that have positive results, or if we suffer any other significant setbacks in our later clinical trials, we may be unable to successfully develop, obtain regulatory approval for and commercialize our current or potential future product candidates.
- Targeting the NBD1 domain of the CFTR protein is novel, and we do not know whether we will be able to successfully develop any products.
- Our preclinical studies and clinical trials may fail to demonstrate the safety and efficacy of our product candidates, or serious or unacceptable adverse side effects or unexpected toxicology

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findings may be identified during the development of our product candidates, which could prevent or delay further clinical development, regulatory approvals and commercialization, impact the product's labeling, if approved, increase our costs or necessitate the abandonment or limitation of the development of some of our product candidates.

- We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.
- We are dependent on licensed intellectual property. If we were to lose our rights to licensed intellectual property, we may not be able to continue developing or commercializing our product candidates, if approved. If we breach any of the agreements under which we license the use, development and commercialization rights to our product candidates from third parties or, in certain cases, we fail to meet certain development deadlines, we could lose license rights that are important to our business.
- We contract with third parties for the manufacture of our product candidates for clinical drug supply and expect to continue to do so for commercialization, if our product candidates are approved. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts.
- If we or our licensors are unable to obtain, maintain and enforce intellectual property rights relating to any of our product candidates, or if the scope of the protection obtained is not sufficiently broad, our competitors or other third parties could develop and commercialize products similar or identical to ours, our ability to successfully commercialize our product candidates may be adversely affected and we may not be able to compete effectively in our markets.
- We face substantial competition. Our main competitor in the CF market holds substantial market share and has substantially greater resources than we do. We may not be able to compete successfully in this environment and, in particular, against a much larger competitor.
- There has been no prior public market for our common stock. An active trading market for our common stock may not develop or be sustained.
- The trading price of the shares of our common stock may be volatile, and purchasers of our common stock could lose all or part of their investment.

The summary risk factors described above should be read together with the text of the full risk factors in the section titled "*Risk Factors*" and the other information set forth in this prospectus, including our audited consolidated financial statements and the related notes, as well as in other documents that we file with the Securities and Exchange Commission (the "SEC"). The risks summarized above or described in full elsewhere in this prospectus are not the only risks that we face. Additional risks and uncertainties not presently known to us, or that we currently deem to be immaterial may also materially adversely affect our business, financial condition, results of operations and future growth prospects.

Corporate Information

We were incorporated under the laws of the State of Delaware in August 2019 under the name Sling Therapeutics, Inc., and changed our name to Sionna Therapeutics, Inc. in July 2021. Our principal executive offices are located at 21 Hickory Drive, Suite 500, Waltham, MA 02451, and our telephone number is (617) 819-2020. We have one subsidiary, Sionna Therapeutics Securities

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Corporation (f/k/a Sling Therapeutics Securities Corporation), formed in 2020 under the laws of the Commonwealth of Massachusetts. Our website address is *www.sionnatx.com*. The information contained in or accessible from our website is not incorporated into this prospectus, and you should not consider it part of this prospectus. We have included our website address in this prospectus solely as an inactive textual reference.

Implications of Being an Emerging Growth Company and a Smaller Reporting Company

We qualify as an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, as amended (the "JOBS Act"). As an emerging growth company, we may take advantage of specified reduced disclosure and other requirements that are otherwise applicable generally to public companies. These provisions include:

- being permitted to present only two years of audited consolidated financial statements, in addition to any required unaudited interim consolidated financial statements, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure in this prospectus;
- reduced disclosure about our executive compensation arrangements;
- not being required to hold advisory votes on executive compensation or to obtain stockholder approval of any golden parachute arrangements not previously approved;
- an exemption from the auditor attestation requirement in the assessment of our internal control over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act of 2002 ("Sarbanes-Oxley Act"); and
- an exemption from compliance with the requirements of the Public Company Accounting Oversight Board regarding the communication of critical audit matters in the auditor's report on the consolidated financial statements.

We may take advantage of these exemptions for up to five years or such earlier time that we are no longer an emerging growth company. We may choose to take advantage of some but not all of these exemptions. We would cease to be an emerging growth company on the date that is the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1.235 billion or more; (ii) the last day of our fiscal year following the fifth anniversary of the date of the completion of this offering; (iii) the date on which we have issued more than $1.0 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the SEC. We have taken advantage of reduced reporting requirements in this prospectus. Accordingly, the information contained herein may be different from the information you receive from other public companies in which you hold stock. Additionally, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have elected to avail ourselves of this exemption and, therefore, while we are an emerging growth company we will not be subject to new or revised accounting standards at the same time that they become applicable to other public companies that are not emerging growth companies. As a result of this election, our consolidated financial statements may not be comparable to those of other public companies that comply with new or revised accounting pronouncements as of public company effective dates. We may choose to early adopt any new or revised accounting standards whenever such early adoption is permitted for private companies.

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We are also a “smaller reporting company,” meaning that the market value of our shares held by non-affiliates plus the proposed aggregate amount of gross proceeds to us as a result of this offering is less than $700 million and our annual revenue was less than $100 million during the most recently completed fiscal year. We may continue to be a smaller reporting company after this offering if either (i) the market value of our shares held by non-affiliates is less than $250 million or (ii) our annual revenue was less than $100 million during the most recently completed fiscal year and the market value of our shares held by non-affiliates is less than $700 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, we may choose to present only the two most recent fiscal years of audited consolidated financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

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THE OFFERING	
Common stock offered by us	10,588,233 shares.
Option to purchase additional shares of common stock	We have granted a 30-day option to the underwriters to purchase up to 1,588,234 additional shares of common stock from us at the public offering price, less underwriting discounts and commissions.
Common stock to be outstanding immediately after this offering	42,520,700 shares (or 44,108,934 shares if the underwriters exercise their option to purchase additional shares of common stock in full).
Use of proceeds	We estimate that the net proceeds from the sale of our common stock in this offering will be approximately $173.0 million (or approximately $199.6 million if the underwriters exercise their option to purchase additional shares of common stock in full), based on the initial public offering price of $18.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us. We currently intend to use the net proceeds from this offering, together with our existing cash, cash equivalents and investments in marketable securities, (i) to complete our ongoing Phase 1 clinical trials, our planned Phase 2a proof of concept trial, and certain preclinical and other early clinical activities to enable potential advancement of our selected dual combination of an NBD1 stabilizer and complementary modulator; (ii) to initiate and progress a dual combination Phase 2b dose-ranging clinical trial of our selected NBD1 stabilizer and complementary modulator and to continue manufacturing scale-up to supply drug product for late stage clinical trials; (iii) on other research and development activities; and (iv) to use the remainder for working capital and other general corporate purposes. See the section titled "*Use of Proceeds*" for additional information.
Risk factors	See the section titled "*Risk Factors*" for a discussion of factors you should carefully consider before deciding whether to invest in our common stock.
Trading symbol on The Nasdaq Global Market	"SION"

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The number of shares of our common stock that will be outstanding after this offering is based on 31,932,467 shares of our common stock (which includes 119,639 shares of restricted common stock) outstanding as of September 30, 2024 after giving effect to the automatic conversion of all outstanding shares of our Series Seed convertible preferred stock, par value $0.001 per share (the "Series Seed preferred stock"), Series A convertible preferred stock, par value $0.001 per share (the "Series A preferred stock"), Series B convertible preferred stock, par value $0.001 per share (the "Series B preferred stock") and Series C convertible preferred stock, par value $0.001 per share (the "Series C preferred stock" and, together with the Series Seed preferred stock, Series A preferred stock and Series B preferred stock, the "preferred stock") into an aggregate of 27,149,206 shares of our common stock immediately prior to the completion of this offering, and excludes:

- 3,652,051 shares of common stock issuable upon exercise of outstanding stock options as of September 30, 2024 under our 2020 Stock Option and Grant Plan, as amended ("2020 Plan"), with a weighted average exercise price of $5.77 per share;
- 63,745 shares of common stock issuable upon exercise of outstanding stock options granted after September 30, 2024 pursuant to our 2020 Plan, with a weighted average exercise price of $10.33 per share;
- 1,099,636 shares of common stock reserved for future issuance as of September 30, 2024 under the 2020 Plan, which ceased to be available for issuance at the time that our 2025 Stock Option and Incentive Plan ("2025 Plan") became effective;
- 390,127 shares of common stock reserved for future issuance under our 2025 Employee Stock Purchase Plan ("ESPP"), which became effective on the date immediately prior to the effectiveness of the registration statement of which this prospectus forms a part, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the ESPP; and
- 5,060,000 shares of our common stock that will become available for future issuance under our 2025 Plan, which became effective on the date immediately prior to the effectiveness of the registration statement of which this prospectus forms a part, which includes an aggregate of 1,946,388 shares of our common stock, which were granted to certain of our executive officers, directors and employees at the time of effectiveness of the 2025 Plan with an exercise price equal to $18.00 per share, the initial public offering price per share, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the 2025 Plan and any shares underlying outstanding stock awards granted under the 2020 Plan that expire or are repurchased, forfeited, cancelled or withheld.

Unless otherwise indicated, the information in this prospectus reflects or assumes the following:

- a 1-for-1.4611 reverse stock split of our common stock, effected on January 31, 2025;
- the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 27,149,206 shares of common stock immediately prior to the completion of this offering;
- no exercise of the outstanding stock options described above after September 30, 2024;
- no exercise of the underwriters' option to purchase up to an additional 1,588,234 shares of common stock in this offering; and
- the filing and effectiveness of our fifth amended and restated certificate of incorporation immediately prior to the completion of this offering and the effectiveness of our amended and restated bylaws upon the effectiveness of the registration statement of which this prospectus forms a part.

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SUMMARY CONSOLIDATED FINANCIAL DATA

The following tables set forth our summary consolidated statements of operations for the years ended December 31, 2023 and 2022. The summary consolidated statements of operations and comprehensive loss data for the years ended December 31, 2023 and 2022 have been derived from our audited consolidated financial statements included elsewhere in this prospectus. The summary interim condensed statements of operations data for the nine months ended September 30, 2024 and 2023, and the summary interim condensed balance sheet data as of September 30, 2024, have been derived from our unaudited interim condensed financial statements included elsewhere in this prospectus. Our audited consolidated financial statements and unaudited interim condensed financial statements included elsewhere in this prospectus have been prepared in accordance with U.S. generally accepted accounting principles ("GAAP"). Our unaudited interim condensed financial statements were prepared on a basis consistent with our audited financial statements and include, in our opinion, all adjustments of a normal and recurring nature that are necessary for the fair statement of the financial information set forth in those statements included elsewhere in this prospectus. Our historical results are not necessarily indicative of the results that may be expected for any period in the future. You should read the following summary financial data together with "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our audited consolidated financial statements and unaudited interim condensed financial statements and the related notes included elsewhere in this prospectus. The summary consolidated financial data included in this section are not intended to replace the audited consolidated financial statements and are qualified in their entirety by our audited consolidated financial statements and the related notes included elsewhere in this prospectus.

	Nine Months Ended September 30,		Year Ended December 31,	
	2024	2023	2023	2022
	(in thousands, except for share and per share data)			
	(unaudited)			
Statements of Operations and Comprehensive Loss Data:				
Operating expenses:				
Research and development	$ 43,035	$ 30,736	$ 40,626	$ 34,605
General and administrative	9,388	7,002	9,707	6,767
Total operating expenses	52,423	37,738	50,333	41,372
Loss from operations	(52,423)	(37,738)	(50,333)	(41,372)
Other income:				
Interest income	6,051	2,216	2,769	1,132
Other income	532	135	301	—
Total other income	6,583	2,351	3,070	1,132
Net loss	$ (45,840)	$ (35,387)	$ (47,263)	$ (40,240)
Net loss per share, basic and diluted(1)	$ (12.81)	$ (12.20)	$ (16.11)	$ (15.61)
Weighted-average shares of common stock outstanding, basic and diluted(1)	3,579,423	2,901,137	2,933,218	2,577,544
Pro forma net loss per share, basic and diluted (unaudited) (2)	$ (1.65)		$ (2.73)	
Pro forma weighted average shares of common stock outstanding, basic and diluted (unaudited)(2)	27,797,075		17,332,468	

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(1) See Note 12 to our unaudited condensed consolidated financial statements and Note 2 and Note 14 to our audited consolidated financial statements included elsewhere in this prospectus for an explanation of the method used to calculate historical net loss attributable to common stockholders per share, basic and diluted, and the weighted-average number of shares of common stock used in the computation of the per share amounts.

(2) Unaudited pro forma net loss per share, basic and diluted, attributable to common stockholders, is calculated giving effect to the conversion of all outstanding shares of our convertible preferred stock into shares of our common stock. Unaudited pro forma net loss per share attributable to common stockholders does not include the shares expected to be sold and related proceeds to be received in this offering. Unaudited pro forma net loss per share attributable to common stockholders for the nine months ended September 30, 2024 and the year ended December 31, 2023 was calculated using the weighted-average number of shares of common stock outstanding as of such date, including the pro forma effect of the conversion of all outstanding shares of our convertible preferred stock into 27,149,206 shares of our common stock, as if such conversion had occurred at the beginning of the period, or their issuance dates, if later.

	September 30, 2024		
	Actual	Pro Forma(1)	Pro Forma As Adjusted(2)
	(in thousands, except for share data) (unaudited)		
Balance Sheet Data:			
Cash, cash equivalents and marketable securities	$ 180,874	$ 180,874	$ 354,990
Working capital(3)	133,859	133,859	308,826
Total assets	197,265	197,265	369,461
Total liabilities	15,446	15,446	14,595
Convertible preferred stock	330,368	—	—
Accumulated deficit	(165,238)	(165,238)	(165,238)
Total stockholders' (deficit) equity	(148,549)	181,819	354,866

(1) Pro forma amounts give effect to the automatic conversion of all outstanding shares of our convertible preferred stock into an aggregate of 27,149,206 shares of our common stock and the related reclassification of the carrying value of the convertible preferred stock to permanent equity immediately prior to the closing of this offering.

(2) Pro forma as adjusted amounts give effect to (i) the pro forma adjustments set forth in footnote (1) above and (ii) the issuance and sale of 10,588,233 shares of our common stock in this offering at the initial public offering price of $18.00 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

(3) We define working capital as current assets less current liabilities. See our consolidated financial statements and the related notes included elsewhere in this prospectus for further details regarding our current assets and current liabilities.

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RISK FACTORS

Investing in our common stock involves a high degree of risk. You should consider and read carefully all of the risks and uncertainties described below, as well as the other information in this prospectus, including our audited consolidated financial statements and the related notes appearing elsewhere in this prospectus and "Management's Discussion and Analysis of Financial Condition and Results of Operations" before deciding whether to invest in our common stock. The risks described below are not the only ones facing us. The following risks or additional risks and uncertainties not presently known to us or that we currently believe to be immaterial could materially and adversely affect our business, financial condition, results of operations and growth prospects. In such an event, the trading price of our common stock could decline, and you may lose all or part of your investment.

This prospectus also contains forward-looking statements and estimates that involve risks and uncertainties not presently known to us or that we currently deem immaterial also may impair our business operations. Our actual results could differ materially from those anticipated in our forward-looking statements as a result of specific factors, including the risks and uncertainties described below.

Risks Related to Our Limited Operating History, Financial Condition and Need for Additional Capital

We are a clinical-stage biopharmaceutical company and have incurred significant operating losses since inception and anticipate that we will continue to incur significant operating losses for the foreseeable future. Our net losses were $47.3 million and $40.2 million for the years ended December 31, 2023 and 2022, respectively. We had an accumulated deficit of $165.2 million and $119.4 million as of September 30, 2024 and December 31, 2023, respectively. We may never achieve or maintain profitability.

We are a clinical-stage biopharmaceutical company with a limited operating history. We have incurred operating losses in each year since our inception. Our net losses were $47.3 million and $40.2 million for the years ended December 31, 2023 and 2022, respectively. We had an accumulated deficit of $165.2 million and $119.4 million as of September 30, 2024 and December 31, 2023, respectively. Substantially all of our operating losses have resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' deficit and working capital.

Since our inception in 2019, we have devoted substantially all of our efforts and financial resources to the development of our product candidates, the continuation of ongoing clinical trials, the commencement of new clinical trials and ongoing manufacturing to support our product candidates. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We have not yet demonstrated an ability to conduct later-stage clinical trials, obtain regulatory approval, manufacture any product on a commercial scale or conduct sales and marketing activities necessary for successful product commercialization, and there is no assurance that we will accomplish any of these abilities in the future. Our limited operating history makes any assessment of our future success and viability subject to significant uncertainty. In addition, if we obtain marketing approval for any of our product candidates, we will incur significant sales, marketing and manufacturing expenses. Once we are a public company, we will incur additional costs associated with operating as a public company. As a result, we expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. Because of the numerous risks and uncertainties associated with developing pharmaceutical products, we are unable to predict the extent of any future losses or when we will become profitable, if at all. Even if we become profitable, we may not be able to sustain or increase our profitability on a quarterly or annual basis.

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The amount of our future losses is uncertain, and our quarterly operating results may fluctuate significantly or may fall below the expectations of investors or securities analysts, each of which may cause our stock price to fluctuate or decline. Our operating losses may fluctuate significantly from quarter to quarter and from year to year. We anticipate that our expenses will increase substantially if, and as, we:

- continue to advance clinical development of our current and future product candidates, including conducting our ongoing clinical trials;
- continue to advance our research and preclinical activities and seek to discover and develop additional product candidates;
- continue to utilize third parties to manufacture our product candidates and ensure sufficient supply of our manufacturing of drug substances and drug products;
- continue to develop, maintain, expand and protect our intellectual property portfolio (including intellectual property obtained through license agreements) and provide reimbursement of third-party expenses related to our patent portfolio;
- attract, hire and retain additional qualified personnel;
- seek regulatory approvals for any current or future product candidates that successfully complete clinical trials;
- make any potential milestones, royalties or other payments due under any current or future in-license, collaboration or other agreements;
- undertake any pre-commercial activities and scale up external commercial-scale manufacturing capabilities;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain regulatory approval;
- add additional operational, financial, clinical, quality and management information systems; and
- incur additional audit, legal, regulatory, tax and other expenses with being a public company.

We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue.

Given the numerous risks and uncertainties associated with pharmaceutical product development, it is not certain if any of our current or future product candidates will advance through late-stage development or be approved for commercial sale; therefore, we are unable to predict if or when we will generate product revenue or achieve or maintain profitability.

Even if we successfully complete development and obtain the necessary regulatory approval for commercialization for any of our product candidates, we anticipate incurring significant costs associated with launching and commercializing such products. If we fail to become profitable or do not sustain profitability on a continuing basis, we may be unable to continue our operations at planned levels and be forced to reduce or cease operations.

Even if this offering is successful, we will need substantial additional funding. We may be unable to raise capital on acceptable terms, if at all, and, as a result, we may be required to delay, reduce or eliminate our product development programs or commercialization efforts.

Our operations have consumed substantial amounts of cash since inception. Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory approvals and achieve product sales. We expect to continue to

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incur significant and increasing expenses and operating losses for the foreseeable future as we initiate and conduct clinical trials of our current and future product candidates, scale-up and manufacture our product candidates, advance our preclinical programs, seek marketing and regulatory approvals for any product candidates that successfully complete clinical trials and commercialize our products, if approved. Because the outcome of any clinical trial or preclinical study is highly uncertain, we cannot reliably estimate the actual amount of financing necessary to successfully complete the development and commercialization of any of our product candidates.

We believe that the anticipated net proceeds from this offering, together with our existing cash, cash equivalents and investments in marketable securities, will be sufficient to fund our operating expenses and capital requirements into 2028. This estimate is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we expect. Changes may occur beyond our control that would cause us to consume our available capital before that time, including but not limited to changes in progress of our development activities, acquisitions of additional product candidates and changes in regulation. Our future capital requirements will depend on many factors, including:

- the scope, timing, progress, costs, complexity and results of discovery, preclinical development and clinical trials for our current or future product candidates, including our planned clinical trials of our lead nucleotide-binding domain ("NBD1") stabilizer product candidate in combination with Vertex Pharmaceuticals, Inc.'s ("Vertex") Trikafta, the current standard of care for the treatment of CF, and in combination with our lead complementary modulator product candidate;
- the number of clinical trials required for regulatory approvals of our current or future product candidates;
- the extent to which we develop, in-license or acquire other product candidates in our pipeline;
- the costs and timing of process development and manufacturing scale-up activities associated with our product candidates and other programs as we advance them through preclinical and clinical development and, if approved, commercialization;
- the number and development requirements of product candidates that we may pursue;
- the timing and amount of the milestone, royalty or other payments we must make to Sanofi SA ("Sanofi"), the Cystic Fibrosis Foundation ("CFF"), AbbVie Global Enterprises Ltd. ("AbbVie") and any other third parties;
- the costs, timing and outcome of regulatory review of our product candidates;
- our headcount growth and associated costs as we expand our research and development capabilities and establish a commercial infrastructure;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any of our product candidates for which we receive marketing approval;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- our ability to achieve sufficient market acceptance, coverage and adequate reimbursement from third-party payors (or patients' willingness to pay out-of-pocket for any approved products in the absence of such coverage) and adequate market share and revenue for any approved products;
- the revenue, if any, received from commercial sales of any product candidates for which we receive marketing approval;

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- the effect of macroeconomic trends including inflation and interest rates;
- addressing any potential supply chain interruptions or delays; and
- the costs of operating as a public company.

We will require additional capital to achieve our business objectives. Additional funds may not be available on a timely basis, on favorable terms or at all, and such funds, if raised, may not be sufficient to enable us to continue implementing our long-term business strategy. Further, our ability to raise additional capital may be adversely impacted by potentially worsening global economic conditions and the recent disruptions to and volatility in the credit and financial markets in the United States ("U.S.") and worldwide resulting from factors that include but are not limited to, inflation, the Russia-Ukraine and Israel-Gaza conflicts, diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates, uncertainty about economic stability and other factors. If the equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult to obtain, more costly and more dilutive. If we are unable to raise sufficient additional capital, we could be forced to curtail our planned operations and the pursuit of our growth strategy, or even cease operations.

Raising additional capital may cause dilution to our stockholders, including purchasers of our common stock in this offering, restrict our operations or require us to relinquish rights to our product candidates.

Until such time, if ever, as we can generate substantial revenues from product sales, we expect to finance our cash needs through a combination of equity offerings, debt financings or other capital sources, such as grants, collaborations, licenses or other similar arrangements. We do not currently have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest may be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing and preferred equity financing may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt or making capital expenditures. Such restrictions could adversely impact our ability to conduct our operations and execute our business plan.

If we raise additional funds through grants, collaborations, licenses or other similar arrangements with third parties, we may be required to relinquish valuable rights to our future revenue streams, intellectual property or product candidates, grant licenses on terms that may not be favorable to us and/or that may reduce the value of our common stock or commit us to future payment streams. If we are unable to raise additional funds through equity or debt financings when needed or on terms acceptable to us, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves, or on less favorable terms than we would otherwise choose.

We maintain the majority of our cash and cash equivalents in accounts with major U.S. and multi-national financial institutions, and our deposits at certain of these institutions exceed insured limits. Market conditions and changes in financial regulations and policies can impact the viability of these institutions. In the event of failure of any of the financial institutions where we maintain our cash and cash equivalents, there can be no assurance that we would be able to access uninsured funds in a timely manner or at all. Any inability to access or delay in accessing these funds could adversely affect our business and financial position. In addition, changes in regulations governing financial institutions are beyond our control and difficult to predict; consequently, the impact of such changes on our business and results of operations is difficult to predict and may have an adverse effect on us.

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Risks Related to the Discovery and Development of Our Product Candidates

We are substantially dependent on the success of our NBD1 stabilizers. If we are unable to advance a lead NBD1 stabilizer product candidate into later-stage clinical development or unable to obtain regulatory approval and commercialize an NBD1 stabilizer-anchored therapy for the treatment of cystic fibrosis, or experience significant delays in doing so, our business will be materially harmed.

To date, as an organization, we have not completed the development of any product candidates. We are substantially dependent on the success of at least one of our NBD1 stabilizer product candidates, including SION-719 and SION-451, which are currently in Phase 1 clinical trials in healthy volunteers and in development for the treatment of cystic fibrosis ("CF"). Our NBD1 stabilizers are being developed for use either in combination with one of our complementary modulator candidates or the standard of care. We intend to select a lead complementary modulator candidate from our two most advanced modulator candidates, galicaftor (SION-2222), which has been evaluated in Phase 2 clinical trials conducted by AbbVie, and SION-109, which has been evaluated in a Phase 1 clinical trial.

The success of SION-719, SION-451 and our other product candidates will depend on several factors, including the following:

- successful and timely initiation and enrollment of clinical trials and completion of clinical trials with favorable results;
- the safety, tolerability and pharmacokinetic profile of our product candidates observed in clinical trials, potentially including in combination with the current standard of care;
- acceptance of regulatory submissions by the U.S. Food and Drug Administration ("FDA") and/or comparable foreign regulatory authorities for the conduct of clinical trials of our product candidates, including acceptance by the FDA of an investigational new drug application ("IND") for our lead NBD1 stabilizer product candidate prior to commencement of our planned Phase 2 trials and our proposed design of such planned clinical trials;
- the frequency and severity of adverse safety findings in nonclinical studies and adverse events ("AEs") in clinical trials;
- timely and successful completion of preclinical studies, including toxicology studies, biodistribution studies and *in vitro* dose projection studies in animals, where applicable;
- acceptance of our products, if approved, by CF patients, the medical community and third-party payors, and their perspective on the cost, safety, tolerability and efficacy and perceived advantages of alternative therapies for CF, including the current standard of care;
- maintaining relationships with contract research organizations ("CROs") and clinical sites for the clinical development of our product candidates and ability of such CROs and clinical sites to comply with clinical trial protocols, Good Clinical Practices ("GCPs") and other applicable requirements;
- demonstrating the safety and efficacy of our product candidates to the satisfaction of applicable regulatory authorities;
- receipt and maintenance of marketing approvals from applicable regulatory authorities for our initial target indication and label expansions to include new populations;
- maintaining relationships with our third-party manufacturers and their ability to comply with current good manufacturing practices ("cGMPs"), as well as making arrangements with our third-party manufacturers for commercial manufacturing capabilities at a cost and scale sufficient to support commercialization;

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- establishing sales, marketing and distribution capabilities and launching commercial sales of our product candidates, if and when approved, whether alone or in collaboration with others;
- obtaining, establishing, maintaining and enforcing patent and any potential trade secret protection or regulatory exclusivity for our product candidates;
- maintaining an acceptable safety profile of our product candidates following regulatory approvals, if any;
- the sufficiency of our financial resources to fund our operations; and
- maintaining and growing an organization of people who can develop and, if approved, commercialize, market and sell our current or future product candidates.

If we are unable to develop, receive marketing approval for and successfully commercialize our product candidates, or if we experience delays as a result of any of the above factors or otherwise, our business would be significantly harmed.

We are early in our development efforts. If we are unable to successfully develop, receive regulatory approval for and commercialize any product candidate or successfully develop any other product candidate or experience significant delays in doing so, our business will be substantially harmed.

We are early in our development efforts. Each of our product candidates will require additional preclinical and/or clinical development, regulatory approval, obtaining manufacturing supply, capacity and expertise, building a commercial organization or successfully outsourcing commercialization, substantial investment and significant marketing efforts before we generate any revenue from product sales.

Our assumptions about the development potential of SION-719 and SION-451 are based entirely on the data generated from our ongoing Phase 1 clinical trials of SION-719 and SION-451 in healthy subjects and from preclinical studies. We have not, as a company, completed any clinical trials of our product candidates in any CF patients to date. We may also observe materially and adversely different safety results as we continue to conduct our clinical trials.

Our product candidates will require substantial additional investment, clinical development, regulatory review, approval in one or more jurisdictions and significant marketing efforts before we could generate any revenue from product sales, if ever. Given our early stage of development, it will take several years before we can demonstrate the safety and efficacy of a product candidate sufficient to warrant approval for commercialization, if we can do so at all.

We do not have complete control over many of these factors, including certain aspects of clinical development and the regulatory submission process, potential threats to our intellectual property rights and the manufacturing, marketing, distribution and sales efforts of any future collaborator. If we are not successful with respect to one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize the product candidates we develop, which would materially harm our business. If we do not receive marketing approvals for our product candidates or any future product candidate we develop or if we experience delays in such approvals, we may not be able to continue our operations.

Further, conducting clinical trials in foreign countries, as we may do for our current or future product candidates, presents additional risks that may delay completion of our clinical trials. These risks include the failure of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, failure to properly translate or interpret patient-reported outcome endpoints, managing additional administrative burdens associated with foreign regulatory schemes as well as political and economic risks relevant to such foreign countries.

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The foregoing makes our ability to successfully and timely complete development of our product candidates and obtain regulatory approval for them less certain. If we are unable to develop, or obtain marketing approval for, or, if approved, successfully commercialize our product candidates, our business, financial condition, results of operations and prospects could be materially harmed.

We intend to develop our lead NBD1 stabilizer product candidate to be administered in combination with one of our complementary modulators or as an add-on to the standard of care. Developing combination treatments increases complexity and risk, including risks of drug-drug interactions, unforeseen side effects or failures in our clinical trials that could delay or prevent their regulatory approval or limit the commercial profile of an approved label.

The current treatment paradigm in CF for all patients with the F508del mutation is based on a combination of drug therapies. After the completion of our ongoing Phase 1 clinical trials of SION-719 and SION-451, we intend to select a lead NBD1 stabilizer product candidate for further development in combination with other compounds, specifically, with one of our complementary modulator product candidates, or with the standard of care. We intend to select a lead complementary modulator candidate from our two most advanced modulator candidates, galicaftor and SION-109. The use of our product candidates in combination with each other and with an approved product may subject us to risks that we would not face if our product candidates were being developed to be administered as a monotherapy.

For example, either the combination of our product candidates with each other, or our NBD1 stabilizer with the standard of care, may result in adverse side effects or toxicities that the product candidates or other therapy do not produce when used alone. In addition, the product candidates may interact with each other, or with the approved product, in undesirable ways that could negatively impact the efficacy of our product candidates, any components of the approved product, or the combination as a whole. Testing product candidates in combination with each other and with an approved therapy may increase the risk of significant adverse effects or failed clinical trials. The timing, outcome and cost of developing products to be used in combination with other therapies is difficult to predict and dependent on a number of factors that are outside our reasonable control.

In addition, to the extent we choose to develop a product candidate for use in combination with an approved therapy, the FDA or comparable foreign regulatory authorities could revoke approval of, or that safety, efficacy, manufacturing or supply issues could arise with, the therapy used in combination with our product candidate. If the therapies we use in combination with our product candidates are replaced as the standard of care, the FDA or comparable foreign regulatory authorities may require us to conduct additional clinical trials, or we may not be able to obtain adequate reimbursement from third-party payors. The occurrence of any of these risks could result in our product candidates, if approved, being removed from the market or being less successful commercially. If the FDA or comparable foreign regulatory authorities do not approve or revoke their approval of, or if safety, efficacy, manufacturing, or supply issues arise with, therapies we choose to evaluate in combination with any of our product candidates, we may be unable to obtain regulatory approval of or to commercialize such product candidates in combination with these therapies. If we experience safety, tolerability or toxicity issues in our planned combination treatment clinical trials, or the data from these trials regarding the efficacy of the combinations are not favorable, our clinical development plans could be materially negatively affected or delayed, or we may not receive regulatory approval for our product candidates, which would materially harm our business and likely cause the market price of our common stock to decline.

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The regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time-consuming and inherently unpredictable. If we are not able to obtain the required regulatory approval for any product candidate, our business will be substantially harmed.

We are not permitted to market, commercialize, sell or promote any product candidate in the U.S. until we receive regulatory approval of a new drug application ("NDA") for such product candidate in a specific indication from the FDA. Our business is dependent on our ability to successfully complete preclinical and clinical development of, obtain regulatory approval for, and, if approved, successfully commercialize our product candidates and any future product candidates in a timely manner. The time required to obtain approval or other marketing authorizations by the FDA and comparable foreign authorities is unpredictable, and it typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, approval policies, regulations and the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. We have not obtained regulatory approval for any product candidate, and it is possible that we may never obtain regulatory approval for any product candidates we may seek to develop in the future.

Prior to obtaining approval to commercialize any product candidate in the U.S. or abroad, we must demonstrate with substantial evidence from well-controlled clinical trials, to the satisfaction of the FDA or comparable foreign regulatory authorities, that such product candidates are safe and effective for their intended uses. Results from preclinical studies and clinical trials can be interpreted in different ways. Even if we believe the preclinical or clinical data for our product candidates are promising, such data may not be sufficient to support approval by the FDA and other regulatory authorities. The FDA may also require us to conduct additional preclinical studies or clinical trials for our product candidates either prior to or after approval, or it may object to elements of our clinical development programs. For example, for our NBD1 stabilizer product candidates, which are being developed as a potential add-on to standard of care, we would be required to satisfy the regulatory agencies' requirements to demonstrate clinically meaningful improvements in efficacy with the NBD1 stabilizer co-administered with the standard of care, as compared to the standard of care alone. It may be harder to show a clinically meaningful improvement in efficacy in this clinical trial where all participants are receiving the standard of care. Additional safety, efficacy and/or drug-drug interaction data may be required relative to what would be required for a single-agent study. Furthermore, because we intend to develop our NBD1 stabilizer product candidate in combination with our complementary modulator candidate, we must satisfy the regulatory agencies' requirements to demonstrate the contribution of each component in the combination. As neither product candidate in this potential combination is an approved drug, the FDA and other regulatory authorities will require full demonstration of the safety of each component (alone and in combination), as well as the efficacy of the combination (and the contribution of each component).

Our current and future product candidates could fail to receive regulatory approval for many reasons, including the following:

- the FDA or comparable foreign regulatory authorities may disagree as to the design or implementation of our clinical trials and interpretation of data from clinical trials or preclinical studies;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;

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- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of an NDA to the FDA or other submission to obtain regulatory approval in the European Union or elsewhere;
- the FDA or comparable foreign regulatory authorities may find deficiencies with or fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

Of the large number of products in development, only a small percentage successfully complete the FDA or foreign regulatory approval processes and are commercialized. The lengthy approval and marketing authorization process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market our product candidates, which would significantly harm our business, financial condition, results of operations and prospects. The FDA and comparable foreign authorities have substantial discretion in the approval process and determining when or whether regulatory approval will be granted for any product candidate that we develop. The U.S. Supreme Court's July 2024 decision to overturn prior established case law giving deference to regulatory agencies' interpretations of ambiguous statutory language has introduced uncertainty regarding the extent to which the FDA's regulations, policies and decisions may become subject to increasing legal challenges, delays or changes. Even if we believe the data collected from future clinical trials of our product candidates are promising, such data may not be sufficient to support approval by the FDA or any other regulatory authority.

Even if we complete clinical testing and receive approval of a NDA or foreign marketing application for our current or future product candidates, the FDA or the applicable foreign regulatory agency may grant approval or other marketing authorization contingent on the performance of costly additional clinical trials, including post-marketing clinical trials. The FDA or the applicable foreign regulatory agency also may approve or authorize for marketing a product candidate for a more limited indication or patient population than we originally request, and the FDA or applicable foreign regulatory authority may not approve or authorize the labeling that we believe is necessary or desirable for the successful commercialization of a product candidate. Any delay in obtaining, or inability to obtain, applicable regulatory approval or other marketing authorization, or failure to obtain our desired product label, would delay or prevent commercialization of that product candidate and would materially adversely impact our business and prospects.

In addition, the FDA and other regulatory authorities may change their policies, issue additional regulations or revise existing regulations or take other actions, which may prevent or delay approval of our future product candidates under development on a timely basis. Such policy or regulatory changes could impose additional requirements upon us that could delay our ability to obtain approvals, increase the costs of compliance or restrict our ability to maintain any marketing authorizations we may have obtained. Further, macroeconomic and other global conditions have impacted and could in the future impact the ability of the FDA and comparable foreign regulatory authorities to provide any required approvals or marketing authorizations for our product candidates or result in the delay of such approvals or authorizations.

Preclinical and clinical product development involves a lengthy and expensive process, with an uncertain outcome.

Our current assumptions about our product candidates' development potential are based on the data generated from preclinical studies and clinical trials; however, we may observe materially and adversely different safety results as we continue to conduct our clinical trials. In order to obtain FDA

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approval to market a new drug product, we must demonstrate the safety and efficacy of the drug in humans in a manner that satisfies the agency's standards. It is impossible to predict when or if any of our product candidates will prove effective or safe in humans or will receive regulatory approval. Before obtaining marketing approval from regulatory authorities, including the FDA, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety, potency and efficacy of our product candidates in humans. A failure of one or more clinical trials can occur at any stage of testing or at any time during the trial process. The outcome of preclinical testing and early clinical trials may not be predictive of the results of later clinical trials as to safety or efficacy, particularly if later clinical trials have a materially different trial design. The historical failure rate for product candidates in our industry is high, particularly in the earlier stages of development. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

We have not completed all of the clinical trials required for the approval of any of our product candidates. We cannot assure you that any preclinical study or clinical trial that we are conducting, or may conduct in the future, will demonstrate consistent or adequate efficacy and safety to obtain regulatory approval to market our product candidates.

We may incur additional costs and experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.

We may incur additional costs and experience delays in ongoing clinical trials for our product candidates, and we do not know whether future clinical trials, if any, will begin on time, need to be redesigned, enroll an adequate number of patients on time or be completed on schedule, if at all. We may experience numerous unforeseen events during or as a result of preclinical studies or clinical trials that could delay or prevent our ability to continue or complete clinical development, receive marketing approval or commercialize our product candidates, including:

- regulators or institutional review boards not authorizing us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- experiencing delays in reaching, or failing to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites or prospective CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- clinical trials of our product candidates producing negative or inconclusive results, including failure to demonstrate statistical significance, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs;
- failing to demonstrate statistical significance in clinical trials of our product candidates, which may impact the timing and design of late-stage clinical trials for such product candidates, or failing to demonstrate statistical significance in late-stage trials despite promising early stage results;
- the number of patients required for clinical trials of our product candidates being larger than we anticipate; enrollment in these clinical trials being slower than we anticipate, for example, due to the availability of standard of care therapy, changes to standard of care therapy, and the reluctance of patients to discontinue standard of care therapy in order to participate in certain of our future clinical trials; or participants dropping out of these clinical trials or failing to return for post-treatment follow-up at a higher rate than we anticipate;
- our product candidates having undesirable side effects (including drug-drug interactions), unexpected toxicology findings, or other unexpected characteristics, causing us or our investigators, regulators or institutional review boards to suspend or terminate the trials;

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- our third-party contractors failing to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- regulators or institutional review boards requiring that we or our investigators suspend or terminate clinical development for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- future collaborators, if any, may conduct clinical trials in ways they view as advantageous to them but that are suboptimal to us;
- the cost of clinical trials of our product candidates being greater than we anticipate; and
- the supply or quality of our product candidates or other materials necessary, including comparator drug, to conduct clinical trials of our product candidates being insufficient, inadequate or too costly.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not favorable or if there are safety concerns, we may, among other things:

- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to additional post-marketing testing requirements; or
- have the product removed from the market after obtaining marketing approval.

Moreover, principal investigators for our current and future clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or a comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA or a comparable foreign regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or a comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval.

We have not yet completed all testing of any product candidate in clinical trials. Preclinical, interim, topline and preliminary results from our preclinical studies or clinical trials are not necessarily predictive of the results or analyses of such results of later clinical trials. If we cannot replicate the positive results from any preclinical studies or clinical trials of our current or potential future product candidates that have positive results, or if we suffer any other significant setbacks in our later clinical trials, we may be unable to successfully develop, obtain regulatory approval for and commercialize our current or potential future product candidates.

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will generate the same results or otherwise provide adequate data to demonstrate the efficacy and safety of a product candidate. Preclinical studies, Phase 1 and Phase 2a clinical trials are primarily designed to test safety, to study pharmacokinetics and pharmacodynamics, and to understand the side effects of

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product candidates at various doses and dosing schedules. Success in preclinical or animal studies and early clinical trials does not ensure that later large-scale efficacy trials will be successful, nor does it predict final results. Our product candidates may fail to show the desired safety, tolerability, pharmacokinetic profile, and efficacy in clinical development despite positive results in preclinical studies or having successfully advanced through initial clinical trials, particularly because our NBD1 stabilizer product candidates target the NBD1 domain of the cystic fibrosis transmembrane conductance regulator ("CFTR") protein, a novel target which has not yet been tested in CF patients.

Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials. Such setbacks have occurred and may occur for many reasons, including: clinical sites and investigators may deviate from clinical trial protocols, whether due to lack of training or otherwise, and we may fail to detect any such deviations in a timely manner; patients may fail to adhere to any required clinical trial procedures, including post-treatment follow-up; our product candidates may fail to demonstrate effectiveness or safety in certain patient subpopulations or at all; or our clinical trials may not adequately represent the patient populations we intend to treat, whether due to limitations in our trial designs or otherwise. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may experience regulatory delays or rejections as a result of many factors, including changes in regulatory policy during the period of our product candidate development.

Similarly, from time to time, we may publish interim, topline or preliminary results from our preclinical studies and clinical trials, which are based on a preliminary analysis of then-available data. We also make assumptions, estimations, calculations and conclusions as part of our preliminary analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. Interim results from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or topline results also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim, topline and preliminary data should be viewed with caution until the final data are available. Adverse differences between interim, topline or preliminary data and final data could significantly harm our business prospects and may cause the trading price of our common stock to fluctuate significantly.

For example, central to our pipeline prioritization strategy is our application of an *in vitro* electrophysiology assay to measure CFTR function in a CF model, known as the cystic fibrosis human bronchial epithelial ("CFHBE") model, which uses lung cells from CF patients and has been shown to be predictive of clinical outcomes for approved CFTR modulators. In preclinical studies using our CFHBE model, the combination of either SION-719 or SION-451 with one of our complementary modulators or the components of Trikafta showed significant improvement in *in vitro* CFTR activity and the potential for restoration of CFTR function to wild-type levels. However, we have not yet replicated such results in clinical trials, and our product candidates may fail to show the desired safety and efficacy at the dose ranges we have identified using our CFHBE model. Positive results in our preclinical studies using our CFHBE model may not be predictive of the results in clinical trials and may fail to be replicated in clinical trials.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is

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based on what is typically extensive information, and investors or others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure. If the interim, topline or preliminary data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our product candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

Targeting the NBD1 domain of the CFTR protein is novel, and we do not know whether we will be able to successfully develop any products.

Our NBD1 stabilizer product candidates target the NBD1 domain of the CFTR protein, which is a novel target. Other companies have discontinued programs targeting NBD1 as they were unable to develop compounds that could successfully bind to NBD1. We intend to develop one of our NBD1 stabilizers as a combination therapy with either one of our complementary modulators, galicaftor or SION-109, or as an add-on to the current standard of care. Given the novelty of our product candidates and the inherent risk in developing a combination or add-on therapy, we may not be able to successfully develop any products.

While products modulating the CFTR protein have been approved by the FDA and comparable foreign regulatory authorities, to date, no product that directly targets the NBD1 domain has been approved, and to our knowledge, no product candidate directly targeting the NBD1 domain is currently in development. As a result, it is difficult to predict the developmental challenges we may encounter as our NBD1 stabilizers proceed through clinical trials, including our planned future clinical trials of our lead NBD1 stabilizer product candidate in combination with galicaftor or SION-109, or as an add-on to the current standard of care. It is also difficult for us to predict the time and cost of development of an NBD1 stabilizer-anchored combination therapy, whether any of our clinical trials will be successful, and whether our novel approach will result in the successful development and regulatory approval of any product candidates. Any development problems we experience in the future related to our NBD1 stabilizer product candidates or any of our complementary modulator candidates may cause significant delays or unanticipated costs, and such development problems may not be able to be solved. The novelty of our product candidates and our combination therapy approach may lengthen the regulatory review process, require us to conduct additional studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. For example, the FDA could require additional studies that may be difficult or impossible to perform, or prohibitively costly. Any of these factors may prevent us from completing clinical trials that we may initiate, and obtaining regulatory approval of or commercializing any product candidates we may develop, on a timely or profitable basis, if at all.

Our preclinical studies and clinical trials may fail to demonstrate the safety and efficacy of our product candidates, or serious or unacceptable adverse side effects or unexpected toxicology findings may be identified during the development of our product candidates, which could prevent or delay further clinical development, regulatory approvals and commercialization, impact the product's labeling, if approved, increase our costs or necessitate the abandonment or limitation of the development of some of our product candidates.

Clinical trials often fail to demonstrate safety or efficacy of the product candidate studied for the target indication. If our product candidates are associated with serious or significant adverse side effects in clinical trials or have adverse safety findings in nonclinical studies, we may need to abandon their development or limit development to more narrow uses in which the side effects or other characteristics are less prevalent, less severe or more acceptable from a benefit-risk perspective. The FDA or other comparable foreign regulatory authority or an institutional review board or ethics committee may also require that we suspend, discontinue or limit our clinical trials based on safety information, or that we

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conduct additional animal or human studies regarding the safety and efficacy of our product candidates, which we have not planned or anticipated. Such findings could further result in regulatory authorities failing to provide marketing authorization for our product candidates or limiting the scope of the indication, if approved. Many product candidates that initially showed promise in early-stage testing have later been found to cause adverse side effects that prevented further development of the product candidate.

Additionally, if one or more of our product candidates receives marketing approval, and we or others subsequently identify undesirable side effects caused by such products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw, suspend or limit approvals of such product or seek an injunction against its manufacture or distribution;
- we may be required to recall a product;
- regulatory authorities may require additional warnings on the labels, such as a boxed warning or a contraindication;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients;
- we may be required to change the way a product is distributed or administered, conduct additional clinical trials or change the labeling of a product or be required to conduct additional post-marketing studies or surveillance;
- we could be sued and held liable for harm caused to patients;
- sales of the product may decrease significantly or the product could become less competitive;
- we may not be able to achieve or maintain third-party payor coverage and adequate reimbursement; and
- our reputation and physician or patient acceptance of our products may suffer.

There can be no assurance that we will resolve any issues related to any product-related AEs to the satisfaction of the FDA or comparable foreign regulatory authorities in a timely manner or at all. Moreover, any of these events could prevent us from achieving or maintaining market acceptance of a particular product candidate, if approved, and could significantly harm our business, results of operations and prospects.

We may not be able to obtain orphan drug designation or exclusivity for our product candidates, and even if we do, we may be unable to maintain the benefits associated with orphan drug designation, including the potential for market exclusivity.

We may seek orphan drug designation or exclusivity in the indications targeted by our current or future product candidates. Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. For example, under the Orphan Drug Act, the FDA may designate a product candidate as an orphan drug if it is intended to treat a rare disease or condition. In order for the FDA to grant orphan drug exclusivity to one of our product candidates, the agency must find that the product candidate is indicated for the treatment of a condition or disease that affects fewer than 200,000 individuals in the U.S. or that affects 200,000 or more individuals in the U.S. and for which there is no reasonable expectation that the cost of developing and making the product candidate available for the disease or condition will be recovered from sales of the product in the U.S. Orphan drug designation must be requested before submitting an NDA. The FDA may conclude that the condition or disease for which we seek orphan drug exclusivity does not meet the required standard.

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If a product that has orphan drug designation subsequently receives the first FDA approval for a particular drug for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same drug for the same indication for seven years, except in limited circumstances such as if the FDA finds that the holder of the orphan drug exclusivity has not shown that it can assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the disease or condition for which the drug was designated.

In addition, even after an orphan drug is approved, the FDA can subsequently approve the same product candidate for the same condition if the FDA concludes that the later product candidate is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care compared with the product that has orphan exclusivity. In the U.S., orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and user-fee waivers. After the FDA grants orphan drug designation, the generic identity of the drug or biologic and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

A similar regulatory scheme governs approval of orphan product candidates by the European Medicines Agency (“EMA”) in the European Union. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA (as applicable) from approving another marketing application for the same or another similar product candidate for the same orphan therapeutic indication for that time period. The applicable period is seven years in the U.S. and ten years in the European Union. The exclusivity period in the European Union can be reduced to six years if at the end of the fifth year it is determined that a product no longer meets the criteria for orphan drug designation, including if the product is sufficiently profitable so that market exclusivity is no longer justified.

While we may in the future seek designations for our product candidates with the FDA and comparable foreign regulatory authorities that are intended to confer benefits such as a faster development process, an accelerated regulatory pathway or priority review, there can be no assurance that we will successfully obtain such designations. In addition, even if one or more of our product candidates are granted such designations, we may not be able to realize the intended benefits of such designations.

The FDA and comparable regulatory authorities offer certain designations for product candidates that are designed to encourage the research and development of product candidates that are intended to address conditions with significant unmet medical need. These designations may confer benefits such as additional interaction with regulatory authorities, a potentially accelerated regulatory pathway and priority review of the marketing application(s). However, there can be no assurance that we will successfully obtain such designations for our product candidates. In addition, while such designations could expedite the development or approval process, they generally do not change the standards of product quality, safety or efficacy required to be demonstrated in support of approval. Even if we obtain such designations for our product candidates, there can be no assurance that we will realize their intended benefits.

For example, we may seek a Fast Track Designation for one or more of our product candidates. If a product is intended for the treatment of a serious or life-threatening condition and preclinical or clinical data demonstrate the potential to address an unmet medical need for this condition, the product sponsor may apply for Fast Track Designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, we

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cannot assure you that the FDA would decide to grant it. Even if we do receive Fast Track Designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may rescind the Fast Track Designation if it believes that the designation is no longer supported by data from our clinical development activities.

We may seek Breakthrough Therapy Designation for any product candidate that we develop. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over currently approved therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as breakthrough therapies by the FDA are also eligible for accelerated approval and priority review.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. Further, the receipt of Breakthrough Therapy Designation for a product candidate may not result in a faster development process, review or approval compared to conventional FDA procedures and does not assure approval by the FDA. In addition, even if any product candidate we develop qualifies for Breakthrough Therapy Designation, the FDA may later decide that the drug no longer meets the conditions for qualification and rescind the designation.

Even in the absence of obtaining Fast Track and/or Breakthrough Therapy Designations, a sponsor can seek priority review at the time of submitting a marketing application. The FDA may designate a product for priority review if it is a product that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness when compared with other available therapies. Significant improvement may be illustrated by evidence of increased effectiveness in the treatment of a condition, elimination or substantial reduction of a treatment-limiting adverse reaction, documented enhancement of patient compliance that may lead to improvement in serious outcomes, or evidence of safety and effectiveness in a new subpopulation. A priority review designation is intended to direct overall attention and resources to the evaluation of such applications, and to shorten the FDA's goal for taking action on a marketing application from ten months to six months from FDA's acceptance of the application for review. Priority review designation may be rescinded if a product no longer meets the qualifying criteria.

We may not be able to submit INDs or IND amendments to commence clinical trials on the timelines we expect, and even if we are able to, the FDA may not permit us to proceed.

We may not be able to submit INDs, including the IND for our lead NBD1 stabilizer product candidate, on the timelines we expect. For example, we may experience manufacturing delays or other delays with IND-enabling studies. Moreover, we cannot be sure that submission of an IND will result in the FDA allowing clinical trials to begin, or that, issues will not arise that suspend or terminate clinical trials. Additionally, even if such regulatory authorities agree with the design and implementation of the clinical trials set forth in an IND, we cannot guarantee that such regulatory authorities will not change their requirements in the future. These considerations also apply to new clinical trials we may submit as amendments to existing INDs.

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Any product candidate for which we obtain marketing approval could be subject to restrictions or withdrawal from the market, and we may be subject to substantial penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved.

As part of its decision to approve or grant marketing authorization for one or more of our product candidates, the FDA or other regulatory agencies may require us to perform certain post-marketing activities, such as completion of ongoing or planned studies, initiation of new studies or post-marketing clinical trials (including to assess safety risks), or additional analyses of existing data. Typically, we are required to provide annual updates on the progress of such required activities and to complete the activities by the assigned completion dates. Later discovery of previously unknown problems with our product candidates, including AEs of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of such products, withdrawal of the product from the market or voluntary or mandatory product recalls;
- restrictions on or revisions to the labeling or marketing of a medicine;
- restrictions on the distribution or use of a medicine, including under a risk evaluation and mitigation strategy (“REMS”) program;
- fines, receipt of warning or untitled letters or suspension of clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or suspension or withdrawal of marketing approvals;
- product seizure or detention or refusal to permit the import or export of our product candidates; and
- injunctions or the imposition of civil or criminal penalties.

Additionally, the FDA and other regulatory agencies closely regulate the post-approval marketing and promotion of medicines to ensure that they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. Although physicians may prescribe products for uses not described in the product’s labeling, known as off-label uses, in their professional medical judgment, the FDA and comparable foreign regulatory agencies impose stringent restrictions on manufacturers’ communications regarding off-label use, and if we market our products, if approved, in a manner inconsistent with their approved labeling, we may be subject to enforcement action for off-label marketing by the FDA and other federal and state enforcement agencies, including the Department of Justice and other comparable foreign regulatory agencies. Violation of the Federal Food, Drug, and Cosmetic Act and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription products may also lead to investigations or allegations of violations of federal and state healthcare fraud and abuse laws, state consumer protection laws and laws of other comparable foreign regulatory agencies.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize any product candidates we develop and adversely affect our business, financial condition, results of operations and prospects.

If we experience delays or difficulties in the enrollment and/or retention of patients in clinical trials, our clinical development activities could be delayed or otherwise adversely affected, and our receipt of necessary regulatory approvals could be delayed or prevented.

Successful and timely completion of clinical trials will require that we identify and enroll a sufficient number of patients. Patient enrollment, a significant factor in the timing of clinical trials, is

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affected by many factors, including the size and nature of the patient population and competition for patients with other trials. Trials may be subject to delays as a result of patient enrollment taking longer than anticipated or patient withdrawal. We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or comparable foreign regulatory authorities, or if a large number of patients withdraw. We cannot predict how successful we will be at enrolling subjects in future clinical trials. We may conduct clinical trials that would require patients to discontinue standard of care therapy, and we may experience challenges finding, enrolling and retaining CF patients in our planned clinical trials who are willing to discontinue their current treatment regimens to participate in our trials. For example, AbbVie previously terminated part of a Phase 2 trial that was intended to evaluate multiple doses of navocaftor in combination with a fixed dose of galicaftor because this part was deemed not enrollable due to, among other reasons, the increasing availability of Trikafta. Subject enrollment is affected by other factors including:

- the patient eligibility criteria as defined in the applicable protocol;
- the size of the patient population required for analysis of the trial's primary endpoints and the process for identifying patients;
- the actual and perceived risks and benefits of the product candidate in the trial;
- the design of the trial;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- competing clinical trials and clinicians' and patients' perceptions as to the potential advantages and risks of the product candidate being studied in relation to other available therapies, including the current standard of care and any new drugs that may be approved for CF, which may vary across the jurisdictions where we plan to conduct our clinical trials;
- the willingness of patients to be enrolled in our clinical trials;
- the success of efforts to facilitate timely enrollment in clinical trials;
- the patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment;
- our ability to obtain and maintain informed consent;
- the risk that patients enrolled in our clinical trials will drop out of the trials prior to completion;
- the cost to, or lack of adequate compensation for, prospective patients; and
- the proximity and availability of clinical trial sites to prospective patients.

Our inability to enroll a sufficient number of patients for clinical trials would result in significant delays and could require us to abandon one or more clinical trials altogether. Enrollment delays in these clinical trials may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing. Furthermore, we expect to rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials and we will have limited influence over their performance.

Furthermore, even if we are able to enroll a sufficient number of patients for our clinical trials, we may have difficulty maintaining enrollment of such patients. We cannot assure you that our assumptions used in determining expected clinical trial timelines are correct or that we will not experience delays or difficulties in enrollment, or be required by the FDA or comparable foreign regulatory authorities to increase our enrollment, which would result in the delay of completion of such trials beyond our expected timelines.

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The results of clinical trials conducted at clinical trial sites outside the U.S. might not be accepted by the FDA, and data developed outside of a foreign jurisdiction similarly might not be accepted by such foreign regulatory authority.

We are currently conducting our Phase 1 clinical trials for SION-719 and SION-451 in Australia, and may conduct additional clinical trials outside of the U.S. in the future. Clinical trials conducted in Australia using "unapproved therapeutic goods," or those that have not yet been evaluated by the Therapeutic Goods Association ("TGA") for quality, safety and efficacy, must occur pursuant to either the Clinical Trial Notification Scheme or the Clinical Trial Approval Scheme. In each case, the trial is supervised by a Human Research Ethics Committee ("HREC"), an independent review committee set up under the guidelines of the Australian National Health and Medical Research Council that reviews, approves and provides continuing oversight of trial protocols and amendments, and of the methods and material to be used in obtaining and documenting informed consent of the trial subjects. Although the FDA or comparable foreign regulatory authorities may accept data from clinical trials conducted outside the relevant jurisdiction, acceptance of these data is subject to certain conditions. For example, the FDA requires that the clinical trial must be well-designed and conducted and performed by qualified investigators in accordance with ethical principles such as institutional review board or ethics committee approval and informed consent, the trial population must adequately represent the U.S. population and the data must be applicable to the U.S. population and U.S. medical practice in ways that the FDA deems clinically meaningful. Further, the FDA may consider an on-site inspection to be necessary in which case they must be able to validate the data through such an inspection or other appropriate means. In addition, while these clinical trials are subject to the applicable local laws, acceptance of the data by the FDA will be dependent upon its determination that the trials were conducted consistent with all applicable U.S. laws and regulations. There can be no assurance that the FDA will accept data from trials conducted outside of the U.S. as adequate support of a marketing application. Similarly, any data submitted to foreign regulatory authorities may not adhere to their standards and requirements for clinical trials and data from trials conducted outside of such jurisdiction may not be accepted.

If the FDA or any comparable foreign regulatory authority does not accept such data, it would result in the need for additional trials, which could be costly and time-consuming, and which may result in current or future product candidates that we may develop not receiving approval for commercialization in the applicable jurisdiction. In addition, such foreign trials would be subject to the applicable local laws of the foreign jurisdictions where the trials are conducted, which may increase costs or time required to complete the clinical trial.

Conducting clinical trials outside the U.S. also exposes us to additional risks, including risks associated with:

- additional foreign regulatory requirements;
- foreign exchange fluctuations;
- compliance with foreign manufacturing, customs, shipment and storage requirements;
- inconsistent standards for reporting and evaluating clinical data and AEs;
- varying standards or availability of CF care, resulting in data that may differ from patients who have received the U.S. standard of care therapy;
- any pandemic, epidemic or public health emergencies;
- diminished protection of intellectual property in some countries; and
- political instability, civil unrest, war or similar events that may jeopardize our ability to commence, conduct or complete a clinical trial and evaluate resulting data.

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If any third-party manufacturer of our product candidates is unable to increase the scale of its production of our product candidates or increase the product yield of its manufacturing, then our manufacturing costs may increase and commercialization may be delayed.

In order to produce sufficient quantities to meet the demand for clinical trials and, if approved, subsequent commercialization of our product candidates, our third-party manufacturers will be required to increase their production and optimize their manufacturing processes while maintaining the quality of our product candidates. The transition to larger scale production could prove difficult. In addition, if our third-party manufacturers are not able to optimize their manufacturing processes to increase the product yield for our product candidates, or if they are unable to produce increased amounts of our product candidates while maintaining the same quality, then we may not be able to meet the demands of clinical trials or market demands, which could decrease our ability to generate profits and have a material adverse impact on our business and results of operations.

Changes in methods of product candidate manufacturing or formulation may result in additional costs or delay.

As product candidates proceed through preclinical studies to late-stage clinical trials towards potential approval and commercialization, various aspects of the development program, such as manufacturing methods and formulation, may be altered in an effort to optimize processes and product characteristics, and such optimization may not be achieved. Any of these changes could cause our product candidates to perform differently and affect the results of our current or future clinical trials. Such changes may also require additional testing, or notification to or approval by the FDA or another comparable regulatory authority. This could delay completion of clinical trials, require the conduct of bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our product candidates and jeopardize our ability to commence sales and generate revenue.

Certain of our programs may compete with our other programs, which could negatively impact our business and reduce our future revenue.

We are developing all of our product candidates for the same indication, CF, and we may develop additional product candidates for the same indication in the future. We have several programs targeting different regions of the CFTR protein, in order to support our goal of creating a differentiated combination treatment that delivers clinically meaningful benefit to CF patients. However, developing multiple programs for a single indication may negatively impact our business if the programs compete with each other. For example, if multiple programs are conducting clinical trials at the same time, they could compete for the enrollment of participants. In addition, if multiple product candidates are approved for the same indication, they may compete for market share, which could limit our future revenue.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and management resources, we are focused on a single indication, CF, and we focus our research and development efforts on certain selected development programs and product candidates. We are currently primarily focused on the development of SION-719 and SION-451, our NBD1 stabilizers, and our portfolio of complementary modulators. As a result, we may forego or delay pursuit of opportunities with other product candidates that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future

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development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

Risks Related to Our Dependence on Third Parties

We are dependent on licensed intellectual property. If we were to lose our rights to licensed intellectual property, we may not be able to continue developing or commercializing our product candidates, if approved. If we breach any of the agreements under which we license the use, development and commercialization rights to our product candidates from third parties or, in certain cases, we fail to meet certain development deadlines, we could lose license rights that are important to our business.

We are a party to a number of license agreements under which we are granted rights to intellectual property that are important to our business and we expect that we may need to enter into additional license agreements in the future. In particular, we have entered into license agreements with Sanofi and AbbVie, pursuant to which, among other things, we have secured exclusive licenses for certain intellectual property and know-how relating to CFTR modulator therapies to commercialize certain compounds, patents and proprietary information and inventions. Our existing license agreements impose, and we expect that future license agreements will impose on us, various development, regulatory and/or commercial diligence obligations, payment of milestones and/or royalties and other obligations. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to market products covered by the license. Our business could suffer, for example, if any current or future licenses terminate, if the licensors fail to abide by the terms of the license, if the licensed patents or other rights are found to be invalid or unenforceable, or if we are unable to enter into necessary licenses on acceptable terms. In particular, we are substantially dependent on the success of one of our NBD1 stabilizer product candidates, and to the extent we are unable to develop other product candidates at the time of such termination, it would have a material adverse effect on our business, financial condition, results of operations and prospects, including, but not limited to, cessation of our operations. See "*Business—License and Collaboration Agreements*" for a description of our license agreements.

As we have done previously, we may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates, and we cannot provide any assurances that third-party patents do not exist that might be enforced against our current product candidates or future products in the absence of such a license. We may fail to obtain any of these licenses on commercially reasonable terms, if at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same intellectual property licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement intellectual property. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could materially harm our business and the third parties owning such intellectual property rights could seek either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation.

Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;

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- whether and the extent to which our product candidates and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patent and other rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed intellectual property in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

We currently rely on, and in the future intend to rely on, third parties to conduct a significant portion of our clinical trials and potential future clinical trials for product candidates, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.

We currently engage CROs and other vendors to conduct our ongoing clinical trials, including our ongoing Phase 1 clinical trials of SION-719 and SION-451, and similarly expect to engage CROs and other vendors for future clinical trials for these and other product candidates that we may progress to clinical development. We expect to continue to rely on third parties, including but not limited to clinical data management organizations, healthcare institutions operating as clinical sites and clinical investigators, to conduct those clinical trials. Any of these third parties may terminate their engagements with us, some in the event of an uncured material breach and some at any time for convenience. If any of our relationships with these third parties terminate, we may not be able to timely enter into arrangements with alternative third parties or to do so on commercially reasonable terms, if at all. Switching or adding CROs or other vendors involves substantial cost and requires management time and focus. In addition, there is a natural transition period when a new CRO or vendor commences work. As a result, delays may occur, which can materially impact our ability to meet our desired clinical development timelines. We may encounter challenges or delays in our CRO/vendor relationships in the future which may cause a material adverse impact on our business, financial condition and prospects.

In addition, any third parties conducting our clinical trials will not be our employees, and, except for including contractual obligations and remedies for breach of such obligations in our agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our clinical programs. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approvals for or successfully commercialize our product candidates. Consequently, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase substantially and our ability to generate revenue could be delayed significantly. In addition, many of the third parties with whom we contract may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other development activities that could harm our competitive position.

We rely on these parties for execution of our preclinical studies and clinical trials, and generally do not directly control their businesses or related activities. Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us

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of our responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with GCPs for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We also are required to register certain ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions. If we or any of our CROs or other third parties, including trial sites, fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. Further, upon inspection by a given regulatory authority, such regulatory authority may not agree with our determination that any of our clinical trials complies with GCP requirements. In addition, our clinical trials must be conducted with product produced under cGMP conditions. Our failure to comply with these requirements may require us to repeat clinical trials, which would delay the regulatory approval process.

We also expect to rely on other third parties to store and distribute product supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential revenue.

We contract with third parties for the manufacture of our product candidates for clinical drug supply and expect to continue to do so for commercialization, if our product candidates are approved. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts.

We do not have any cGMP manufacturing facilities and have no plans to develop our own clinical or commercial-scale manufacturing capabilities. We currently rely, and expect to continue to rely, on contract development and manufacturing organizations ("CDMOs") for the cGMP manufacture of our product candidates and related raw materials for clinical development. In addition, we expect to rely on CDMOs for the commercial supply of any products for which we receive marketing approval. This reliance increases the risk that we will not have sufficient quantities of our product candidates or products, if approved, or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts. For example, any of these third parties may terminate their engagements with us at any time or may face production shortages or other supply interruptions or otherwise be unable to secure the requisite raw materials to support our planned clinical activities. In addition, the availability of CDMOs to manufacture our product candidates/drugs may depend in part on the CDMOs' schedules for manufacturing other companies' products or product candidates. If we need to modify our development plans or enter into alternative arrangements, which may not be readily available or available on acceptable terms, it could delay our product development activities and increase our expenses.

Our reliance on CDMOs for manufacturing activities will reduce our control over these activities, but will not relieve us of our responsibility to ensure compliance with all required regulations. In particular, we do not have control over a supplier's or manufacturer's compliance with laws, regulations and applicable cGMP standards or similar regulatory requirements and other laws and regulations, such as those related to environmental health and safety matters. If our CDMOs cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or comparable foreign regulatory authorities, we may be unable to obtain regulatory approval of our potential future marketing applications. In addition, although we conduct routine qualification audits and have quality agreements in place with our CDMOs, we have no direct operational control over their

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ability to maintain adequate quality control, quality assurance and qualified personnel. Our CDMOs may face manufacturing or quality control problems causing production and shipment delays, or CDMOs may fail to maintain compliance with the applicable cGMP requirements. The facilities used by our CDMOs are subject to continual review and periodic inspections by the FDA and comparable foreign regulatory authorities. If the FDA or a comparable foreign regulatory authority finds deficiencies with or does not approve these facilities for the manufacture of our product candidates or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or drugs, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supply of our products.

Our use of foreign CROs and CDMOs in some jurisdictions may be or may become subject to U.S. legislation, including sanctions, trade restrictions and other regulatory requirements, which may increase the cost of and cause delays in the procurement or supply of materials for, or manufacture of, our product candidates or have an adverse effect on our ability to secure significant commitments from governments to purchase its potential therapies. For example, we are party to an agreement with WuXi AppTec (HongKong) Limited, an affiliate of WuXi Biologics ("WuXi"), pursuant to which it may manufacture certain of our clinical trial starting materials from time to time. The recently proposed BIOSECURE Act would have banned U.S. government contracts, grants, and loans from being used towards biotechnology equipment and services produced or provided by certain named Chinese biotechnology companies, including WuXi, and would authorize the U.S. government to name additional Chinese biotechnology companies of concern. The legislation did not pass the U.S. Congress in 2024; however, if a future version of the BIOSECURE Act or similar laws are passed into law, they would have the potential to severely restrict the ability of companies to work with certain Chinese biotechnology companies of concern without losing the ability to contract with, or otherwise receive funding from, the U.S. government.

Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. If our current CDMOs cannot perform as agreed, we may be required to replace such manufacturers. We may incur added costs and delays in identifying and qualifying any such replacement. In addition, our current and anticipated dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins and our ability to commercialize any products that receive marketing approval on a timely and competitive basis.

We may seek to establish collaborations, license agreements and other similar arrangements with third parties for the development or commercialization of our product candidates. If we are not able to establish them on commercially reasonable terms, or if those arrangements are not successful, we may have to alter our development and commercialization plans.

The development and potential commercialization of our product candidates will require substantial additional funding. For some of our product candidates, we may seek to collaborate with other pharmaceutical and biotechnology companies for the development and potential commercialization of our product candidates, including for the commercialization of any of our product candidates that are approved for marketing outside the U.S. If we enter into any such additional arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our future collaborators dedicate to the development or commercialization of our product candidates. Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all.

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We face significant competition in seeking appropriate collaborators, and the negotiation process is time-consuming and complex. Whether we reach a definitive agreement for any collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the potential differentiation of our product candidate from competing product candidates, the design or results of clinical trials, the likelihood of approval by the FDA or comparable foreign regulatory authorities outside the U.S., the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, and industry and market conditions generally. The collaborator may also consider alternative product candidates for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

Collaborations involving our product candidates would pose the following risks to us:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected, or at all;
- collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- a collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such products;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly maintain or defend our or their intellectual property rights or may use our or their proprietary information in such a way as to invite litigation that could jeopardize or invalidate such intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated, including for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

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We may not be able to negotiate future collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of any product candidate that we planned to collaborate on, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate revenue, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, any future collaborations that we enter into may not be successful. The success of our future collaboration arrangements will depend heavily on the efforts and activities of our future collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations. Disagreements between parties to a collaboration arrangement regarding clinical development and commercialization matters can lead to delays in the development process or commercializing the applicable product candidate and, in some cases, termination of the collaboration arrangement. These disagreements can be difficult to resolve if neither of the parties has final decision-making authority. If conflicts arise between any future collaborators and us, the other party may act in a manner adverse to us and could limit our ability to implement our strategies. For example, our future collaborators could conduct multiple product development efforts and could develop, either alone or with others, products in related fields that are competitive with the product candidates we may develop. In addition, collaborations with pharmaceutical or biotechnology companies and other third parties often are terminated or allowed to expire by the other party, and any such termination or expiration may adversely affect us financially or harm our business.

Risks Related to Our Intellectual Property

If we or our licensors are unable to obtain, maintain and enforce intellectual property rights relating to any of our product candidates, or if the scope of the protection obtained is not sufficiently broad, our competitors or other third parties could develop and commercialize products similar or identical to ours, our ability to successfully commercialize our product candidates may be adversely affected and we may not be able to compete effectively in our markets.

We rely upon a combination of patents, access to certain third-party trade secrets and confidentiality agreements to protect the intellectual property related to our product candidates. These legal measures afford only limited protection, and competitors or others may gain access to or use our intellectual property and proprietary information. Our success depends in large part on our ability to obtain and maintain patent and other intellectual property protection in the U.S. and in other countries with respect to our proprietary product candidates.

We cannot predict whether the patent applications we currently or may in the future pursue will issue as patents in any particular jurisdiction or will provide sufficient protection against competitors or other third parties. Currently, much of our patent portfolio, including the applications related to our NBD1 product candidates, are in various, relatively early stages of the patent prosecution process. Nor can we predict the outcome of any challenge by our competitors to the validity or enforceability of any such patents. We cannot offer any assurances that the breadth of our granted patents will be sufficient to stop a competitor from developing and commercializing a product, including a generic product that would be competitive with one or more of our product candidates. Furthermore, any successful challenge to these patents or any other patents owned by or licensed to us after patent issuance could

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deprive us of the competitive advantage necessary for the successful commercialization of any of our product candidates. Further, if we encounter delays in regulatory approval, the period of time during which we could market a product candidate under patent protection could be reduced (unless we obtain a patent term extension corresponding to such delays).

Our ability to obtain and maintain valid and enforceable patents depends on our inventions being patentable in light of the prior art. Furthermore, publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the U.S. and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we or our licensors were the first to invent the inventions claimed in any of our owned or licensed patents or pending patent applications, or that we or our licensors were the first to make the inventions claimed in those owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions. If a third party can establish that we or our licensors were not the first to make or the first to file for patent protection of such inventions, our owned or licensed patents and patent applications may not issue as patents and even if issued, may be challenged and invalidated or rendered unenforceable. Furthermore, even if a patent is granted, our competitors or other third parties may be able to circumvent the patent by developing similar or alternative products in a non-infringing manner which could materially adversely affect our business, financial condition, results of operations and prospects.

The patent prosecution process is expensive and time-consuming. We may not be able to prepare, file and prosecute all necessary or desirable patent applications at a commercially reasonable cost or in a timely manner or in all jurisdictions. It is also possible that we may fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. Moreover, depending on the terms of any existing and future in-licenses to which we may become a party, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering intellectual property in-licensed from third parties. Therefore, these patents and patent applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions, and has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our and our licensors' patent rights are highly uncertain. Our and our licensors' pending and future patent applications may not result in patents being issued which protect our product candidates or which effectively prevent others from commercializing competitive products.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the U.S. and abroad. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. We or our licensors may in the future, become subject to a third-party pre-issuance submission of prior art, opposition, derivation, revocation, re-examination, post-grant and *inter partes* review, or interference proceeding and other similar proceedings challenging our patent rights or the patent rights of others in the U.S. Patent and Trademark Office (the "USPTO") or other foreign patent office. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated, or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical products, or limit the duration of the patent protection of our product candidates.

Furthermore, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly

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after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to our product candidates.

In addition to the protection provided by our patent portfolio, we rely on access to certain third-party trade secret protection as well as confidentiality agreements to protect proprietary know-how that is not amenable to patent protection. Although we generally require all of our employees to assign their inventions to us, and all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or intellectual property to enter into confidentiality agreements, certain employees may not have signed such agreements and we cannot provide any assurances that all such agreements have been duly executed, or that such trade secrets and other confidential proprietary information will not be disclosed. Moreover, our competitors may independently develop knowledge, methods and know-how equivalent to the above-mentioned trade secrets. Competitors could purchase our products, if approved, and replicate some or all of the competitive advantages we derive from our development efforts for intellectual property on which we do not have patent protection. If any trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that intellectual property or information to compete with us. If any trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

We also seek to preserve the integrity and confidentiality of our proprietary data and third-party trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. However, our agreements and security measures may be breached, and we may not have adequate remedies for any breach. Also, if the steps taken to maintain trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. In addition, others may independently discover the trade secrets and our proprietary information. For example, the FDA is considering whether to make additional information publicly available on a routine basis, and it is not clear at the present time how the FDA's disclosure policies may change in the future. If we are unable to prevent material disclosure of the non-patented intellectual property related to our product candidates to third parties, we may not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, results of operations and financial condition.

Patent terms may be inadequate to protect our competitive position on our products for an adequate amount of time, and if we do not obtain protection under the Hatch-Waxman Amendments and similar non-U.S. legislation for extending the term of patents covering each of our product candidates, our business may be materially harmed.

Patents have a limited lifespan. In the U.S., if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if additional patents covering our current or future product candidates are obtained, once the patent life has expired for a product, we may be open to competition from competitive medications, including generic medications. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. Because of these term limits, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours. If we do not have sufficient patent life to protect our product candidates, our business, financial condition, results of operations, and prospects may be adversely affected.

Depending upon the timing, duration and conditions of FDA marketing approval of our product candidates, one or more of our U.S. patents, including any patents that may issue covering our product

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candidates, SION-719 and SION-451 may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments, and similar legislation in the European Union. The Hatch-Waxman Amendments permit a patent term extension of up to five years for a patent covering an approved product that is a new chemical entity as compensation for effective patent term lost during product development and the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval. Only one patent may be extended, and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. However, we may not receive an extension if we fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the length of the extension could be less than we request, and we cannot be certain what the length of the extension would be or if we will receive an extension at all. If we are unable to obtain patent term extension or the term of any such extension is less than we request, the period during which we can enforce our patent rights for that product will be shortened and our competitors may obtain approval to market competing products sooner. As a result, our revenue from applicable products could be reduced and could have a material adverse effect on our business.

If we fail to comply with our obligations in any current intellectual property licenses with third parties, or fail to obtain such licenses in the future, we could lose rights that are material to our business.

We have licensed third-party intellectual property that is material to our business, and may enter into additional license agreements in the future. We do not and will not own the patents or patent applications that underlie these licenses, and we may not control either the prosecution or the enforcement of the patents. Under such circumstances, we may be forced to rely upon our licensors to properly prosecute and file those patent applications and prevent infringement of those patents. Therefore, we cannot be certain that the prosecution, maintenance and enforcement of these patent rights will be in a manner consistent with the best interests of our business.

If we or our licensors fail to maintain such patents, or if we or our licensors lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our product candidates that are the subject of such licensed rights could be adversely affected.

Our rights to use the intellectual property and practice the inventions claimed in the licensed patents and patent applications are subject to our licensors abiding by the terms of those licenses and not terminating them. In addition, existing license agreements do, and future agreements may, impose diligence, development and commercialization timelines and milestone payment, royalty, insurance and other obligations, including rights of first negotiation (“ROFN”) for development of certain programs. For example, under our license agreement with AbbVie, AbbVie has a ROFN pursuant to which it would have an exclusive period to negotiate in good faith the terms of a commercial license transaction if we decide to pursue a license or sublicense to commercialize a licensed product under the AbbVie agreement prior to initiating Phase 3 clinical trials. This ROFN may delay or undermine our ability to enter into an otherwise beneficial transaction. If we fail to comply with our obligations, our licensors may have the right to terminate the licenses, in which event we might not be able to develop, manufacture or market any product that is covered by the intellectual property we in-license from such licensor and may face other penalties. If any of our licenses are terminated, we may lose our patent rights on a territory-by-territory basis, and such rights may be lost worldwide. Termination of any license agreement could reduce or eliminate our rights under these agreements and may result in our having to negotiate new or reinstated agreements with less favorable terms or cause us to lose our rights under these agreements, including our rights to important intellectual property. Any of the foregoing outcomes could prevent us from commercializing relevant product candidates, which could have a material adverse effect on our operating results and overall financial condition.

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In addition, disputes regarding obligations in licenses may require us to take expensive and time-consuming legal action to resolve, and, even if we are successful, may delay our ability to commercialize products and generate revenue. Further, if we are unable to resolve license issues that arise, we may lose rights to practice intellectual property that is required to make, use or sell products. We may require additional licenses in the future. Licenses to additional third-party intellectual property and materials that may be required for our development programs may not be available on commercially reasonable terms, or at all. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more-established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us.

In addition, disputes may also arise between us and our licensors regarding intellectual property subject to a license agreement, including disputes concerning scope of rights granted under the license agreement and other interpretation-related issues; whether and the extent to which our product candidates and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement; and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners. If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates. If we are unable to successfully obtain rights to required third-party intellectual property rights, we may have to abandon development of the relevant program or product candidate or expend time and resources re-designing the program or product candidate, which could have a material adverse effect on our business.

Intellectual property rights that we in-license in the future may also be granted through sublicenses under intellectual property owned by third parties, in some cases through multiple tiers. The actions of our licensors may therefore affect our rights to use our sublicensed intellectual property, even if we are in compliance with all of the obligations under our license agreements. Should our licensors or any of the upstream licensors fail to comply with their obligations under the agreements pursuant to which they obtain the rights that are sublicensed to us, or should such agreements be terminated or amended, our ability to develop and commercialize our product candidates may be materially harmed.

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of any future patents we obtain.

Our ability to obtain patents is highly uncertain because, to date, some legal principles remain unresolved, and there has not been a consistent policy regarding the breadth or interpretation of claims allowed in patents in the U.S. Furthermore, the specific content of patents and patent applications that are necessary to support and interpret patent claims is highly uncertain due to the complex nature of the relevant legal, scientific, and factual issues. Changes in either patent laws or interpretations of patent laws in the U.S. and other countries may diminish the value of our intellectual property or narrow the scope of our patent protection.

Patent reform legislation in the U.S. and other countries, including the Leahy-Smith America Invents Act (the “Leahy-Smith Act”) signed into law on September 16, 2011, and its implementation could increase the uncertainties around patent protection, costs, and the enforcement or defense of our patents, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects. The Leahy-Smith Act included a number of significant changes to U.S. patent law. Such provisions affect the way patent applications are prosecuted, redefine prior art, and provide more efficient and cost-effective avenues for competitors to challenge the validity of

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patents. In addition, the Leahy-Smith Act has transformed the U.S. into a "first-to-file" system for deciding which party should be granted a patent when two or more patent applications are filed by different parties claiming the same invention. The first-to-file provisions, however, only became effective on March 16, 2013. Accordingly, it is not yet clear what, if any, impact the Leahy-Smith Act will have on the operation of our business.

Accordingly, it is not yet clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. The Leahy-Smith Act and its implementation could make it more difficult to obtain patent protection for our inventions and increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could harm our business, results of operations and financial condition. Further, recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our or our licensors' ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on actions by the U.S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our or our licensors' existing patents and patents that we or our licensors might obtain in the future. We cannot predict how future decisions by the courts, Congress or the USPTO may impact the value of our or our licensors' patents. Similarly, any adverse changes in the patent laws of other jurisdictions could have a material adverse effect on our business and financial condition. Changes in the laws and regulations governing patents in other jurisdictions could similarly have an adverse effect on our ability to obtain and effectively enforce our patent rights.

We may be involved in lawsuits to protect or enforce our patents, which could be expensive, time consuming and unsuccessful.

While we are not currently involved in any disputes relating to our intellectual property, competitors may infringe the patents we have applied for. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. If we initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product or product candidate is invalid and/or unenforceable. In patent litigation in the U.S., counterclaims alleging invalidity and/or unenforceability are common, and there are numerous grounds upon which a third party can assert invalidity or unenforceability of a patent.

In an infringement proceeding, a court may decide that the patent claims we are asserting are invalid and/or unenforceable, or may refuse to stop the other party from using the intellectual property at issue on the grounds that our patent claims do not cover the intellectual property in question. Third parties may also raise similar claims before administrative bodies in the U.S. or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, *inter partes* review and equivalent proceedings in foreign jurisdictions (for example, opposition proceedings). Such proceedings could result in revocation of or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of infringement, invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our patent counsel, and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly, could put our patent applications at risk of not issuing and could have a material adverse impact on our business.

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Our defense of litigation or interference proceedings may fail and require us to cease using certain intellectual property or force us to take a license under the intellectual property rights of the prevailing party, if available. Even if successful, litigation or interference proceedings may result in substantial costs and distract our management and other employees. We may not be able to prevent misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the U.S.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain.

While no third parties to our knowledge have initiated legal proceedings against us to date, as our current and future product candidates progress toward commercialization, the possibility of a patent infringement claim against us increases. We cannot provide any assurance that our current and future product candidates do not infringe other parties' patents or other proprietary rights, and competitors or other parties may assert that we infringe their proprietary rights in any event. We may become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our current and future product candidates, including infringement, interference or derivation proceedings, post-grant review and *inter partes* review before the USPTO or similar adversarial proceedings or litigation in other jurisdictions. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could have a negative impact on our ability to commercialize our product candidates or any future product candidates. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is high and requires us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would agree with us and invalidate the claims of any such U.S. patent. Similarly, the burdens on us to invalidate patent claims in foreign jurisdiction may vary substantially and courts in those jurisdictions may not agree with us that the claims are invalid. The outcome of proceedings involving assertions of infringement, invalidity and unenforceability during patent litigation is unpredictable. Furthermore, if a patent holder believes that one of our product candidates infringes its patent, the patent holder may sue us even if we have received patent protection for our intellectual property. Moreover, we may face patent infringement claims from non-practicing entities that have no relevant revenue and against whom our own patent portfolio may thus have no deterrent effect. If a patent infringement suit were threatened or brought against us, we could be forced to stop or delay manufacturing or sales of the drug or product candidate that is the subject of the actual or threatened suit. Moreover, given the vast number of patents in our field of intellectual property, we cannot be certain that our current and future product candidates do not or will not infringe existing patents or that we will not infringe patents that may be granted in the future.

If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such third party to continue commercializing our product candidates. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if a license can be obtained on acceptable terms, the rights may be non-exclusive, which could give our competitors access to the same intellectual property rights licensed to us. If we fail to obtain a required license, we may be unable to effectively market product candidates based on our intellectual property, which could limit our ability to generate revenue or achieve profitability and possibly prevent us from generating revenue sufficient to sustain our operations. Alternatively, we may need to redesign our

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products, which may be impossible or require substantial time and monetary expenditure. Under certain circumstances, we could be forced, including by court orders, to cease commercializing our product candidates. In addition, in any such proceeding or litigation, we could be found liable for substantial monetary damages, potentially including treble damages and attorneys' fees, if we are found to have willfully infringed the patent at issue. A finding of infringement that prevents us from commercializing our product candidates, requires us to redesign our products, or forces us to cease some of our business operations could materially harm our business.

The cost to us in defending or initiating any litigation or other proceeding relating to patent or other proprietary rights, even if resolved in our favor, could be substantial, and litigation would divert our management's attention. Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could delay our research and development efforts and limit our ability to continue our operations.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information or trade secrets of third parties.

We employ individuals who were previously employed at other biotechnology or biopharmaceutical companies. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information, trade secrets or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed confidential information of our employees' former employers or other third parties. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and even if we are successful, litigation could result in substantial cost and be a distraction to our management and other employees.

We may be subject to claims challenging the inventorship or ownership of our intellectual property, including any patents we obtain.

We or our licensors may be subject to claims that former employees, collaborators or other third parties have an ownership interest in our patent applications, any patents we obtain, or other intellectual property. We may be subject to ownership disputes in the future arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates. Although it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own, and we cannot be certain that our agreements with such parties will be upheld in the face of a potential challenge, or that they will not be breached, for which we may not have an adequate remedy. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. If we no longer own intellectual property rights that are required to commercialize and protect our products, we may need to obtain license to those rights, which may not be available on commercially reasonable terms, or at all. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

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Reliance on third parties requires us to share trade secrets, which increases the possibility that trade secrets will be misappropriated or disclosed, and confidentiality agreements with employees and third parties may not adequately prevent disclosure of trade secrets and protect other proprietary information.

We rely on certain third-party trade secrets, technical know-how, proprietary information and other confidential information to protect our product candidates and as otherwise useful to our business. Monitoring unauthorized uses and disclosures of trade secrets and other confidential information is difficult, and we do not know whether the steps we have taken to protect our confidential intellectual property will be effective. We seek to protect our confidential information, in part, through confidentiality and non-disclosure agreements with our employees, consultants, collaborators, suppliers and other parties. These agreements typically restrict the ability of our employees, consultants, advisors and third-party contractors to use or disclose our proprietary information or publish data potentially relating to our proprietary information. Despite our efforts to protect trade secrets, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other proprietary information by the parties to these agreements. There can be no assurance that these agreements will not be breached, including by disclosure of our confidential information. If any of the collaborators, scientific advisors, employees, contractors and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation, and trade secret status could be lost as a result. We also cannot guarantee that we have entered into such agreements with each party that may have or have had access to our confidential information or proprietary product candidates and processes. Moreover, if confidential information that is licensed or disclosed to us by our partners, collaborators or others is inadvertently disclosed or subject to a breach or violation, we may be liable to the owner of that confidential information. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary product candidates and processes will be effective.

If we rely on third parties to manufacture or commercialize our product candidates, or if we collaborate with additional third parties for the development of our product candidates, we must, at times, share proprietary information with them. We may also conduct joint research and development programs that may require us to share potential trade secrets under the terms of our research and development partnerships or similar agreements. Despite the contractual provisions employed when working with third parties, the need to share confidential information increases the risk that such potential trade secrets become known by our competitors, are inadvertently incorporated into the product candidates of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and other confidential information, a competitor's discovery of such information or other unauthorized use or disclosure thereof could have an adverse effect on our business and results of operations.

Enforcing a claim that a third party illegally obtained and is using trade secrets or proprietary information is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the U.S. are sometimes less willing to protect trade secrets and the enforceability of confidentiality or similar types of agreements may vary from jurisdiction to jurisdiction.

We may enjoy only limited geographical protection with respect to certain patents and we may not be able to protect our intellectual property rights throughout the world.

Filing and prosecuting patent applications and defending patents covering our product candidates in all countries throughout the world would be prohibitively expensive. Competitors may use our intellectual property in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement rights are not as strong as that in the U.S. or Europe. These products may

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compete with our product candidates, and our and our licensors' future patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

In addition, we may decide to abandon national and regional patent applications before they are granted. The examination of each national or regional patent application is an independent proceeding. As a result, patent applications in the same family may issue as patents in some jurisdictions, such as in the U.S., but may issue as patents with claims of different scope or may even be refused in other jurisdictions. Furthermore, the requirements for patentability differ in certain jurisdictions and countries. Some countries do not grant claims directed to methods of treatment or have additional restrictions on the scope of method of treatment claims compared to the U.S. Accordingly, depending on the country, the scope of patent protection may vary for the same product candidate.

While we intend to protect our intellectual property rights in our expected significant markets, we cannot ensure that we will be able to initiate or maintain protection efforts in all such markets. Additionally, the prosecution of patent applications in other jurisdictions is often a longer process and patents may be granted at a later date than in the U.S., potentially delaying our ability to assert such patents against competitors. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate, which may have an adverse effect on our ability to successfully commercialize our product candidates in all of our expected significant foreign markets. If we encounter difficulties in protecting, or are otherwise precluded from effectively protecting, the intellectual property rights important for our business in such jurisdictions, the value of these rights may be diminished, and we may face additional competition in those jurisdictions.

The laws of some jurisdictions do not protect intellectual property rights to the same extent as the laws or rules and regulations in the U.S. and Europe, and many companies have encountered significant difficulties in protecting and defending such rights in such jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property rights, which could make it difficult for us to stop the infringement of any patents we obtain or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in other jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and attention from other aspects of our business, could put any patents we obtain at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing as patents, and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Some countries also have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, some countries limit the enforceability of patents against government agencies or government contractors. In those countries, the patent owner may have limited remedies, which could materially diminish the value of such patents. If we are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired.

In Europe, a new unitary patent system took effect on June 1, 2023, which may significantly impact European patents, including those granted before the introduction of the new system. Under the new system, applicants can, upon grant of a patent, opt for that patent to become a unitary patent which will be subject to the jurisdiction of a new unitary patent court ("UPC"). Patents granted before the implementation of the new system can be opted out of UPC jurisdiction, remaining as national patents in the UPC countries. Patents that remain under the jurisdiction of the UPC may be challenged in a single UPC-based revocation proceeding that, if successful, could invalidate the patent in all

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countries who are signatories to the UPC. Further, because the UPC is a new court system and there is no precedent for the court's laws, there is increased uncertainty regarding the outcome of any patent litigation. We are unable to predict what impact the new patent regime may have on our ability to exclude competitors in the European market. In addition to changes in patents laws, geopolitical dynamics, such as Russia's incursion into Ukraine, may also impact our ability to obtain and enforce patents in particular jurisdictions. If we are unable to obtain and enforce patents as needed in particular markets, our ability to exclude competitors in those markets may be reduced.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment, and other requirements imposed by government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the USPTO and various government patent agencies outside of the U.S. over the lifetime of our patents and/or applications and any patent rights we may obtain or license in the future. Furthermore, the USPTO and various non-U.S. government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. In many cases, an inadvertent lapse of a patent or patent application can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment or lapse of the patents or patent applications, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents within prescribed time limits. If we or our licensors fail to maintain the patents and patent applications covering our product candidates or if we or our licensors otherwise allow our patents or patent applications to be abandoned or lapse, our competitors might be able to enter the market, which would hurt our competitive position and could impair our ability to successfully commercialize our product candidates in any indication for which they are approved.

Any trademarks we have obtained or may obtain may be infringed or otherwise violated, or successfully challenged. If our trademarks and trade names are not adequately protected, or if we are unable to obtain desired trademarks or trade names, then we may not be able to build brand name recognition in our markets of interest and our business may be adversely affected.

We expect to rely on trademarks as one means to distinguish our product candidates, if approved for marketing, from the drugs of our competitors. Once we select new trademarks and apply to register them, our trademark applications may not be approved. During trademark registration proceedings in the U.S. and foreign jurisdictions, we may receive rejections. We are given an opportunity to respond to those rejections, but we may not be able to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties may oppose pending trademark registration applications or seek to cancel registered trademarks.

We have also not yet registered trademarks for any of our product candidates in any jurisdiction. Any trademark applications we file may be rejected and registered trademarks may not be obtained, maintained or enforced. If we do not successfully register our trademarks, we may encounter difficulty in enforcing, or be unable to enforce, our trademark rights against third parties, which could adversely affect our business and our ability to effectively compete in the marketplace.

In addition, any proprietary name we propose to use with any of our product candidate in the U.S. will need to be approved by the FDA, regardless of whether we have registered, or applied to register,

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the proposed proprietary name as a trademark. The FDA conducts a review of proposed proprietary names, including an evaluation of potential for confusion with other products' proprietary names, as part of the NDA review process. If the FDA objects to any of our proposed proprietary product names, we may be required to expend significant additional resources in an effort to identify a suitable proprietary name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA.

In addition, our unregistered trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on, misappropriating or violating other marks. In the USPTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademark registrations may not survive such proceedings. In the event that our trademarks are successfully challenged, we could be forced to rebrand our drugs, which could result in loss of brand recognition and could require us to devote resources to advertising and marketing new brands. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion.

Our competitors may also infringe or otherwise violate our trademarks and we may not have adequate resources to enforce our trademarks. We may not be able to protect our rights to our trademarks and trade names, which we need to build name recognition among potential collaborators or customers in our markets of interest. Any of the foregoing events may have a material adverse effect on our business.

Furthermore, in many countries, owning and maintaining a trademark registration may not provide an adequate defense against a subsequent infringement claim asserted by the owner of a senior trademark. Over the long term, if we are unable to successfully register our trademarks and trade names and establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively, and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade names, domain names or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely impact our financial condition or results of operations.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. The following examples are illustrative:

- others may be able to make products that are similar to or otherwise competitive with our product candidates but that are not covered by the claims of our current or future patents;
- an in-license necessary for the manufacture, use, sale, offer for sale or importation of one or more of our product candidates may be terminated by the licensor;
- we or future collaborators might not have been the first to conceive and reduce to practice the inventions covered by the patents or patent applications that we own, license or will own or license;
- we or future collaborators might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative product candidates or duplicate any of our product candidates without infringing our intellectual property rights;

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- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own or in-license may be held invalid or unenforceable as a result of legal challenges by our competitors;
- issued patents that we own or in-license may not provide coverage for all aspects of our product candidates in all countries;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- ownership of our patents or patent applications may be challenged by third parties;
- we may not develop additional proprietary intellectual property that is patentable; and
- the patents of third parties may have an adverse effect on our business.

Should any of these events occur, they could significantly harm our business, results of operations and prospects.

If we are unable to obtain licenses from third parties on commercially reasonable terms or fail to comply with our obligations under such agreements, our business could be harmed.

We currently have rights to intellectual property, through licenses from third parties, to identify and develop certain product candidates, and we may add additional licenses in the future as we expand our product candidate pipeline. Although we have succeeded in licensing intellectual property from third-party licensors, including AbbVie, in the past, we cannot assure our stockholders that we will be able to in-license or acquire the rights to any potential product candidates from third parties on acceptable terms or at all.

In addition, our license agreements may provide that our fields of use exclude particular fields. If we determine that rights to such fields are necessary to commercialize our product candidates or maintain our competitive advantage, we may need to obtain additional license rights in order to continue developing, manufacturing or marketing our product candidates. In addition, we may seek to obtain additional licenses from our licensors and, in connection with obtaining such licenses, we may agree to amend our existing licenses in a manner that may be more favorable to the licensors, including by agreeing to terms that could enable third parties (potentially including our competitors) to receive licenses to a portion of the intellectual property that is subject to our existing licenses.

Various third parties practice in competitive areas and may have issued patents or patent applications that will issue as patents in the future, which could impede or preclude our ability to commercialize our product candidates. For any third-party patents that could be relevant to our product candidates, we rely in part on the “safe harbor” or research exemption under 35 U.S.C. § 271(e)(1), which exempts activities related to pursuing FDA approval for a drug product from patent infringement. However, while U.S. patent law provides such a “safe harbor” to our clinical product candidates under this provision, that exemption may expire when an NDA is submitted. Given the uncertainty of clinical trials, we cannot be certain of the timing of their completion and it is possible that we may submit an NDA for one of our future product candidates at a time when one or more relevant third-party patents is in force. It may therefore be necessary for us to use the patented or proprietary intellectual property of third parties to commercialize our products, in which case we would be required to obtain a license from these third parties. If we are unable to license such intellectual property, or if we are forced to license such intellectual property on unfavorable terms, our business could be materially harmed. If we are unable to obtain a necessary license, we may be unable to develop or commercialize the affected product candidates, which could materially harm our business and the third parties owning such

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intellectual property rights could seek either an injunction prohibiting our sales or an obligation on our part to pay royalties and/or other forms of compensation. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same intellectual property licensed to us.

Additionally, we may collaborate with academic institutions to accelerate our preclinical research or development under written agreements with these institutions. In certain cases, these institutions provide us with an option to negotiate a license to any of the institution's rights in intellectual property resulting from the collaboration. Even if we hold such an option, we may be unable to negotiate a license from the institution within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to others, potentially blocking our ability to pursue our program.

In addition, the licensing or acquisition of third-party intellectual property rights is a highly competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third party intellectual property rights on terms that would allow us to make an appropriate return on our investment or at all. If we are unable to successfully obtain rights to required third party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the applicable product candidate, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

If we are unable to obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may be required to expend significant time and resources to redesign our product candidates, or the methods for manufacturing them or to develop or license replacement intellectual property, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business, financial condition, results of operations, and prospects significantly.

Additionally, if we fail to comply with our obligations under license agreements, our counterparties may have the right to terminate these agreements, in which event we might not be able to develop, manufacture or market, or may be forced to cease developing, manufacturing or marketing, any product that is covered by these agreements or may face other penalties under such agreements. Such an occurrence could materially adversely affect the value of the product candidate being developed under any such agreement. Termination of these agreements or reduction or elimination of our rights under these agreements, or current and future restrictions on our ability to freely assign or sublicense our rights under such agreements when it is in the interest of our business to do so, may result in our having to negotiate new or reinstated agreements with less favorable terms, cause us to lose our rights under these agreements, including our rights to important intellectual property or impede, or delay or prohibit the further development or commercialization of one or more potential product candidates that rely on such agreements.

Risks Related to Legal and Regulatory Compliance Matters

Our business operations and our relationships with healthcare providers, third-party payors, patients and other parties in the healthcare industry are subject, directly or indirectly, to significant regulation under a broad range of healthcare laws, including fraud and abuse laws.

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Any action against us for violation of such laws could harm our reputation and require significant resources for defense. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

Pharmaceutical manufacturers, and the parties with which such manufacturers interact, are subject to extensive and complex regulation under a broad range of healthcare laws. Such laws, some of which will apply only if and when we have a marketed product, constrain our business operations, including the research and development, manufacturing, distribution, sales and promotion of our product candidates and products, if any are approved in the future, as well as educational and charitable activities. Arrangements with healthcare provider, third-party payors, patients and other parties in the healthcare industry professionals, which may have a significant impact on our business, are regulated by fraud and abuse and other healthcare laws. For more information, see the section titled "*Business—Government Regulation—Other U.S. Healthcare Laws and Compliance Requirements*."

Healthcare laws regulating our business activities are broad and any exceptions may be narrow. Requirements may differ across jurisdictions. There may be limited guidance on the interpretation of the laws and their application to our specific activities. Interpretations of these laws by government enforcement agencies and courts are evolving. Efforts to ensure that our business operations will comply with applicable healthcare laws and regulations will involve substantial costs. We will need to develop and implement robust compliance policies and processes to seek to prevent and detect non-compliance and update such policies and processes as our operations and government expectations evolve, which will involve substantial and ongoing costs, and even with such policies and processes we cannot be certain to prevent non-compliance.

Given the broad scope, limited guidance and evolving government interpretations, our business activities may nonetheless potentially be subject to challenge under these healthcare laws despite efforts to ensure compliance. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Such an action could also harm our reputation and adversely affect our business as a result. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant penalties, including, without limitation, civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from participating in federal and state funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, diminished profits and future earnings, reputational harm and the curtailment or restructuring of our operations, any of which could harm our business.

Even if we obtain regulatory approvals for our product candidates or any future product candidates, they will remain subject to ongoing regulatory oversight.

Even if we obtain any regulatory approvals for our product candidates or any future product candidates, such product candidates, once approved, will be subject to ongoing regulatory requirements applicable to manufacturing, labeling, packaging, storage, advertising, promoting, sampling, record-keeping and post-marketing activities, among other things. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with cGMP and GCP requirements for any clinical trials that we conduct post-approval. Manufacturers of approved products and their facilities are subject to continual review and periodic and unannounced inspections by the FDA and other regulatory authorities for compliance with cGMP regulations and standards. Any regulatory approvals that we receive for our product candidates

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or any future product candidates may also be subject to a REMS, limitations on the approved indicated uses for which the drug may be marketed or to the conditions of approval, or requirements that we conduct potentially costly post-marketing testing, including additional trials and heightened surveillance to monitor the quality, safety and efficacy of the drug. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of marketing approval. We will further be required to promptly report any serious and unexpected adverse drug experiences and certain quality or production problems with our products to regulatory authorities along with other periodic reports.

Any new legislation addressing drug safety issues could result in delays in product development or commercialization, or increased costs to ensure compliance. We will also have to comply with requirements concerning advertising and promotion for our products. Promotional communications with respect to prescription drug products are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved label. As such, we will not be allowed to promote our products for indications or uses for which they do not have approval, commonly known as off-label promotion. Physicians, on the other hand, may prescribe products for off-label uses. Although the FDA and other regulatory agencies do not regulate a physician's choice of drug treatment made in the physician's independent medical judgment, they do restrict promotional communications from companies, including their sales force, with respect to off-label uses of products for which marketing approval has not been issued. However, companies may share truthful and not misleading information that is otherwise consistent with a product's FDA approved labeling. The holder of an approved NDA must submit new or supplemental applications and obtain prior approval for certain changes to the approved product, product labeling, or manufacturing process. A company that is found to have improperly promoted off-label uses of their products may be subject to significant civil, criminal and administrative penalties.

In addition, drug manufacturers are subject to payment of annual fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP requirements and adherence to commitments made in the NDA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with any product, if approved, such as adverse experiences of unanticipated severity or frequency, or problems with the facility where the product is manufactured or if a regulatory authority disagrees with the promotion, marketing or labeling of that product, a regulatory authority may impose restrictions relative to that product, the manufacturing facility or us, including requesting revisions to the approved labeling to add new safety information, imposing of post-market studies or clinical trials to assess new safety risks or imposing distribution restrictions or other restrictions under a REMS program, requesting a recall or requiring withdrawal of the product from the market or suspension of manufacturing. If we fail to comply with applicable regulatory requirements following approval of any of our product candidates, a regulatory authority may:

- issue an untitled letter or warning letter asserting that we are in violation of the law;
- seek an injunction or impose administrative, civil or criminal penalties or monetary fines, disgorgement or profits or revenue, warning letters or adverse publicity requirements;
- suspend or withdraw regulatory approvals;
- restrict product distribution or use, including full or partial holds on any ongoing or planned clinical trials;
- refuse to approve a pending NDA or comparable foreign marketing application (or any supplements thereto) submitted by us or our strategic partners;
- restrict the marketing or manufacturing of the drug;
- seize or detain the drug or otherwise require the withdrawal of the drug from the market;

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- refuse to permit the import or export of product candidates; or
- refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates and harm our business, financial condition, results of operations and prospects.

Healthcare and other reform initiatives may have an adverse impact on our business and results of operations.

In the U.S. and some foreign jurisdictions, there have been and continue to be ongoing efforts to implement legislative and regulatory changes regarding the healthcare system. Such changes could prevent or delay marketing approval of any product candidates that we may develop; restrict or regulate post-approval activities; and affect our ability to profitably sell any product candidates for which we obtain marketing approval. Although we cannot predict what healthcare reform efforts will be successful, such efforts may result in more rigorous coverage criteria, additional downward pressure on the price that we, or our future collaborators, may receive for any approved products, or in other consequences that may adversely affect our ability to achieve or maintain profitability. For more information, see the section titled "*Business—Government Regulation—Healthcare Reform*."

Among policy makers and third-party payors in the U.S. and elsewhere, there is significant and ongoing interest in implementing changes in the delivery and payment for healthcare services in order to contain healthcare costs, improve quality and/or expand access. In the U.S., the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives and changing policies and practices of the third-party payors. There has also been heightened governmental scrutiny in the U.S. of pharmaceutical pricing practices considering the rising cost of prescription drugs and biologics. Such scrutiny has resulted in several congressional inquiries and proposed and enacted legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient support programs, or reform government program reimbursement methodologies for products. As an example, the Inflation Reduction Act of 2022 ("IRA") includes a number of changes intended to address rising prescription drug prices in Medicare Parts B and D. These changes, which have varying implementation dates, include caps on Medicare Part D out-of-pocket costs for beneficiaries, Medicare Part B and Part D drug price inflation rebates, a new Medicare Part D manufacturer discount drug program (replacing the ACA Medicare Part D coverage gap discount program) and a drug price negotiation program for certain high spend Medicare Part B and D drugs (with the first set of negotiated prices going into effect January 1, 2026). The IRA drug price negotiation program has been challenged in litigation filed by various pharmaceutical manufacturers and industry groups, the outcome of which is unknown. Individual states in the U.S. have also become increasingly active in implementing regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Adoption of new legislation at the federal or state level could affect demand for, or pricing of, any future products if approved for sale. We cannot, however, predict the ultimate content, timing or effect of any federal and state reform efforts. There is no assurance that federal or state healthcare reform will not adversely affect our future business and financial results. Additionally, implementation by third party payors of policies and practices to limit coverage, manage utilization, reduce payment of drug products could adversely affect our ability to sell our products profitably. All these efforts may prevent us from being able to generate revenue, attain profitability or commercialize our drugs.

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In addition, FDA regulations and guidance may be revised or reinterpreted by the FDA in ways that may significantly affect our business and our products. If executive actions impose restrictions on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted. Any new regulations or guidance, or revisions or reinterpretations of existing regulations or guidance, may impose additional costs or lengthen FDA review times for our product candidates. We cannot determine how changes in regulations, statutes, policies or interpretations when and if issued, enacted or adopted, may affect our business in the future. Such changes could, among other things, require:

- significant changes to the design of planned clinical trials that impact their duration or cost;
- additional clinical trials to be conducted prior to obtaining approval;
- changes to manufacturing methods;
- recalls, replacements, or discontinuance of one or more of our products, if approved; and
- additional recordkeeping.

Such changes would likely require substantial time and impose significant costs, or could reduce the potential commercial value of our product candidates, and could materially harm our business and our financial results. In addition, delays in receipt of or failure to receive regulatory approvals for any of our product candidates would harm our business, financial condition, and results of operations. Further, we cannot predict the likelihood, nature, or extent of healthcare reform initiatives that may arise from future legislation or administrative action.

General legislative cost control measures may also affect reimbursement for our product candidates. The Budget Control Act, as amended, resulted in the imposition of reductions in Medicare (but not Medicaid) payments to providers in 2013 and will remain in effect into 2032 unless additional Congressional action is taken. Any significant spending reductions affecting Medicare, Medicaid or other publicly funded or subsidized health programs that may be implemented and/or any significant taxes or fees that may be imposed on us could have an adverse impact on our results of operations.

Risks Related to the Commercialization of Our Product Candidates

We face substantial competition. Our main competitor in the CF market holds substantial market share and has substantially greater resources than we do. We may not be able to compete successfully in this environment and, in particular, against a much larger competitor.

The biotechnology and pharmaceutical industries are characterized by rapid advances, intense competition and a strong emphasis on proprietary and novel products and product candidates. We face substantial competition with respect to our current product candidates and will face competition with respect to any product candidates that we may seek to develop or commercialize in the future, from many different sources, including major pharmaceutical and biotechnology companies, academic institutions, governmental agencies, consortiums and public and private research institutions.

In particular, we expect to compete with Vertex, which has multiple approved products, as well as additional product candidates in development, for the treatment of CF that would compete with our product candidates, if approved. Vertex holds substantial market share in our product candidates' proposed markets, and has substantially greater name recognition, financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Our commercial opportunity could be reduced or eliminated if Vertex or another competitor develops and commercializes products that are safer or more effective, have fewer or less severe side effects, or are more convenient or are less expensive than any product candidate that we may develop. Vertex or another competitor may also

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obtain approval by FDA or comparable foreign regulatory authorities for its product candidates currently in development more rapidly than we may obtain approval for our product candidates. Competing products could present superior treatment alternatives and could render our product candidates obsolete or noncompetitive before we recover the expense of developing and commercializing our product candidates. Even if one or more of our product candidates achieves marketing approval, it may be priced at a significant premium over competitive products, resulting in reduced competitiveness. If we do not compete successfully, we may not generate or derive sufficient revenue from any product candidate for which we obtain marketing approval and may not become or remain profitable.

Smaller or early-stage companies could also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. This may include other small-molecule drug discovery companies using similar approaches or other types of therapies, such as small molecule, gene therapy, gene editing and/or mRNA therapies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring intellectual property complementary to, or that may be necessary for, our programs. If we are unable to compete effectively, our opportunity to generate revenue from the sale of our products we may develop, if approved, could be adversely affected.

Even if any of our product candidates receive marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

If any of our product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. There is currently a well-established standard of care for CF, Trikafta, with which physicians, CF patients and payors are very familiar and for which an established benefit-risk profile exists. In addition, in December 2024, the FDA approved Alyftrek, which targets the same mechanisms as Trikafta. Even if our product candidates are successful in registrational clinical trials, they may not be successful in displacing the current standard of care if we are unable to demonstrate competitive efficacy, safety, ease of administration and/or cost-effectiveness. For example, physicians may be reluctant or unwilling to take their patients off their current medication and switch their treatment regimen to our product candidates, if approved, if the current medication is effective. Further, patients often acclimate to the treatment regimen that they are currently taking and may not want to switch unless recommended to do so by their physician for clinical reasons or required to do so due to lack of coverage and adequate reimbursement. Even if we are able to demonstrate our product candidates' safety and efficacy to the FDA and other regulators and receive approval, concerns in the medical community related to the comparative efficacy or side effect profile of our products may hinder market acceptance and uptake.

Efforts to educate the medical community and third-party payors regarding the benefits of our product candidates, if approved, may require significant resources, including management time and financial resources, and may not be successful. If our product candidates do not achieve an adequate level of market acceptance, we may not generate significant revenue and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy, safety and potential advantages compared to alternative treatments;
- whether a product candidate is approved, if ever, as an add-on to standard of care or as part of a proprietary combination therapy;
- the prevalence and severity of any side effects;

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- our ability to offer our products at competitive prices;
- the convenience and ease of administration compared to alternative treatments;
- product labeling or product insert requirements of the FDA or comparable foreign regulatory authorities, including any limitations or warnings contained in a product's approved labeling, including any boxed warning;
- the product's acceptance into current standard of care treatment algorithms by medical societies that could affect payor and physician uptake;
- the effectiveness of sales and marketing efforts, and the strength of sales, marketing and distribution support;
- the availability of third-party coverage and adequate reimbursement for any product candidates, once approved;
- the willingness of the target patient population to try, and of physicians to prescribe, the product;
- any restrictions on the use of our products together with other medications; and
- potential product liability claims and unfavorable publicity related to our products.

Any failure by one or more of our product candidates that obtains regulatory approvals to achieve market acceptance or commercial success would adversely affect our business prospects.

Due to the significant resources required for the development of our pipeline, and depending on our ability to access capital, we must prioritize the development of certain product candidates over others. Moreover, we may fail to expend our limited resources on product candidates or indications that may have been more profitable or for which there is a greater likelihood of success.

Due to the significant resources required for the development of our product candidates, we must decide which product candidates and indications to pursue and advance and the amount of resources to allocate to each. Our decisions concerning the allocation of research, development, collaboration, management and financial resources toward particular product candidates, therapeutic areas or indications may not lead to the development of viable commercial products and may divert resources away from better opportunities. For example, we intend to select a lead NBD1 stabilizer product candidate after the completion of our ongoing Phase 1 clinical trials of SION-719 and SION-451, and a lead complementary modulator product candidate from our two most advanced modulator product candidates, galicaftor and SION-109. If we make incorrect determinations regarding the viability or market potential of either lead product candidate, or any of our other current or future product candidates, or misread trends in the pharmaceutical industry, our business, financial condition and results of operations could be materially and adversely affected. As a result, we may fail to capitalize on viable commercial products or profitable market opportunities, be required to forego or delay pursuit of opportunities with other product candidates or other diseases and disease pathways that may later prove to have greater commercial potential than those we choose to pursue, or relinquish valuable rights to such product candidates through collaboration, licensing or royalty arrangements in cases in which it would have been advantageous for us to invest additional resources to retain sole development and commercialization rights.

The success of our product candidates or any future product candidate will depend significantly on coverage and adequate reimbursement by third party payors or the willingness of patients to pay for these products if not covered.

We believe that for any product candidates which may be approved, our success depends on obtaining and maintaining coverage and adequate reimbursement for such products for their respective

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approved indications, and the extent to which patients will be willing to pay out-of-pocket for such products in the absence of reimbursement for all or part of the cost. Accordingly, we will need to establish a coverage and reimbursement strategy for any approved product candidate. In the U.S. and markets in other countries, patients generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Adequate coverage and reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors is critical to new product acceptance. Government and private third-party payors decide which products they will cover and establish reimbursement levels. Coverage and reimbursement varies among third party payors and new products face significant challenges in obtaining and maintaining coverage and adequate reimbursement, particularly if approved for indications with established treatments already on the market. For more information, see the section titled "*Business—Government Regulation—Coverage and Reimbursement*."

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage for certain products, managing utilization of covered products and restricting the amount of reimbursement for covered products. Within the U.S., net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or requested by private payors in exchange for favorable coverage. Our inability to promptly obtain coverage and adequate reimbursement rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

There can be no assurance that our product candidates, even if they are approved for sale in the U.S. or in other countries, will be considered medically reasonable and necessary for a specific indication or cost-effective by third-party payors, or that coverage and an adequate level of reimbursement will be available or that third-party payors' reimbursement policies will not adversely affect our ability to sell our product candidates profitably.

The market for our product candidates may be smaller than we estimate.

Our estimates of the potential market opportunity for our product candidates include several key assumptions, based on our industry knowledge, industry publications and third-party research reports. These assumptions include the number of patients who have CF, as well as the estimated reimbursement levels for each product candidate, if approved. While we believe our assumptions and the data underlying our estimates are reasonable, we have not independently verified the accuracy of the third-party data on which we have based our assumptions and estimates, and these assumptions and estimates may not be correct and the conditions supporting our assumptions or estimates may change at any time, including as a result of factors outside our control, thereby reducing the predictive accuracy of these underlying factors. Further, new studies may change the estimated incidence or prevalence of these diseases, and the potentially addressable patient population for our product candidates may not ultimately be amenable to treatment with our product candidates. If the actual market for any product candidates we may develop is smaller than we estimate, our revenues, if any, may be limited and it may be more difficult for us to achieve or maintain profitability.

Clinical trial and product liability lawsuits against us could divert our resources, cause us to incur substantial liabilities and limit commercialization of any products that we may develop.

We face an inherent risk of clinical trial and product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk if we commercially sell any products that we may develop, especially if our products are prescribed for off-label uses (even if we do not promote such uses). For example, we may be sued if our product candidates allegedly cause injury or are found to be otherwise unsuitable during product testing, manufacturing, marketing or sale.

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Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product candidate, negligence, strict liability and a breach of warranties. Claims may be brought against us by clinical trial participants, patients or others using, administering or selling products that may be approved in the future. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against claims that our product candidates caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates that we may develop;
- termination of clinical trials;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards paid to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of revenue;
- reduced resources of our management to pursue our business strategy;
- the inability to commercialize any products that we may develop; and
- a decline in our stock price.

Although we maintain clinical trial liability insurance coverage, such insurance may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we expand our clinical trials. We intend to expand our insurance coverage for products to include the sale of commercial products if we obtain marketing approval on any current or potential product candidates, but we may be unable to obtain commercially reasonable product liability insurance for any products approved for marketing. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

Risks Related to Our Business Operations, Employee Matters and Managing Our Growth

Our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel.

Our success depends in part on our continued ability to attract, retain and motivate highly qualified management, clinical and scientific personnel. Each of our executive officers may currently terminate their employment with us at any time. We do not maintain “key person” insurance for any of our executives or employees. This lack of insurance means that we may not have adequate compensation for the loss of the services of these individuals.

The loss of the services of our executive officers or other key employees could impede the achievement of our development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approvals of and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar

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personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

Further, job candidates and existing employees often consider the value of the stock awards they receive in connection with their employment. If the perceived benefits of our stock awards decline, either because we are a public company or for other reasons, it may harm our ability to recruit and retain highly skilled employees. Our employees may be more likely to leave us if the shares they own have significantly appreciated in value relative to the original purchase prices of the shares, or if the exercise prices of the options that they hold are significantly below the market price of our common stock, particularly after the expiration of the lock-up agreements described herein.

We expect to expand our clinical development and regulatory capabilities and potentially implement sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

As of December 31, 2023, we had 28 full-time employees, including 18 who were engaged in research and development activities. As we continue to build our organization and execute on our strategy, we expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of clinical development, regulatory affairs and, if any of our product candidates receives marketing approval, sales, marketing and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management, business, and development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations. Our future financial performance and our ability to compete effectively will depend, in part, on our ability to manage our growth effectively.

Our business could be affected by litigation, government investigations and enforcement actions.

We currently operate and plan to operate in a highly regulated industry and we could now or in the future be subject to litigation, government investigation and enforcement actions on a variety of matters in the U.S. or foreign jurisdictions, including, without limitation, intellectual property, regulatory, product liability, environmental, whistleblower, false claims, privacy, anti-kickback, anti-bribery, securities, commercial, employment and other claims and legal proceedings which may arise from conducting our business. Any determination that our operations or activities are not in compliance with existing laws or regulations could result in the imposition of fines, civil and criminal penalties, equitable remedies, including disgorgement, injunctive relief and/or other sanctions against us, and remediation of any such findings could have an adverse effect on our business operations.

Legal proceedings, government investigations and enforcement actions can be expensive and time-consuming. An adverse outcome resulting from any such proceedings, investigations or enforcement actions could result in significant damages awards, fines, penalties, exclusion from the federal healthcare programs, healthcare debarment, injunctive relief, product recalls, reputational damage and modifications of our business practices, which could have a material adverse effect on our

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business and results of operations. Even if such a proceeding, investigation or enforcement action is ultimately decided in our favor, the investigation and defense thereof could require substantial financial and management resources and cause reputational harm.

Inadequate funding for the FDA, the Securities and Exchange Commission and other government agencies, including from government shut downs, or other disruptions to these agencies' operations, could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory, and policy changes. In addition, government funding of the Securities and Exchange Commission (the "SEC") and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical employees and stop critical activities. Currently, federal agencies in the U.S. are operating under a continuing resolution that is set to expire on March 14, 2025. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

Our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs, suppliers and vendors may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements.

Our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs, suppliers and vendors may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violates FDA or other comparable regulatory authority regulations, including those laws requiring the reporting of true, complete and accurate information to the FDA or other comparable regulatory authority, manufacturing standards, foreign, federal and state healthcare laws and regulations, and laws that require the true, complete and accurate reporting of financial information or data.

In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud and abuse, such as the payment of kickbacks in return for business. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Misconduct by these parties could also involve the improper use or misrepresentation of individually identifiable information, including, without limitation, information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have adopted a code of business conduct and ethics, but it is not always possible to identify and deter misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws

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or regulations. In addition, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, including, without limitation, damages, fines, disgorgement, imprisonment, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, imprisonment, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations. Any of these could adversely affect our ability to operate our business and our results of operations.

If our third-party manufacturers or suppliers do not comply with laws regulating the protection of the environment and health and human safety, our business could be affected adversely.

We and any CDMOs and suppliers we engage are subject to numerous federal, state and local environmental, health, and safety laws, regulations and permitting requirements, including those governing laboratory procedures; the generation, handling, use, storage, treatment and disposal of hazardous and regulated materials and wastes; the emission and discharge of hazardous materials into the ground, air and water; and employee health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from the use of hazardous materials by us or by one of our third party-manufacturers, we could be held liable for any resulting damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended. We and our third-party manufacturers and suppliers cannot eliminate the risk of accidental injury or contamination from these materials or wastes. Although we maintain general liability insurance as well as workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities.

In addition, we and our third-party manufacturers and suppliers may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations, which may increase the cost of their services to us. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions or liabilities for us or our third-party manufacturers and suppliers, or adversely impact our supply chain or reputation, which could in turn materially adversely affect our business, financial condition, results of operations and prospects.

Our insurance policies are expensive and only protect us from some business risks, which will leave us exposed to significant uninsured liabilities.

We do not carry insurance for all categories of risk that our business may encounter. Some of the policies we currently maintain include property, general liability, employment directors' and officers' and employment practices insurance, however, we may not be able to maintain insurance adequate levels of coverage in the future. Further, an insurance carrier may seek to cancel or deny coverage after a claim has occurred.

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We may engage in strategic transactions that could increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities, subject us to other risks, adversely affect our liquidity, increase our expenses, present significant distractions to our management and harm our financial condition and results of operations.

From time to time, we may consider strategic transactions, such as acquisitions of companies, asset purchases and strategic partnerships or out-licensing or in-licensing of intellectual property, product candidates or products. For example, we have entered into a license agreement with AbbVie that gives us exclusive worldwide rights to develop and commercialize three clinical-stage compounds. Additional potential transactions that we may consider in the future include a variety of business arrangements, including spin-offs, strategic partnerships, joint ventures, restructurings, divestitures, business combinations and investments. We may not be able to find suitable partners or acquisition candidates, and we may not be able to complete such transactions on favorable terms, if at all. Any future transactions could increase our near and long-term expenditures, result in potentially dilutive issuances of our equity securities, including our common stock, or the incurrence of debt, contingent liabilities, amortization expenses or acquired in-process research and development expenses, any of which could affect our financial condition, liquidity and results of operations. Future acquisitions may also require us to obtain additional financing, which may not be available on favorable terms or at all. These transactions may never be successful and may require significant time and attention of our management. In addition, the integration of any licensed assets that we may acquire rights to in the future may disrupt our existing business, may cause delays related to the integration of any licensed or acquired assets, and may be a complex, risky and costly endeavor for which we may never realize the full benefits. Furthermore, we may experience losses related to our entry into any licensing or partnerships, including as a result of failure to realize expected benefits or the materialization of unexpected liabilities or risks, which could have a material negative effect on our results of operations and financial condition. Accordingly, although there can be no assurance that we will undertake or successfully complete any additional transactions of the nature described above, any additional transactions that we do complete could have a material adverse effect on our business, results of operations, financial condition and prospects.

Business disruptions could seriously harm our future revenue and financial condition and increase our costs and expenses.

Our operations and the operations of our suppliers, CROs, CDMOs and clinical sites could be subject to earthquakes, power shortages, telecommunications or infrastructure failures, cybersecurity incidents, physical security breaches, water shortages, floods, hurricanes, typhoons, blizzards and other extreme weather conditions, fires, public health pandemics or epidemics and other natural or manmade disasters or business interruptions, for which we are predominantly self-insured. We rely or expect to rely on third-party manufacturers or suppliers to produce our product candidates and their components and on CROs and clinical sites to conduct our clinical trials, and do not currently have a redundant source of supply for all components of our product candidates. Our ability to obtain clinical or, if approved, commercial, supplies of our product candidates or any future product candidates could be disrupted if the operations of these suppliers were affected by a man-made or natural disaster or other business interruption, and our ability to commence, conduct or complete our clinical trials in a timely manner could be similarly adversely affected by any of the foregoing. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses.

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Risks Related to This Offering, Ownership of Our Common Stock and Our Status as a Public Company

There has been no prior public market for our common stock. An active trading market for our common stock may not develop or be sustained.

Prior to this offering, there has been no public market for our common stock. The initial public offering price for our common stock was determined through negotiations with the underwriters and may vary from the price of our common stock after the closing of this offering. An active trading market for our shares may never develop or, if it does develop, be sustained following this offering. If an active market for our common stock does not develop or is not sustained, the value of your shares may be impaired, and it may be difficult for you to sell shares you purchased in this offering at an attractive price or at all. An inactive market may also impair our ability to raise capital by selling shares, which in turn could materially adversely affect our business.

Our operating results may fluctuate significantly, which makes our future operating results difficult to predict and could cause our operating results to fall below expectations.

Our quarterly and annual operating results may fluctuate significantly, which makes it difficult for us to predict our future operating results. These fluctuations may occur due to a variety of factors, many of which are outside of our control, including:

- the timing and success or failure of clinical trials for our product candidates or competing product candidates or any other change in the competitive landscape of our industry;
- our ability to successfully recruit and retain subjects for clinical trials and any delays caused by difficulties in such efforts;
- the timing and cost of, and level of investment in, research, development, regulatory approvals and commercialization activities relating to our product candidates, which may change from time to time;
- the cost of manufacturing our product candidates, which may vary depending on the quantity of production and the terms of our agreements with manufacturers;
- expenditures that we may incur to acquire, develop or commercialize additional product candidates;
- the level of demand and the indication for any approved products, which may vary significantly;
- the risk/benefit profile, cost, coverage, and reimbursement policies with respect to our product candidates, if approved, and existing and potential future drugs that compete with our product candidates;
- the recruitment or departure of key personnel;
- changes in the structure of healthcare payment systems;
- the timing and amount of any milestone, royalty or other payments payable by us or due to us under any collaboration, licensing or other similar agreement; and
- general market and economic conditions, including market conditions in the pharmaceutical and biotechnology sectors.

The cumulative effects of these factors could result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, comparing our operating results on a period-to-period basis may not be meaningful. Investors should not rely on our past results as an indication of our future performance.

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This variability and unpredictability could also result in our failing to meet the expectations of industry or financial analysts or investors for any period. If our revenue or operating results fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if the forecasts we provide to the market are below the expectations of analysts or investors, the price of our common stock could decline substantially. Such a stock price decline could occur even if we have met any previously publicly stated revenue or earnings guidance.

The trading price of the shares of our common stock may be volatile, and purchasers of our common stock could lose all or part of their investment.

The market price for our stock is likely to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which we cannot control. The stock market in general and the market for biopharmaceutical companies in particular have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of particular companies. As a result of this volatility, investors may not be able to sell their common stock at or above the price paid for the shares. These factors include:

- the commencement, enrollment or results of our current or future clinical trials of our product candidates;
- adverse results from, delays in, suspension or termination of current or future clinical trials or preclinical studies of our product candidates, or any delay in advancing a clinical candidate;
- the success of competitive products gaining approvals or announcements by current and future competitors of their product development efforts;
- any delay in our regulatory filings for our product candidates or any other product candidate we may develop, and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such filings;
- adverse regulatory decisions, including the failure to authorize or approve the conduct of one or more clinical trials of our product candidates, the failure to receive regulatory approvals of our product candidates, or the failure of a regulatory authority to accept data from clinical trials conducted in other countries;
- the reporting of unfavorable preclinical or clinical results;
- our success or failure to identify, develop, acquire or license additional product candidates;
- the degree and rate of physician and market adoption of any of our current and future product candidates, if successfully developed and approved;
- manufacturing, supply or distribution delays or shortages, including our inability to obtain adequate supply of drug product, drug substance, raw materials or any commercially available product to be used in our clinical trials, at acceptable prices, or at all;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- unanticipated serious safety concerns related to the use of our product candidates or any other product candidate;
- our cash position, and any changes in financial estimates by us or by any equity research analysts who might cover our stock;
- changes in our capital structure, such as future issuances of securities and the incurrence of additional debt;
- conditions or trends in our industry;

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- investors' general perception of our company and our business;
- our ability to effectively manage our growth;
- overall performance of the equity markets;
- changes in the market valuations and stock market price and volume fluctuations of comparable companies and, in particular, those that operate in the biopharmaceutical industry;
- publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- announcements by us or our competitors of significant acquisitions, strategic collaborations, joint ventures, capital commitments or divestitures;
- third-party publications and discussions about our business on social media, forums and other websites;
- recruitment or departure of key personnel;
- trading volume of our common stock;
- sales of common stock by us or our stockholders in the future;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our product candidates;
- significant lawsuits, including patent or stockholder litigation;
- changes in the structure of healthcare payment systems;
- changes in accounting standards, policies, guidelines, interpretations or principles;
- regulatory or legal developments in the U.S. and foreign countries;
- general political and economic conditions; and
- other events or factors, many of which are beyond our control.

The stock market in general, and the market for biotechnology companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. The realization of any of the risks described in this section, or any of a broad range of other risks, could have a material adverse impact on the market price of our common stock. In the past, stockholders have initiated class action lawsuits against pharmaceutical and biotechnology companies following periods of volatility in the market prices of these companies' stock. Such litigation, if instituted, could result in substantial costs and divert management's attention and resources.

We may not be able to satisfy listing requirements of Nasdaq or obtain or maintain a listing of our common stock on Nasdaq.

If our common stock is listed on Nasdaq, we must meet certain financial and liquidity criteria to maintain such listing. If we violate Nasdaq's listing requirements, our common stock may be delisted. If we fail to meet any of Nasdaq's listing standards, our common stock may be delisted. In addition, our board of directors may determine that the cost of maintaining our listing on a national securities exchange outweighs the benefits of such listing. A delisting of our common stock from Nasdaq may materially impair our stockholders' ability to buy and sell our common stock and could have an adverse effect on the market price of, and the efficiency of the trading market for, our common stock. The delisting of our common stock could significantly impair our ability to raise capital and the value of your investment.

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If you purchase shares of our common stock in this offering, you will suffer immediate and substantial dilution of your investment.

The initial public offering price of our common stock is substantially higher than the net tangible book value per share of our common stock immediately after the completion of this offering. Therefore, if you purchase shares of our common stock in this offering, you will pay a price per share that substantially exceeds our pro forma as adjusted net tangible book value per share after this offering. Based on the initial public offering price of $18.00 per share, you will experience immediate dilution of $9.65 per share, representing the difference between our pro forma as adjusted net tangible book value per share as of September 30, 2024 and the initial public offering price. For a further description of the dilution that you will face immediately after this offering, see "*Dilution*."

A significant portion of our total outstanding shares are restricted from immediate resale but may be sold into the market in the near future. This could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. If our stockholders sell, or the market perceives that our stockholders intend to sell, substantial amounts of our common stock in the public market, the market price of our common stock could decline significantly and impair our ability to raise adequate capital through the sale of additional equity or equity-linked securities.

Upon the closing of this offering, we will have 42,520,700 shares of common stock outstanding (or 44,108,934 shares if the underwriters exercise their option to purchase additional shares from us in full), based on the number of shares outstanding as of September 30, 2024 and after giving effect to the automatic conversion of our redeemable convertible preferred stock outstanding immediately prior to this offering into 27,149,206 shares of our common stock. Of these, the shares sold in this offering will be freely tradable immediately after this offering and substantially all of the additional shares of common stock will be available for sale in the public market beginning 180 days after the date of this prospectus following the expiration of lock-up agreements between our directors, officers, substantially all of our stockholders and the underwriters. The foregoing agreements are subject to certain limited exceptions, and Goldman Sachs & Co. LLC and TD Securities (USA) LLC may release these stockholders from their lock-up agreements with the underwriters at any time and without notice, which would allow for earlier sales of shares in the public market. See "*Underwriting*."

In addition, promptly following the effectiveness of the registration statement of which this prospectus forms a part, we intend to file one or more registration statements on Form S-8 under the Securities Act of 1933, as amended (the "Securities Act"), registering the issuance of 9,150,462 shares of common stock subject to options or other equity awards issued or reserved for future issuance under our equity incentive plans. Shares registered under these registration statements on Form S-8 will be available for sale in the public market subject to vesting arrangements and exercise of options, the lock-up agreements described above and the restrictions of Rule 144 in the case of our affiliates.

Additionally, after this offering, the holders of an aggregate of 27,149,206 shares of our common stock, or their transferees, will have rights, subject to some conditions, to require us to file one or more registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. If we were to register the resale of these shares, they could be freely sold in the public market. If these additional shares are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

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Provisions in our corporate charter documents and under Delaware law may prevent or frustrate attempts by our stockholders to change our management and hinder efforts to acquire a controlling interest in us, and the market price of our common stock may be lower as a result.

Provisions in our amended and restated certificate of incorporation that will be in effect immediately prior to the consummation of this offering and amended and restated bylaws that became effective upon the effectiveness of this registration statement of which this prospectus forms a part may significantly reduce the value of our shares to a potential acquiror or make it difficult for a third party to acquire, or attempt to acquire, control of our company, even if a change of control was considered favorable by you and other stockholders. For example, our board of directors will have the authority to issue up to 10,000,000 shares of preferred stock and may fix the price, rights, preferences, privileges, and restrictions of the preferred stock without any further vote or action by our stockholders. The issuance of shares of preferred stock may delay or prevent a change of control transaction. As a result, the market price of our common stock and the voting and other rights of our stockholders may be adversely affected. An issuance of shares of preferred stock may result in the loss of voting control to other stockholders.

Our charter documents will also contain other provisions that could have an anti-takeover effect, including:

- only one of our three classes of directors will be elected each year;
- stockholders will not be entitled to remove directors other than by a two-thirds (2/3) vote and only for cause;
- stockholders will not be permitted to take actions by written consent;
- stockholders cannot call a special meeting of stockholders; and
- stockholders must give advance notice to nominate directors or submit proposals for consideration at stockholder meetings.

In addition, we are subject to the anti-takeover provisions of Section 203 of the Delaware General Corporation Law, which regulates corporate acquisitions by prohibiting Delaware corporations from engaging in specified business combinations with particular stockholders of those companies. These provisions could discourage potential acquisition proposals and could delay or prevent a change of control transaction. They could also have the effect of discouraging others from making tender offers for our common stock, including transactions that may be in your best interests. These provisions may also prevent changes in our management or limit the price that investors are willing to pay for our stock.

Concentration of ownership of our common stock among our existing executive officers, directors and principal stockholders may prevent new investors from influencing significant corporate decisions.

Following the completion of this offering, our executive officers, directors and current beneficial owners of 5% or more of our common stock and their respective affiliates will beneficially own approximately 62.9% of our outstanding common stock (assuming no exercise of the underwriters' option to purchase additional shares and no exercise of outstanding options and without giving effect to (i) any potential purchases by such persons in this offering or (ii) issuance of options to be granted to certain of our employees and non-employee directors upon pricing of this offering). As a result, these persons, acting together, would be able to control all matters requiring stockholder approval, including the election and removal of directors, any merger, consolidation, sale of all or substantially all of our assets, or other significant corporate transactions.

Some of these persons or entities may have interests different than yours. For example, because many of these stockholders purchased their shares at prices substantially below the current market

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price of our common stock and have held their shares for a longer period, they may be more interested in selling our company to an acquirer than other investors, or they may want us to pursue strategies that deviate from the interests of other stockholders.

Participation in this offering by our existing stockholders and their affiliated entities may reduce the public float for our common stock.

To the extent certain of our existing stockholders and their affiliated entities participate in this offering, such purchases would reduce the non-affiliate public float of our shares, meaning the number of shares of our common stock that are not held by officers, directors and principal stockholders. A reduction in the public float could reduce the number of shares that are available to be traded at any given time, thereby adversely impacting the liquidity of our common stock and depressing the price at which you may be able to sell shares of common stock purchased in this offering.

We will have broad discretion in the use of proceeds from this offering and may invest or spend the proceeds in ways with which you do not agree and in ways that may not increase the value of your investment.

We will have broad discretion over the use of proceeds from this offering, including for any purposes described under "*Use of Proceeds*." We currently intend to use the net proceeds from this offering, together with our existing cash, (i) to complete our ongoing Phase 1 clinical trials, our planned Phase 2a proof of concept trial, and certain preclinical and other early clinical activities to enable potential advancement of our selected dual combination of an NBD1 stabilizer and complementary modulator; (ii) to initiate and progress a dual combination Phase 2b dose-ranging clinical trial of our selected NBD1 stabilizer and complementary modulator and to continue manufacturing scale-up to supply drug product for late stage clinical trials; (iii) on other research and development activities; and (iv) to use the remainder for working capital and other general corporate purposes. See the section titled "*Use of Proceeds*" for additional information. Because of the number and variability of factors that will determine our use of the net proceeds from this offering, their ultimate use may vary substantially from their currently intended use. You may not agree with our decisions, and our use of the proceeds may not yield any return on your investment and the failure by our management to apply these funds effectively could harm our business. Our failure to apply the net proceeds from this offering effectively could compromise our ability to pursue our growth strategy and we might not be able to yield a significant return, if any, on our investment of these net proceeds. You will not have the opportunity to influence our decisions on how to use our net proceeds from this offering.

Because we do not anticipate paying any cash dividends on our common stock in the foreseeable future, capital appreciation of our common stock, if any, will be your sole source of gains and you may never receive a return on your investment.

You should not rely on an investment in our common stock to provide dividend income. We have not declared or paid cash dividends on our common stock to date. We currently intend to retain our future earnings, if any, to fund the development and growth of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future. Investors seeking cash dividends should not purchase our common stock. There is no guarantee that shares of our common stock will appreciate in value or even maintain the price at which stockholders have purchased their shares.

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Our bylaws that became effective upon the effectiveness of this registration statement designate certain courts as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our bylaws that became effective upon the completion of this offering provide that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for any state law claims for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim of breach of, or a claim based on, fiduciary duty owed by any of our current or former directors, officers, and employees to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law, our certificate of incorporation or our bylaws (including the interpretation, validity or enforceability thereof), or (iv) any action asserting a claim that is governed by the internal affairs doctrine, in each case subject to the Court of Chancery of the State of Delaware having personal jurisdiction over the indispensable parties named as defendants therein (the "Delaware Forum Provision"). The Delaware Forum Provision will not apply to any causes of action arising under the Securities Act or the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our amended and restated bylaws further provide that, unless we consent in writing to the selection of an alternative forum, the federal district courts of the U.S. shall be the sole and exclusive forum for resolving any complaint asserting a cause or causes of action arising under the Securities Act (the "Federal Forum Provision"). In addition, our amended and restated bylaws provide that any person or entity purchasing or otherwise acquiring any interest in shares of our common stock is deemed to have notice of and consented to the foregoing provisions; provided, however, that stockholders cannot and will not be deemed to have waived our compliance with the federal securities laws and the rules and regulations thereunder.

The Delaware Forum Provision and the Federal Forum Provision in our amended and restated bylaws may impose additional litigation costs on stockholders in pursuing any such claims. Additionally, the forum selection clauses in our amended and restated bylaws may limit our stockholders' ability to bring a claim in a forum that they find favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees even though an action, if successful, might benefit our stockholders. In addition, while the Delaware Supreme Court ruled in March 2020 that federal forum selection provisions purporting to require claims under the Securities Act be brought in federal court were "facially valid" under Delaware law, there is uncertainty as to whether other courts will enforce our Federal Forum Provision. If the Federal Forum Provision is found to be unenforceable, we may incur additional costs associated with resolving such matters. The Federal Forum Provision may also impose additional litigation costs on stockholders who assert that the provision is not enforceable or invalid. The Court of Chancery of the State of Delaware and the federal district courts of the U.S. may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments may be more or less favorable to us than our stockholders.

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General Risk Factors

If our information technology systems or data, or those of third parties upon which we rely, are or were compromised, or fail, we could experience adverse consequences resulting from such compromise, or failure, including but not limited to regulatory investigations or actions; litigation; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; and other adverse consequences.

In the ordinary course of our business, we and the third parties upon which we rely, process, collect, receive, store, use, transfer, protect, secure, dispose of, transmit, and share collectively referred to as processing proprietary, confidential, and sensitive data, including personal data (such as health-related data), intellectual property and trade secrets, collectively referred to as sensitive information. The secure processing, maintenance and transmission of sensitive information is critical to our operations. Despite our security measures, our information technology and infrastructure may be vulnerable to attacks by hackers or breached due to employee error, malfeasance or other disruptions.

Cyber-attacks, malicious internet-based activity, online and offline fraud, security breaches and other similar activities threaten the confidentiality, integrity, and availability of our sensitive information and information technology systems, and those of the third parties upon which we rely. Such threats are prevalent and continue to rise, are increasingly difficult to detect, and come from a variety of sources, including traditional computer “hackers,” threat actors, “hacktivists,” organized criminal threat actors, personnel (such as through theft or misuse), sophisticated nation states and nation-state-supported actors. Some actors now engage and are expected to continue to engage in cyber-attacks, including without limitation nation-state actors for geopolitical reasons and in conjunction with military conflicts and defense activities.

These risks, as well as the number and frequency of cybersecurity events globally, may also be heightened during times of war or other major conflicts. We and the third parties upon which we rely are subject to a variety of evolving threats, including but not limited to social-engineering attacks (including through phishing attacks), malicious code (such as viruses and worms), malware (including as a result of advanced persistent threat intrusions), denial-of-service attacks (such as credential stuffing attacks), credential harvesting, personnel misconduct or error, ransomware attacks, supply-chain attacks, software bugs, server malfunctions, software or hardware failures, loss of data or other information technology assets, adware, electrical and telecommunications failures, earthquakes, fires, floods, and other similar threats. In particular, severe ransomware attacks are becoming increasingly prevalent and can lead to significant interruptions in our operations, loss of sensitive data and income, reputational harm and diversion of funds.

We have a hybrid in-office/remote work environment, which, like other companies that have incorporated remote working, has increased risks to our information technology systems and data, as more of our employees utilize network connections, computers and devices outside our premises or network, including working at home, while in transit and in public locations.

Future business transactions (such as acquisitions or integrations) could expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities’ systems and technologies. Furthermore, we may discover security issues that were not found during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

We currently rely on third-party service providers and technologies to operate critical business systems to process sensitive information in a variety of contexts, including, without limitation, cloud-based infrastructure, employee email, and other functions. We also currently rely on commercially

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available tools from third-party service providers to process and safeguard our sensitive information and business data. Our ability to monitor these third parties' information security practices is limited, and these third parties may not have adequate information security measures in place. If our third-party service providers experience a security incident or other interruption, we could experience adverse consequences. While we may be entitled to damages if our third-party service providers fail to satisfy their privacy or security-related obligations to us, any award may be insufficient to cover our damages, or we may be unable to recover such award.

Although, to our knowledge, we have not experienced any material security breach to date, we have experienced and may continue to experience threats or system failures which could cause a security incident or other interruption that could result in unauthorized, unlawful or accidental acquisition, modification, destruction, loss, alteration, encryption, disclosure of, or access to our sensitive information or our information technology systems, or those of the third parties upon whom we rely. We have not yet conducted any internal audits or penetration tests on our information technology systems, nor have we enlisted any external parties to conduct security audits or penetration tests on our behalf. Such assessments, when conducted, could indicate vulnerabilities in our information technology systems which we may not be able to effectively remediate. If a security incident were to occur and cause interruptions in our operations, it could result in a material disruption of our product development programs. For example, the loss of clinical trial data from completed or ongoing or planned clinical trials could result in delays in our regulatory approvals efforts and significantly increase our costs to recover or reproduce the data. We may expend significant resources or modify our business activities (including our clinical trial activities) to try to protect against security incidents.

Certain data privacy and security obligations may require us to implement and maintain specific security measures or industry-standard or reasonable security measures to protect our information technology systems and sensitive information. While we have implemented security measures designed to protect against security incidents, there can be no assurance that these measures will be effective. Additionally, certain federal, state and foreign government requirements include obligations of companies to notify individuals of security breaches involving particular categories of personally identifiable information, which could result from breaches experienced by us or the third parties upon whom we rely. Such disclosures are costly, and the disclosure or the failure to comply with such requirements could lead to adverse consequences.

We may not be able to detect and remediate vulnerabilities in our information technology systems because the threats and techniques used to exploit the vulnerability change frequently and are often sophisticated in nature. Therefore, such vulnerabilities could be exploited but may not be detected until after a security incident has occurred. These vulnerabilities pose material risks to our business. Further, we may experience delays in developing and deploying remedial measures designed to address any such identified vulnerabilities. It is not possible to prevent all threats to our information technology systems and those of our third-party service providers, over which we exert less control, and any controls we implement to do so may prove to be ineffective.

If we (or a third party upon whom we rely) experience a security incident or are perceived to have experienced a security incident, we may experience adverse consequences, such as government enforcement actions (for example, investigations, fines, penalties, audits, and inspections); additional reporting requirements and/or oversight; restrictions on processing sensitive information (including personal data); litigation (including class claims); indemnification obligations; negative publicity; reputational harm; monetary fund diversions; interruptions in our operations (including availability of data); financial loss; and other similar harms. Security incidents and attendant consequences may cause delays or disruptions in our clinical trials and development of product candidates, deter customers from using our products, and negatively impact our ability to grow and operate our business.

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Our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages or claims related to our data privacy and security obligations. We cannot be sure that our insurance coverage will be adequate or sufficient to protect us from or to mitigate liabilities arising out of our privacy and security practices, that such coverage will continue to be available on commercially reasonable terms or at all, or that such coverage will pay future claims. In addition to experiencing a security incident, third parties may gather, collect or infer sensitive information about us from public sources, data brokers or other means that reveals competitively sensitive details about our organization and could be used to undermine our competitive advantage or market position.

We are subject to stringent and evolving U.S. laws and regulations and foreign laws, regulations, rules, contractual obligations, policies and other obligations related to data privacy and security. Our actual or perceived failure to comply with such obligations could lead to regulatory investigations or actions; litigation; fines and penalties; disruptions of our business operations, reputational harm, loss of revenue or profits, and other adverse business consequences.

In the ordinary course of business, we and the third parties on which we rely process personal data and other sensitive information, including proprietary and confidential business data, trade secrets, intellectual property, data we collect about clinical trial participants and sensitive third-party data. Our data processing activities may subject us to numerous data privacy and security obligations, such as various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contractual requirements, and other obligations relating to data privacy and security.

The legislative and regulatory framework for the processing of personal data worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. In the U.S., there are numerous federal and state privacy and data security laws and regulations governing the collection, use, disclosure, transfer, security and processing of personal information, including federal and state health information privacy laws, federal and state security breach notification laws, federal and state consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), and other similar laws (e.g., wiretapping laws). In addition, we may obtain health information from third parties (including research institutions from which we obtain clinical trial data) that are subject to HIPAA, which imposes specific requirements relating to the privacy, security, and transmission of individually identifiable health information. Depending on the facts and circumstances, we could be subject to criminal penalties if we knowingly obtain, use or disclose individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA.

At the state level, numerous U.S. states have enacted comprehensive privacy laws that impose certain obligations on covered businesses, including providing specific disclosures in privacy notices and affording individuals certain rights concerning their personal data. Similar laws are being considered in several other states, as well as at the federal and local levels, and we expect more states to pass similar laws in the future. While these states exempt some data processed in the context of clinical trials, these developments may further complicate compliance efforts, and increase legal risk and compliance costs for us and the third parties upon whom we rely. Furthermore, other states have proposed or enacted legislation that is focused on more narrow aspects of privacy. For example, a number of states have passed laws that protect biometric information and a smaller number of states have passed or are considering laws that are specifically focused upon health privacy, such as Washington's My Health My Data Act. The My Health My Data Act imposes new state restrictions and requirements on the processing and sale of consumer health data and creates a private right of action. The effects of state and federal privacy laws are potentially significant and may require us to modify our data processing practices and policies and to incur substantial costs and potential liability in an effort to comply with such legislation.

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Outside the U.S., an increasing number of laws, regulations, and industry standards may govern data privacy and security. For example, if we commence clinical trials in Europe, our processing of personal data in that context would become subject to the European Union's General Data Protection Regulation ("EU GDPR"), and/or the United Kingdom's so-called "UK GDPR" (together, the "GDPR"). The GDPR is wide-ranging in scope and imposes numerous requirements on companies that are subject to it, including relating to having a legal basis for processing personal data, relating to the processing of sensitive data (such as health data), obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, notification of data breaches, requiring data protection impact assessments for high risk processing and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the European Union and the UK, including the U.S., and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million (£17.5 million) or 4% of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR.

In addition, we may be unable to transfer personal data from Europe and other jurisdictions to the U.S. or other countries due to data localization requirements or limitations on cross-border data flows. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal data to other countries. In particular, the European Economic Area ("EEA") and the United Kingdom ("UK") have significantly restricted the transfer of personal data to the U.S. and other countries whose privacy laws it considers inadequate. Other jurisdictions may adopt similarly stringent data localization and cross-border data transfer laws. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and UK to the U.S. in compliance with law, such as the EEA's standard contractual clauses, the UK's International Data Transfer Agreement / Addendum, and the EU-U.S. Data Privacy Framework and the UK extension thereto (which allows for transfers to relevant U.S.-based organizations who self-certify compliance and participate in the Framework), these mechanisms are subject to legal challenges and there is no assurance that we can satisfy or rely on these measures to lawfully transfer personal data to the U.S. If there is no lawful manner for us to transfer personal data from the EEA, the UK or other jurisdictions to the U.S., or if the requirements for a legally-compliant transfer are too onerous, we could face significant adverse consequences, including the interruption or degradation of our operations, the need to relocate part of or all of our business or data processing activities to other jurisdictions (such as Europe) at significant expense, increased exposure to regulatory actions, substantial fines and penalties, the inability to transfer data and work with partners, vendors and other third parties, and injunctions against our processing or transferring of personal data necessary to operate our business. Additionally, companies that transfer personal data out of the EEA and UK to other jurisdictions, particularly to the U.S., are subject to increased scrutiny from regulators, individual litigants, and activist groups. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers of personal data out of Europe for allegedly violating the GDPR's cross-border data transfer limitations.

We are also bound by contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. If any privacy policies, marketing materials and other statements regarding data privacy and security that we publish are found to be deficient, lacking in transparency, deceptive, unfair or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators, including the Federal Trade Commission, or other adverse consequences.

Laws and regulations related to privacy, data protection and security are continuing to evolve and are becoming increasingly stringent. Additionally, these laws may be subject to differing applications

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and interpretations, which may be inconsistent or conflict among jurisdictions, creating uncertainty. Preparing for and complying with these obligations requires us to devote significant resources, which may necessitate changes to the ways in which we collect and process data, our information technologies, systems and to exercise greater oversight and control over third parties that process personal data on our behalf.

We may at times fail (or be perceived to have failed) in our efforts to comply with our data privacy and security obligations. Moreover, despite our efforts, our personnel or third parties on whom we rely may fail to comply with such obligations, which could negatively impact our business operations. If we or the third parties we rely on fail, or are perceived to have failed, to address or comply with applicable data privacy and security obligations, we may face significant consequences, including but not limited to: government enforcement actions (e.g., investigations, fines, penalties, audits, inspections, and similar); litigation (including class-action claims); additional reporting requirements and/or oversight; bans on processing personal data; orders to destroy or not use personal data; and imprisonment of company officials. Any of these events could have a material adverse effect on our reputation, business, or financial condition, including but not limited to: loss of customers; interruptions or stoppages in our business operations (including, as relevant, clinical trials and development of product candidates); inability to process personal data or to operate in certain jurisdictions; limited ability to develop or commercialize our products; expenditure of time and resources to defend any claim or inquiry; adverse publicity; or substantial changes to our business model or operations.

We are subject to governmental export and import controls, economic sanctions, anti-corruption laws and regulations of the U.S. and other jurisdictions. We can face criminal liability and other serious consequences for violations of these laws and regulations, which could harm our business.

We are subject to and required to comply with various export control, import and trade and economic sanctions laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations and sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls. These laws may prohibit or restrict our ability to transfer, sell or supply, our products to certain governments, persons, entities, countries, and territories, including those that are the target of comprehensive sanctions or an embargo.

We are also subject to anti-corruption and anti-bribery laws, including the U.S. Foreign Corrupt Practices Act of 1977, as amended (the "FCPA"), the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws, including the FCPA, generally prohibit companies and their employees, agents, CROs, contractors and other partners from offering, promising, giving, soliciting or authorizing others to give or receive anything of value, either directly or indirectly, to or from a non-U.S. government official in order to influence official action, or otherwise obtain or retain business. The FCPA also requires public companies to make and keep books and records that accurately and fairly reflect the transactions of the corporation and to devise and maintain an adequate system of internal accounting controls. We can be held liable for the corrupt or other illegal activities of our employees, agents, CROs, contractors, and other partners, even if we do not explicitly authorize or have actual knowledge of such activities. Any violation of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences.

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If we fail to maintain an effective system of internal controls over financial reporting, our ability to produce accurate financial statements on a timely basis could be impaired.

After the closing of this offering, we will be subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act of 2002 (the "Sarbanes-Oxley Act"), and the rules and regulations of the stock market on which our common stock is listed. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting.

Commencing with our second annual report on Form 10-K for the fiscal year ending December 31, 2026, we must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting in our Form 10-K filing for that year, as required by Section 404 of the Sarbanes-Oxley Act. This will require that we incur substantial additional professional fees and internal costs to expand our accounting and finance functions and that we expend significant management efforts. Prior to this offering, we have never been required to test our internal control within a specified period, and, as a result, we may experience difficulty in meeting these reporting requirements in a timely manner. In addition, when we lose our status as an "emerging growth company" and if we do not otherwise qualify as a "non-accelerated filer," our independent registered public accounting firm will be required to attest to the effectiveness of our internal control over financial reporting, which will require additional expense, resources and management commitment.

We may identify material weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our consolidated financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner, or if we are unable to maintain proper and effective internal controls over financial reporting, we may not be able to produce timely and accurate consolidated financial statements. If that were to happen, the market price of our stock could decline and we could be subject to sanctions or investigations by the stock exchange on which our common stock is listed, the SEC or other regulatory authorities.

Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.

Upon completion of this offering, we will become subject to certain reporting requirements of the Exchange Act. Our disclosure controls and procedures are designed to reasonably assure that information required to be disclosed by us in reports we file or submit under the Exchange Act is accumulated and communicated to management, recorded, processed, summarized, and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people, or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements or insufficient disclosures due to error or fraud may occur and not be detected.

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We might not be able to utilize a significant portion of our net operating loss carryforwards.

We have generated, and expect to continue to generate, significant federal and state net operating loss ("NOL") carryforwards. As of December 31, 2023, we had federal and state net operating loss carryforwards of $48.2 million and $47.1 million, respectively. Under current tax laws and regulations, these NOL carryforwards could expire unused and be unavailable to offset future income tax liabilities. Under the Tax Cuts and Jobs Act, as modified by the Coronavirus Aid, Relief, and Economic Security Act, federal NOLs incurred in taxable years beginning after December 31, 2017 generally may be carried forward indefinitely, but the deductibility of such federal NOLs is limited.

In addition, under Section 382 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50% change by value, in its equity ownership over a three-year period, the corporation's ability to use its pre-change NOL carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be limited. We may have experienced ownership changes in the past and may experience ownership changes as a result of this offering and/or subsequent shifts in our stock ownership which may be out of our control. There is also a risk that due to regulatory changes, such as suspensions on the use of NOLs by federal or state taxing authorities or other unforeseen reasons, portions of our existing NOLs could expire or otherwise be unavailable to reduce future income tax liabilities.

New tax laws or regulations, changes to existing tax laws or regulations or changes in their application to us may have a material adverse effect on our business, cash flows, financial condition or results of operations.

U.S. federal, state, local and foreign tax laws, regulations and administrative guidance are subject to change as a result of the legislative process and review and interpretation by the U.S. Internal Revenue Service, the U.S. Treasury Department and other taxing authorities. Changes to tax laws (which changes may have retroactive application), including with respect to net operating losses and research and development tax credits, could adversely affect us or holders of our common stock. In recent years, many such changes have been made and changes are likely to continue to occur in the future. Future changes in tax laws could have a material adverse effect on our business, cash flow, financial condition or results of operations. We urge investors to consult with their legal and tax advisers regarding the implications of potential changes in tax laws on an investment in our common stock.

We are eligible to be treated as an "emerging growth company" and a "smaller reporting company" and, as a result of the reduced disclosure and governance requirements applicable to emerging growth companies and smaller reporting companies, our common stock may be less attractive to investors.

We are an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012 (the "JOBS Act"). For as long as we continue to be an emerging growth company, we may take advantage of certain exemptions from reporting requirements that are applicable to other public companies that are not emerging growth companies, including:

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting; providing only two years of audited financial statements in addition to any required unaudited interim financial statements and a correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure in this prospectus;

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- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation in our periodic reports, proxy statements and registration statements; and
- not being required to hold a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

We cannot predict if investors will find our common stock less attractive because we will rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the closing of our initial public offering, (b) in which we have total annual gross revenue of at least $1.235 billion or (c) in which we are deemed to be a large accelerated filer, which means, among other conditions, that the market value of our common stock that is held by non-affiliates exceeds $700 million as of the last business day of our most recently completed second fiscal quarter, and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

Under Section 107(b) of the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies, and we expect to rely on this exemption. Even after we no longer qualify as an emerging growth company, we may, under certain circumstances, still qualify as a "smaller reporting company," which would allow us to take advantage of many of the same exemptions from disclosure requirements, including reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements.

We will incur increased costs and demands upon management as a result of being a public company.

As a public company, we will incur significant additional legal, accounting and other costs that we did not incur as a private company. We will be subject to the reporting requirements of the Exchange Act, which will require, among other things, that we file with the SEC annual, quarterly and current reports with respect to our business and financial condition. In addition, Sarbanes-Oxley, as well as rules subsequently adopted by the SEC and Nasdaq to implement provisions of Sarbanes-Oxley, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and certain corporate governance practices. Further, pursuant to the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010, the SEC has adopted additional rules and regulations in these areas, such as mandatory "say on pay" voting requirements that will apply to us when we cease to be an emerging growth company. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

We expect the rules and regulations applicable to public companies to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. If these requirements divert the attention of our management and personnel from other business concerns, they could have an adverse effect on our business. The increased costs will increase our net loss, and may require us to reduce costs in other areas of our business.

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Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.

Our operations could be adversely affected by general conditions in the global economy and in the global financial markets and uncertainty about economic stability. The global economy and financial markets have experienced extreme volatility and disruptions, including severely diminished liquidity and credit availability, declines in consumer confidence, inflation, declines in economic growth, global supply chain disruptions, and uncertainty about economic stability. The global economy and financial markets may also be adversely affected by the current or anticipated impact of military conflict, terrorism or other geopolitical events, including the ongoing war in Ukraine, the Israel-Gaza conflict and the increasingly strained relationship between the U.S. and China. Sanctions imposed by the U.S. and other countries in response to such conflicts may adversely impact the financial markets and the global economy, and the economic countermeasures by the affected countries or others could exacerbate market and economic instability.

There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. A severe or prolonged economic downturn could result in a variety of risks to our business, including weakened demand for any product candidates we may develop and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption. If the equity and credit markets deteriorate, it may make any necessary equity or debt financing more difficult, more costly, and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could impair our ability to achieve our growth strategy, could harm our financial performance and stock price and could require us to delay or abandon clinical development plans. In addition, there is a risk that our current or future service providers, manufacturers or other collaborators may not survive such difficult economic times, which could directly affect our ability to attain our operating goals on schedule and on budget. We cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

We may become involved in litigation that could divert management's attention and harm our business, and insurance coverage may not be sufficient to cover all costs and damages.

From time to time, we may be subject to litigation claims through the ordinary course of our business operations regarding, but not limited to, securities litigation, employment matters, security of patient and employee personal data, contractual relations with collaborators and licensors and intellectual property rights. We may be exposed to such litigation even if no wrongdoing on our part has occurred. Litigation to defend ourselves against claims by third parties, or to enforce any rights that we may have against third parties, could result in substantial costs and diversion of our resources, causing a material adverse effect on our business, financial condition, results of operations or cash flows.

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CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements about us and our industry that involve substantial risks and uncertainties. All statements other than statements of historical facts contained in this prospectus, including statements regarding our future results of operations and financial position, business strategy, product candidates, preclinical studies and clinical trials, results of preclinical studies and clinical trials, research and development costs, regulatory approvals, commercial strategy, timing and likelihood of success, as well as plans and objectives of management for future operations, are forward-looking statements. These statements involve known and unknown risks, uncertainties and other important factors that are in some cases beyond our control and may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements.

In some cases, you can identify forward-looking statements by terms such as “may,” “will,” “should,” “would,” “expect,” “plan,” “anticipate,” “could,” “intend,” “target,” “project,” “believe,” “estimate,” “predict,” “potential” or “continue” or the negative of these terms or other similar expressions. Forward-looking statements contained in this prospectus include, but are not limited to, statements about:

- the initiation, timing, progress and results of our research and development programs, preclinical studies and clinical trials;
- the ability of clinical trials to demonstrate safety and efficacy of our product candidates, and other positive results, and the ability of our preclinical studies to predict later clinical trial results;
- the timing, scope and likelihood of regulatory filings and approvals of our product candidates;
- the implementation of our business model, and strategic plans for our business, programs, and current and future product candidates;
- our ability to obtain additional cash and the sufficiency of our existing cash, cash equivalents and investments in marketable securities to fund our future operating expenses and capital expenditure requirements;
- the accuracy of our estimates regarding expenses, preliminary financial information, future revenue, capital requirements and needs for additional financing;
- the size and growth potential of the markets for our product candidates, and our ability to serve those markets; our potential and ability to successfully manufacture and supply our current and future product candidates for clinical trials and for commercial use, if approved; the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates;
- developments relating to our competitors and our industry, including competing product candidates and therapies;
- existing regulations and regulatory developments in the U.S. and other jurisdictions;
- expectations regarding future events under collaboration and licensing agreements, including potential future payments, as well as our plans and strategies for entering into further collaboration and licensing agreements;
- general economic, industry and market conditions, including rising interest rates and inflation;
- our ability to attract and retain the continued service of our key personnel and to identify, hire and then retain additional qualified personnel;
- our expectations regarding the period during which we will qualify as an emerging growth company under the JOBS Act; and

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- our anticipated use of our existing cash, cash equivalents and investments in marketable securities and the proceeds from this offering.

We have based these forward-looking statements largely on our current expectations and projections about our business, the industry in which we operate and financial trends that we believe may affect our business, financial condition, results of operations and prospects, and these forward-looking statements are not guarantees of future performance or development. These forward-looking statements speak only as of the date of this prospectus and are subject to a number of risks, uncertainties and assumptions described in "*Risk Factors*" and elsewhere in this prospectus. Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified, you should not rely on these forward-looking statements as predictions of future events. The events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual results could differ materially from those projected in the forward-looking statements. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein until after we distribute this prospectus, whether as a result of any new information, future events or otherwise.

In addition, statements that "we believe" and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this prospectus, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain, and you are cautioned not to unduly rely upon these statements.

You should read this prospectus and the documents that we reference in this prospectus and have filed with the SEC as exhibits to the registration statement of which this prospectus forms a part with the understanding that our actual future results, levels of activity, performance and events and circumstances may be materially different from what we expect.

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USE OF PROCEEDS

We estimate that the net proceeds from this offering will be approximately $173.0 million (or approximately $199.6 million if the underwriters exercise their option to purchase 1,588,234 additional shares of our common stock in full) based on the initial public offering price of $18.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

The principal purposes of this offering are to create a public market for our common stock and thereby facilitate future access to the public equity markets, increase our visibility in the marketplace and obtain additional capital to support our operations. We currently intend to use the net proceeds from this offering, together with our existing cash, cash equivalents and investments in marketable securities, for the following:

- approximately $40.0 million to complete our ongoing Phase 1 clinical trials, our planned Phase 2a proof of concept trial, and certain preclinical and other early clinical activities to enable potential advancement of our selected dual combination of an NBD1 stabilizer and complementary modulator;
- approximately $110.0 million to initiate and progress a dual combination Phase 2b dose-ranging clinical trial of our selected NBD1 stabilizer and complementary modulator and to continue manufacturing scale-up to supply drug product for late stage clinical trials;
- approximately $35.0 million on other research and development activities; and
- the remainder for working capital and other general corporate purposes, including the costs to operate our business, such as our employee and facility costs and costs associated with being a public company.

We may also use a portion of the remaining net proceeds and our existing cash, cash equivalents and investments in marketable securities to in-license, acquire or invest in complementary businesses, technologies, products or assets. However, we have no current commitments, agreements, understandings or obligations to do so.

We believe, based on our current operating plan, that the net proceeds from this offering, together with our existing cash, cash equivalents and investments in marketable securities, will be sufficient to fund our operations into 2028.

Our expected use of proceeds from this offering described above represents our current intentions based on our present plans and business condition. As of the date of this prospectus, we cannot predict with certainty all of the particular uses for the net proceeds to be received upon the completion of this offering or the actual amounts that we will spend on the uses set forth above. We expect that we will require additional funds in order to fully accomplish the specified uses of the proceeds of this offering. Moreover, certain of our anticipated clinical activities are cross-program. For example, we intend to conduct dual combination trials of our lead NBD1 stabilizer with one or more of our complementary modulators. The specific allocation and timing of our actual expenditures will depend on numerous factors, including progress of our research and development efforts, the status of and results from preclinical studies and ongoing and future clinical trials, the timing and outcome of regulatory submissions, and other factors described in "*Risk Factors*" in this prospectus, as well as the amount of cash used in our operations and any unforeseen cash needs. Therefore, our actual expenditures may differ materially from the estimates described above. We may find it necessary or advisable to use the net proceeds for other purposes.

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We will have broad discretion over how to use the net proceeds to us from this offering and investors will be relying on the judgment of our management regarding the application of the net proceeds. Pending our use of the net proceeds from this offering, we intend to invest the net proceeds in a variety of capital preservation instruments, including short-term and long-term interest-bearing instruments, investment-grade securities, and direct or guaranteed obligations of the U.S. government. We cannot predict whether the proceeds invested will yield a favorable return.

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DIVIDEND POLICY

We have never declared or paid cash dividends on our capital stock. We do not anticipate declaring or paying, in the foreseeable future, any cash dividends on our capital stock. We intend to retain all available funds and future earnings, if any, to fund the development and expansion of our business, and we do not anticipate paying any cash dividends in the foreseeable future. Any future determination regarding the declaration and payment of dividends, if any, will be at the discretion of our board of directors, subject to applicable laws, and will depend on then-existing conditions, including our financial condition, operating results, contractual restrictions, capital requirements, business prospects and other factors our board of directors may deem relevant.

In addition, our ability to pay cash dividends on our capital stock in the future may be limited by the terms of any future debt or preferred securities we issue or any credit facilities we enter into.

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CAPITALIZATION

The following table sets forth our cash, cash equivalents and investments in marketable securities and total capitalization as of September 30, 2024:

- on an actual basis;
- on a pro forma basis, giving effect to (i) the automatic conversion of all outstanding shares of preferred stock into an aggregate of 27,149,206 shares of common stock immediately prior to the completion of this offering and (ii) the filing and effectiveness of our fifth amended and restated certificate of incorporation, which will occur immediately prior to the completion of this offering; and
- on a pro forma as adjusted basis, giving effect to (i) the pro forma adjustments set forth above and (ii) the issuance and sale of 10,588,233 shares of common stock in this offering at the initial public offering price of $18.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

The pro forma as adjusted information below is illustrative only, and our capitalization following the completion of this offering will be adjusted based on the actual initial public offering price and other terms of this offering determined at pricing.

You should read this information in conjunction with our audited consolidated financial statements and the related notes included elsewhere in this prospectus and in "*Management's Discussion and Analysis of Financial Condition and Results of Operations*."

	As of September 30, 2024		
(in thousands, except share and per share data)	Actual	Pro Forma	Pro Forma As Adjusted
Cash, cash equivalents and investments in marketable securities	$ 180,874	$ 180,874	$ 354,990
Convertible preferred stock (Series Seed, A, B and C), par value $0.001 per share; 39,667,715 shares authorized, issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	$ 330,368	$ —	$ —
Stockholders' (deficit) equity:			
Preferred stock, par value $0.001 per share; no shares authorized, issued or outstanding, actual; and 10,000,000 shares authorized and no shares issued or outstanding, pro forma and pro forma as adjusted	—	—	—
Common stock, par value $0.001 per share; 55,200,000 shares authorized, 4,783,261 shares issued and 4,663,622 shares outstanding, actual; 55,200,000 shares authorized, 31,932,467 shares issued and 31,812,828 shares outstanding, pro forma; 55,200,000 shares authorized, 42,520,700 shares issued and 42,401,061 shares outstanding, pro forma as adjusted	5	32	43
Additional paid-in capital	15,911	346,252	519,288
Accumulated other comprehensive loss	773	773	773
Accumulated deficit	(165,238)	(165,238)	(165,238)
Total stockholders' (deficit) equity	(148,549)	181,819	354,866
Total capitalization	$ 181,819	$ 181,819	$ 354,866

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The number of shares of common stock that will be outstanding after this offering on a pro forma and pro forma as adjusted basis is based on 31,932,467 shares of common stock (which includes 119,639 shares of restricted common stock) outstanding as of September 30, 2024 after giving effect to the automatic conversion of all outstanding shares of our preferred stock into the aggregate of 27,149,206 shares of common stock immediately prior to the completion of this offering, and excludes:

- 3,652,051 shares of common stock issuable upon exercise of outstanding stock options as of September 30, 2024 under our 2020 Plan, with a weighted average exercise price of $5.77 per share;
- 63,745 shares of common stock issuable upon exercise of outstanding stock options granted after September 30, 2024 pursuant to our 2020 Plan, with a weighted average exercise price of $10.33 per share;
- 1,099,636 shares of common stock reserved for future issuance as of September 30, 2024 under the 2020 Plan, which ceased to be available for issuance at the time that our 2025 Plan became effective;
- 390,127 shares of common stock reserved for future issuance under our ESPP, which became effective on the date immediately prior to the effectiveness of the registration statement of which this prospectus forms a part, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the ESPP; and
- 5,060,000 shares of our common stock that will become available for future issuance under our 2025 Plan, which became effective on the date immediately prior to the effectiveness of the registration statement of which this prospectus forms a part, which includes an aggregate of 1,946,388 shares of our common stock, which will be granted to certain of our executive officers, directors and employees at the time of effectiveness of the 2025 Plan with an exercise price equal to $18.00, the initial public offering price per share, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the 2025 Plan and any shares underlying outstanding stock awards granted under the 2020 Plan that expire or are repurchased, forfeited, cancelled or withheld.

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DILUTION

If you invest in our common stock in this offering, your ownership interest will be diluted to the extent of the difference between the initial public offering price per share of our common stock and the pro forma as adjusted net tangible book value per share of our common stock immediately after this offering.

Our historical net tangible book deficit as of September 30, 2024 was $(150.5) million, or $(31.46) per share of our common stock. Our historical net tangible book deficit represents the amount of our total tangible assets less our total liabilities and preferred stock. Historical net tangible book deficit per share represents historical net tangible book deficit divided by the number of shares of our common stock outstanding as of September 30, 2024.

Our pro forma net tangible book value as of September 30, 2024 was $179.9 million, or $5.63 per share. Pro forma net tangible book value per share represents the amount of our total tangible assets (net of deferred offering costs) less our total liabilities, divided by the number of shares of our common stock outstanding as of September 30, 2024, after giving effect to the automatic conversion of all outstanding shares of preferred stock into 27,149,206 shares of common stock immediately prior to the completion of this offering.

After giving further effect to the sale of 10,588,233 shares of common stock that we are offering at the initial public offering price of $18.00 per share, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of September 30, 2024 would have been $354.9 million, or $8.35 per share. This amount represents an immediate increase in pro forma net tangible book value of $2.72 per share to our existing stockholders and an immediate dilution in pro forma net tangible book value of $9.65 per share to new investors purchasing shares of common stock in this offering.

Dilution per share to new investors is determined by subtracting pro forma as adjusted net tangible book value per share after this offering from the initial public offering price per share paid by new investors. The following table illustrates this dilution (without giving effect to any exercise by the underwriters of their option to purchase additional shares):

Initial public offering price per share		$18.00
Historical net tangible book deficit per share as of September 30, 2024	$(31.46)	
Pro forma increase in net tangible book value per share as of September 30, 2024 attributable to the pro forma adjustment described above	37.09	
Pro forma net tangible book value per share as of September 30, 2024	5.63	
Increase in pro forma net tangible book value per share attributable to new investors participating in this offering	2.72	
Pro forma as adjusted net tangible book value per share after this offering		8.35
Dilution per share to new investors in this offering		$ 9.65

If the underwriters exercise their option to purchase up to 1,588,234 additional shares of our common stock in full, the pro forma as adjusted net tangible book value after the offering would be $8.65 per share, the increase in pro forma as adjusted net tangible book value per share to existing stockholders would be $0.30 per share and the dilution per share to new investors would be $0.30 per share, in each case based on the initial public offering price of $18.00 per share.

The following table summarizes on the pro forma as adjusted basis described above, as of September 30, 2024, the differences between the number of shares of common stock purchased from

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us by our existing stockholders and common stock by new investors purchasing shares in this offering, the total consideration paid to us in cash and the average price per share paid by existing stockholders for shares of common stock issued prior to this offering and the price to be paid by new investors for shares of common stock in this offering. The calculation below is based on the initial public offering price of $18.00 per share, before deducting underwriting discounts, placement agent fees and commissions and estimated offering expenses payable by us.

	Shares Purchased		Total Consideration		Weighted-Average Price Per Share
	Number	Percentage	Amount	Percentage	
	(in thousands, except share, per share and percent data)				
Existing stockholders before this offering	31,932,467	75.1%	$330,537	63.4%	$ 10.35
New investors purchasing shares in this offering	10,588,233	24.9%	$190,588	36.6%	$ 18.00
Total	42,520,700	100.0%	$521,125	100.0%	

The table above assumes no exercise of the underwriters' option to purchase additional shares in this offering. If the underwriters' option to purchase additional shares of our common stock is exercised in full, the number of shares of our common stock held by existing stockholders would be reduced to 72.4% of the total number of shares of our common stock outstanding after this offering, and the number of shares of common stock held by new investors purchasing common stock in this offering would be increased to 27.6% of the total number of shares of our common stock outstanding after this offering.

The foregoing tables and calculations (other than the historical net tangible book value calculation) are based on 31,932,467 shares of common stock (which includes 119,639 shares of restricted common stock) outstanding as of September 30, 2024 after giving effect to the automatic conversion of outstanding shares of our preferred stock into 27,149,206 shares of common stock immediately prior to the completion of this offering, and excludes:

- 3,652,051 shares of common stock issuable upon exercise of outstanding stock options as of September 30, 2024 under our 2020 Plan, with a weighted average exercise price of $5.77 per share;
- 63,745 shares of common stock issuable upon exercise of outstanding stock options granted after September 30, 2024 pursuant to our 2020 Plan, with a weighted average exercise price of $10.33 per share;
- 1,099,636 shares of common stock reserved for future issuance as of September 30, 2024 under the 2020 Plan, which ceased to be available for issuance at the time that our 2025 Plan became effective;
- 390,127 shares of common stock reserved for future issuance under our ESPP, which became effective on the date immediately prior to the effectiveness of the registration statement of which this prospectus forms a part, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the ESPP; and
- 5,060,000 shares of our common stock that will become available for future issuance under our 2025 Plan, which became effective on the date immediately prior to the effectiveness of the registration statement of which this prospectus forms a part, which includes an aggregate of 1,946,388 shares of our common stock, which will be granted to certain of our executive officers, directors and employees at the time of effectiveness of the 2025 Plan with an exercise

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price equal to $18.00, the initial public offering price per share, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the 2025 Plan and any shares underlying outstanding stock awards granted under the 2020 Plan that expire or are repurchased, forfeited, cancelled or withheld.

To the extent any outstanding options are exercised, new options or other equity awards are issued under our equity incentive plans, or we issue additional equity or convertible debt securities in the future, there will be further dilution to new investors.

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MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations in conjunction with our consolidated financial statements and related notes and other financial information included elsewhere in this prospectus. This discussion and analysis and other parts of this prospectus contain forward-looking statements based upon our current plans and expectations that involve risks, uncertainties and assumptions, such as statements regarding our plans, objectives, expectations, intentions and beliefs. Our actual results and the timing of events could differ materially from those anticipated in these forward-looking statements as a result of various factors, including those set forth under "Risk Factors" and elsewhere in this prospectus. You should carefully read the "Risk Factors" section of this prospectus to gain an understanding of the important factors that could cause actual results to differ materially from our forward-looking statements. Also see the section titled "Cautionary Note Regarding Forward-Looking Statements."

Overview

We are a clinical-stage biopharmaceutical company on a mission to revolutionize the current treatment paradigm for cystic fibrosis ("CF") patients by developing novel medicines that normalize the function of the cystic fibrosis transmembrane conductance regulator ("CFTR") protein to deliver clinically meaningful benefit to CF patients. Our goal is to deliver differentiated medicines for people living with CF that can restore their CFTR function to as close to normal as possible by directly stabilizing CFTR's nucleotide-binding domain 1 ("NBD1"). We believe stabilizing NBD1 is central to unlocking dramatic improvements in clinical outcomes and quality of life for CF patients.

We are conducting ongoing Phase 1 trials of our two highly potent NBD1 stabilizers—SION-719 and SION-451—evaluating the safety, tolerability and PK of single and multiple ascending doses of each product candidate in healthy subjects. These trials are randomized, doubled-blinded, placebo-controlled trials being conducted in Australia. As of January 14, 2025, five SAD cohorts and three MAD cohorts of SION-719 have been completed, with over 60 healthy subjects dosed (randomized 3:1 active:placebo), and six SAD cohorts and three MAD cohorts of SION-451 have been completed, with over 70 healthy subjects dosed (randomized 3:1 active:placebo). Both SION-719 and SION-451 have been generally well tolerated based on interim Phase 1 clinical data as of the cutoff date of January 14, 2025. In these trials, at both single and multiple doses, SION-719 and SION-451 exposures were achieved that have the potential, based on our preclinical CFHBE model, to provide clinically meaningful benefit if SION-719 or SION-451 were administered as part of a dual combination or as an add-on to the standard of care ("SOC"). We plan to continue enrolling healthy subjects in the trial.

We are also developing a portfolio of complementary CFTR modulators designed to work synergistically with our NBD1 stabilizers to improve CFTR function, as seen in preclinical models. In July 2024, we in-licensed three clinical-stage compounds from AbbVie to expand our portfolio of combination product opportunities, including galicaftor (SION-2222), which targets CFTR's transmembrane domain 1 ("TMD1"), and has completed Phase 2 clinical trials. In addition, we have recently completed a Phase 1 clinical trial evaluating SION-109, which targets CFTR's intracellular loop 4 ("ICL4") region.

We believe our robust pipeline of NBD1 stabilizers and complementary modulators provide multiple potential pathways to achieving our goal, either in combination with each other to produce a proprietary combination CF therapy, or in combination with the current standard of care. We plan to evaluate multiple NBD1 stabilizer candidates and complementary modulator candidates and select the most promising candidates to advance into later-stage development. Initially, we intend to evaluate the lead NBD1 stabilizer candidate in combination with the current standard of care in a proof-of-concept

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trial. In parallel, we will determine the proprietary dual combination that we believe is optimal to advance into a Phase 2b dose-ranging trial in CF patients. We currently have exclusive rights to develop and commercialize our compounds.

Since our inception in 2019, we have not generated any revenue. To date, we have funded our operations primarily with proceeds from the sale and issuance of our preferred stock. We have received aggregate net proceeds of $330.4 million from the sale and issuance of our preferred stock.

Due to our significant research, development and manufacturing expenditures, we have accumulated substantial losses and negative cash flows since our inception, including net losses of $45.8 million and $35.4 million for the nine months ended September 30, 2024 and 2023, respectively, and $47.3 million and $40.2 million for the years ended December 31, 2023 and 2022, respectively. As of September 30, 2024, we had an accumulated deficit of $165.2 million.

We expect our expenses and operating losses will increase substantially as we:

- continue to advance the clinical development of our current and future product candidates, including our lead NBD1 stabilizer product candidate, including conducting our ongoing clinical trials;
- continue to advance our research activities and seek to discover and develop additional product candidates to expand our pipeline;
- pursue regulatory approvals for any current or future product candidates, including our lead NBD1 stabilizer product candidate, that successfully complete clinical trials;
- continue to utilize third parties to manufacture our product candidates;
- continue to develop, maintain, expand, enforce, defend and protect our intellectual property portfolio (including intellectual property obtained through license agreements) and provide reimbursement of third-party expenses related to our patent portfolio;
- attract, hire and retain additional qualified personnel;
- add operational, financial and management information systems;
- undertake pre-commercial activities, and scale-up external commercial-scale manufacturing capabilities, to commercialize any current or future product candidates which may obtain regulatory approval;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any current or future product candidates which may receive regulatory approval; and
- incur additional audit, legal, regulatory, tax and other expenses associated with being a public company.

In addition, we have several clinical development, regulatory, and commercial milestones, as well as royalty payment obligations under our licensing arrangements. Our net losses may fluctuate significantly from quarter to quarter and year to year, depending on the timing of our ongoing and planned clinical trials and our expenditures on other research and development activities.

We do not have any products approved for sale and have not generated any revenue from product sales. We will not generate revenue from product sales unless and until we successfully complete clinical development and obtain regulatory approval for our current and any future product candidates, which we expect will take a number of years or may never occur. As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of equity offerings, debt financings or other capital sources, potentially including collaborations, licenses or other strategic arrangements. See the section titled

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"*—Liquidity and Capital Resources*." We may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such agreements as, and when, needed, we may have to significantly delay, scale back or discontinue the development and commercialization of one or more of our product candidates, or grant rights to develop and market our product candidates even if we would otherwise prefer to develop and market such product candidates ourselves.

Due to the numerous risks and uncertainties associated with drug development, we are unable to accurately predict the timing or amount of increased expenses or the timing of when, or if, we will be able to achieve or maintain profitability. Even if we generate product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

As of September 30, 2024, we had cash, cash equivalents and marketable securities of $180.9 million. Based upon our current operating plans, we believe that the estimated net proceeds from this offering, together with our existing cash, cash equivalents and investments in marketable securities, will be sufficient to fund our operations into 2028. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. In addition, we could utilize our available capital resources sooner than we expect. See the sections titled "*—Liquidity and Capital Resources*" and "*Risk Factors—Risks Related to Our Limited Operating History, Financial Condition and Need for Additional Capital"* included elsewhere in this prospectus. We believe that we will have sufficient funds to meet our obligations within the next twelve months after the date that our condensed consolidated financial statements included in this prospectus are issued. See the section titled "*Use of Proceeds*."

License and Collaboration Agreements

Below is a summary of the key terms for certain of our license and collaboration agreements. For a more detailed description of these agreements, see the section titled "*Business—License and Collaboration Agreements*."

Sanofi License Agreement

In December 2019, we entered into a license agreement, which has been subsequently amended (as amended, the "Sanofi License Agreement"), with Sanofi, pursuant to which we have been granted an exclusive, worldwide, sublicensable, royalty-bearing license to develop and commercialize products using the licensed compounds and know-how for CFTR modulator therapies. The licensed and derived rights are being utilized in SION-719, SION-109 and SION-451.

As initial consideration for the license, we paid a non-refundable, upfront payment of $1.5 million, as well as a reimbursement of $0.3 million for Sanofi's research and development expenses, which was recorded as research and development expense in the condensed consolidated statements of operations and comprehensive loss because the acquired license represented in-process research and development with no alternative future use. In addition, we are required to pay Sanofi a total of up to $40.0 million upon achievement of certain late-stage developmental and commercial milestones. The developmental milestone payment will be recorded when the milestone is achieved, and the commercial milestone payment and royalties will be recorded when the sales occur. As of September 30, 2024, none of such milestones have been achieved. We are also required to pay royalties to Sanofi in the low single-digit percentage range based on net sales of licensed products, subject to customary reductions and offsets. Such royalty payments shall be reduced for products covered by derived patents.

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CFF Payment Agreement

In December 2019, we entered into a payment agreement (the "CFF Payment Agreement") with CFF, pursuant to which we agreed to provide CFF with compensation in exchange for the grant of, or forbearance from exercising, certain of CFF's rights existing under the license agreement, by and between CFF (through an assignment by Cystic Fibrosis Foundation Therapeutics, Inc.) and Genzyme Corporation, an affiliate of Sanofi (the "CFFT-Genzyme Agreement"). Under the CFF Payment Agreement, we are obligated to compensate CFF in connection with our development and commercialization of licensed products under the Sanofi License Agreement.

As initial consideration for CFF's grant of, and forbearance from exercising, its rights under the CFFT-Genzyme Agreement, we paid an upfront fee of $0.2 million and issued CFF 300,300 shares of our Series Seed preferred stock, valued at $1.0 million. In addition, we agreed to pay CFF a sub-teen double-digit percentage of any amounts paid by us to Sanofi under the Sanofi License Agreement, other than milestone, royalty or reimbursement payments. We are required to pay CFF a total of up to $40.0 million upon achievement of certain late-stage developmental and commercial milestones. The developmental milestone payment will be recorded when the milestone is achieved, and the commercial milestone payment and royalties will be recorded when the sales occur. As of September 30, 2024, none of such milestones have been achieved. We are also required to pay revenue-shares of royalty payments to CFF in the low single-digit percentage range based on net sales of licensed products, subject to customary reductions and offsets. Such milestone and royalty payments shall be reduced for products covered by derived patents. Further, a side letter was executed between us and Sanofi, which clarifies the relationship between us, Sanofi and CFF, under which we are obligated to pay Sanofi 20% of the milestones it would have been obligated to pay CFF, net of the milestone amounts it is obligated to pay under the Sanofi License Agreement.

AbbVie License Agreement

In July 2024, we entered into a license agreement (the "AbbVie License Agreement") with AbbVie, pursuant to which we have been granted an exclusive, worldwide, royalty-bearing, sublicensable license to research, develop and commercialize certain CFTR compounds. The licensed rights are directed, among other things, to three clinical-stage CFTR modulator therapies: SION-2222, SION-2851 and SION-3067. The license granted to us under the AbbVie License Agreement is subject to certain preexisting rights held by AbbVie and Galapagos NV ("Galapagos"). In particular, certain of the licensed patents and other intellectual property rights were developed by or on behalf of Galapagos and are sublicensed to us subject to the terms of the second amended and restated collaboration agreement between Galapagos and AbbVie in October 2018 (the "Galapagos License Agreement"), as amended by a side letter between Galapagos and AbbVie in July 2024.

As initial consideration for the license, we paid a non-refundable, upfront payment of $5.0 million and issued 1,414,445 shares of our common stock with a fair value of $8.6 million to AbbVie. We determined that the AbbVie License Agreement represented an asset acquisition as it did not meet the definition of the business. We recorded the total initial consideration of $13.6 million as research and development expense during the nine month period ended September 30, 2024 because the acquired license represented in-process research and development with no alternative future use. In addition, we are required to pay AbbVie a total of up to $360.0 million upon achievement of certain development and commercial milestones, consisting of up to $70.0 million in late-stage development milestones and up to $290.0 million in commercial milestones. We are also required to pay royalties to AbbVie in the low to mid single-digit percentage range based on net sales of licensed products, subject to customary reductions and offsets. The royalty term will terminate on a product-by-product and country-by-country basis upon the later of (i) the expiration of the last-to-expire valid claim within the relevant licensed patent rights, (ii) the expiration of regulatory exclusivity in such country for such licensed product and (iii) the tenth anniversary of the first commercial sale of a licensed product in such country.

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In addition, we are required to pay AbbVie up to $130.0 million in commercial and sales-based milestone payments, mid to high single-digit royalties on the licensed products or other payments due to Galapagos pursuant to the Galapagos License Agreement, to the extent such payments are triggered by our use of the licensed rights owned by Galapagos under the AbbVie License Agreement. As of September 30, 2024, none of such milestones under the AbbVie License Agreement and Galapagos License Agreement have been achieved.

Components of Results of Operations

Revenue

To date, we have not generated any revenue. In the future, we may generate revenue from product sales from any approved product, which approval we do not expect to occur for at least the next several years, if ever, as well as collaboration or license agreements we may enter into with respect to our current or future product candidates. If our development efforts for our current or future product candidates are successful and result in regulatory approval, we may generate revenue in the future from product sales. We cannot predict if, when or to what extent we will generate revenue as we may never succeed in obtaining regulatory approval for any of our product candidates. If we fail to complete preclinical and clinical development of our current or future product candidates or fail to obtain regulatory approval for any that successfully complete clinical trials, our ability to generate future revenues, and our results of operations and financial position would be adversely affected.

Operating Expenses

Our operating expenses consist of (i) research and development expenses and (ii) general and administrative expenses.

Research and Development Expenses

Research and development expenses consist primarily of costs associated with the preclinical and clinical development of our current and potential future product candidates. In particular, our research and development expenses include:

- personnel-related costs, including salaries, payroll tax, bonuses, benefits and stock-based compensation for employees engaged in research and development functions;
- the costs to acquire in-process research and development with no alternative future use acquired in an asset acquisition;
- external expenses, including expenses incurred under arrangements with third parties, such as contract research organizations (“CROs”), contract development and manufacturing organizations (“CDMOs”), consultants and our scientific advisors;
- the cost of manufacturing our product candidates, including costs for laboratory supplies, research materials and reagents;
- facility costs, depreciation and other expenses, which include direct and allocated expenses; and
- the cost of obtaining and maintaining patent and trade secret protection for our product candidates.

We recognize research and development costs in the periods in which they are incurred. Most of our research and development expenses have been related to identifying and developing our product candidates. Typically, external expenses are recognized based on an evaluation of the progress to

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completion of specific tasks using information provided to us by our service providers as of each reporting date. Advance payments that we make for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses, which are expensed as the related goods are delivered or the services are performed, or when it is no longer expected that the goods will be delivered, or the services rendered. Significant judgments and estimates are made in determining the accrued, or prepaid expense balances at the end of any reporting period.

External costs represent a significant portion of our research and development expenses, which we track on a program-by-program basis following the nomination of a product candidate. Our internal research and development expenses consist primarily of personnel-related expenses, including stock-based compensation expenses and allocated expenses. We do not track our internal research and development expenses on a program-by-program basis as they either relate to early-stage research expenses, such as lab supplies or our personnel expenses, consulting fees or other costs that are deployed across multiple programs.

Product candidates in later stages of development generally have higher development costs than those in earlier stages resulting from larger and more complex clinical trials, manufacturing scale-up and an increase in research and development headcount to oversee these activities. As a result, management expects that our research and development expenses will increase substantially over the next several years as we potentially advance our product candidates into later-stage development efforts.

Our future development costs may vary significantly based on a variety of factors, including:

- the timing, complexity and progress of preclinical and clinical development activities;
- the number and scope of preclinical and clinical programs we decide to pursue;
- the extent to which we in-license or acquire other product candidates and technologies to further develop our pipeline;
- the successful initiation and completion of clinical trials with safety, tolerability and efficacy profiles that are satisfactory to domestic and foreign regulatory authorities;
- receipt of marketing approvals, if any, from applicable regulatory authorities;
- the development of commercial-scale manufacturing and distribution processes for our current any future product candidates;
- our ability to obtain, maintain and protect patent, trade secret protection and regulatory exclusivity for our product candidates, both in the U.S. and internationally;
- our ability to successfully recruit and retain additional employees;
- the timing and amount of milestones, royalties or other payments we must make to our licensing partners; and
- the commercialization of our product candidates, if and when approved.

A change in the outcome of any of these variables with respect to the development of any current or future product candidate could mean a significant change in the costs and timing associated with the development of that product candidate. For example, if the FDA or another regulatory authority were to delay a planned start of a clinical trial or require us to conduct clinical trials beyond those that we currently anticipate would be required for the completion of clinical development of a product candidate, or if we experience significant delays in our clinical trials due to slower than expected patient enrollment or other reasons, we could be required to expend significant additional financial resources and time on the completion of clinical development of that product candidate. We do not

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have control over many of these factors, including certain aspects of clinical development, the regulatory submissions process, potential threats to our intellectual property rights and general political and economic conditions that may negatively impact our business in the future. We may never obtain regulatory approval for any of our product candidates, and, even if we do, drug commercialization takes several years and millions of dollars in development costs.

General and Administrative Expenses

General and administrative expenses consist primarily of personnel-related costs, including salaries, payroll tax, bonuses, benefits and stock-based compensation charges for those individuals in executive, legal, finance, human resources, information technology and other administrative functions. Other significant costs include legal fees relating to intellectual property and corporate matters, professional fees for auditing, accounting, tax and consulting services, office and information technology costs, insurance costs and facilities, depreciation and other general and administrative expenses that are allocated. We recognize general and administrative expenses in the periods in which they are incurred.

We anticipate that our general and administrative expenses will increase in the future to support our increased research and development activities, pre-commercial preparation activities for our product candidates and any future product candidates and, if any product candidate receives marketing approval, commercialization activities. These increases will likely include increased costs related to the hiring of additional personnel and fees paid to outside consultants, among other expenses. We also anticipate increased expenses related to audit, accounting, legal and regulatory services associated with public company reporting and compliance, director and officer insurance premiums, investor and public relations costs and other administrative and professional services associated with operating as a public company.

Interest Income

Interest income consists primarily of interest earned and the amortization of discount or premiums on our cash equivalents and investments in marketable securities. We expect our interest income will increase as we invest the cash received from the net proceeds from this offering.

Other Income

Other income consists of sublease income through our subleasing agreement which is further discussed within Note 7, "*Leases*" in our consolidated financial statements included elsewhere in this prospectus.

Income Taxes

Since our inception, we have not recorded any income tax benefits for the net losses we have incurred in each period or for our earned research and development tax credits, as we believe, based upon the weight of available evidence, that it is more likely than not that all of our net operating loss carryforwards and tax credits will not be realized.

As of December 31, 2023, we had $48.2 million and $47.1 million of federal and state operating loss carryforwards, respectively. The federal NOLs are not subject to expiration and the state NOLs begin to expire in 2043. These loss carryforwards are available to reduce future federal taxable income, if any. As of the nine months ended September 30, 2024 and 2023, and the years ended December 31, 2023 and 2022, we have recorded a full valuation allowance against our net deferred tax assets.

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Results of Operations

Comparison of the Nine Months Ended September 30, 2024 and 2023

The following table summarizes our results of operations (in thousands):

	Nine Months Ended September 30,		
	2024	2023	Change
Operating expenses:			
Research and development	$ 43,035	$ 30,736	$ 12,299
General and administrative	9,388	7,002	2,386
Total operating expenses	52,423	37,738	14,685
Loss from operations	(52,423)	(37,738)	(14,685)
Other income:			
Interest income	6,051	2,216	3,835
Other income	532	135	397
Total other income:	6,583	2,351	4,232
Net loss	$ (45,840)	$ (35,387)	$(10,453)

Research and Development Expenses

The following table summarizes our research and development expenses (in thousands):

	Nine Months Ended September 30,		
	2024	2023	Change
Direct research and development expenses by program:			
NBD1 programs	$ 13,588	$20,624	$ (7,036)
Complementary modulator programs	5,388	1,069	4,319
Unallocated research and development expenses:			
IPR&D acquisition related costs	13,639	—	13,639
Personnel-related (including stock-based compensation)	6,521	6,123	398
Facility, lab and depreciation	1,925	1,783	142
Other R&D related costs	1,974	1,137	837
Total research and development expenses	$ 43,035	$30,736	$12,299

Research and development expenses increased by $12.3 million to $43.0 million for the nine months ended September 30, 2024 from $30.7 million for the nine months ended September 30, 2023. The increase in research and development expenses was primarily due to direct research and development expenses of:

- $4.3 million increase in complementary modulator programs cost due to the initiation of the SION-109 Phase 1 clinical trial in 2024 offset by a $7.0 million decrease in NBD1 program costs due to a decrease in manufacturing expenses; and
- unallocated research and development expenses increased by $15.0 million due to in-process research and development (“IPR&D”) costs incurred in connection with the AbbVie License Agreement of $13.6 million and other unallocated research and development expenses of $1.4 million due to increased professional services and external research and development spend.

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General and Administrative Expenses

The following table summarizes our general and administrative expenses (in thousands):

	Nine Months Ended September 30,		
	2024	2023	Change
Personnel-related (including stock-based compensation)	$ 5,608	$4,452	$1,156
Professional services & fees	2,636	1,499	1,137
Facility expenses	1,144	1,051	93
Total general and administrative expenses	$ 9,388	$7,002	$2,386

General and administrative expenses increased by $2.4 million to $9.4 million for the nine months ended September 30, 2024, from $7.0 million for the nine months ended September 30, 2023. The increase in general and administrative expenses was primarily due to:

- $1.2 million increase in personnel-related (including stock-based compensation) primarily due to an increase in stock-based compensation; and
- $1.1 million increase in fees related to increased use of consultants and professional service organizations.

Interest Income

Interest income increased by $3.9 million to $6.1 million for the nine months ended September 30, 2024, from $2.2 million for the nine months ended September 30, 2023, primarily driven by an increased investment in debt securities due to the proceeds received from the issuance of our Series C preferred stock (the “Series C Financing”) and the amortization of discounts on our investment activity.

Other Income

Other income increased by $0.4 million to $0.5 million for the nine months ended September 30, 2024, from $0.1 million for the nine months ended September 30, 2023, driven by sublease income in connection with our subleasing agreement.

Comparison of the Years Ended December 31, 2023 and 2022

The following table summarizes our results of operations (in thousands):

	Year Ended December 31,		
	2023	2022	Change
Operating expenses:			
Research and development	$ 40,626	$ 34,605	$ 6,021
General and administrative	9,707	6,767	2,940
Total operating expenses	50,333	41,372	8,961
Loss from operations	(50,333)	(41,372)	(8,961)
Total other income:	3,070	1,132	1,938
Net loss	$ (47,263)	$(40,240)	$(7,023)

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Research and Development Expenses

The following table summarizes our research and development expenses (in thousands):

	Year Ended December 31,		
	2023	2022	Change
Direct research and development expenses by program:			
NBD1 programs	$ 26,357	$ 19,794	$ 6,563
Complementary modulator programs	2,056	5,541	(3,485)
Unallocated research and development expenses:			
Personnel-related (including stock-based compensation)	8,681	7,068	1,613
Facility, lab and depreciation	2,004	777	1,227
Other R&D related costs	1,528	1,425	103
Total research and development expenses	$ 40,626	$ 34,605	$ 6,021

Research and development expenses increased by $6.0 million to $40.6 million for the year ended December 31, 2023 from $34.6 million for the year ended December 31, 2022. The increase in research and development expenses was primarily due to direct research and development expenses of:

- $6.6 million increase in NBD1 program costs due to initiation of the Phase 1 clinical trial of our first-generation NBD1 stabilizer and ongoing IND-enabling activities for SION-719 and SION-451;
- partially offset by $3.5 million decrease in complementary modulator programs cost as the SION-109 Phase 1 clinical trial start-up activities began in late 2023 and the primary IND-enabling and scale-up activities for this trial were completed in 2022;
- unallocated research and development expenses consisting of $1.6 million increase in personnel-related expenses due to an increase in headcount; and
- $1.2 million increase in facility, lab and depreciation expense due to the commencement of the Waltham Lease (as defined below) in January 2023.

General and Administrative Expenses

The following table summarizes our general and administrative expenses (in thousands):

	Year Ended December 31,		
	2023	2022	Change
Personnel-related (including stock-based compensation)	$ 5,924	$ 4,484	$1,440
Professional services & fees	2,384	2,190	194
Facility expenses	1,399	93	1,306
Total general and administrative expenses	$ 9,707	$ 6,767	$2,940

General and administrative expenses increased by $2.9 million to $9.7 million for the year ended December 31, 2023, from $6.8 million for the year ended December 31, 2022. The increase in general and administrative expenses was primarily due to:

- $1.4 million increase in personnel-related (including stock-based compensation) due to an increase in headcount;
- $1.3 million increase in facility expenses due to the commencement of the Waltham Lease (as defined below) in January 2023; and

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- $0.2 million increase in professional services and fees primarily due to an increase in consulting and audit fee costs.

Interest Income

Interest income increased by $1.7 million to $2.8 million for the year ended December 31, 2023, from $1.1 million for the year ended December 31, 2022, primarily driven by increased interest income and the amortization of discounts on our investment activity.

Other Income

Other income for the year ended December 31, 2023 was $0.3 million, driven by sublease income in connection with our subleasing agreement which was entered into during the year ended December 31, 2023.

Liquidity and Capital Resources

Sources of Liquidity

Since our inception, we have not generated any revenue from product sales and have incurred significant operating losses and negative cash flows from operations. We expect to continue to incur significant expenses and operating losses for the foreseeable future as we advance the clinical development of our product candidates and any future product candidates. As such, we expect our research and development and general and administrative costs will continue to increase significantly, including the costs associated with operating as a public company following the completion of this offering. As a result, we will need additional capital to fund our operations, which we may obtain from additional equity or debt financings or strategic agreements.

To date, we have funded our operations primarily from the sale of proceeds of preferred stock. As of September 30, 2024, we had received aggregate net proceeds of $330.4 million from the sale of our preferred stock in private placements. As of September 30, 2024, we had $180.9 million in cash, cash equivalents and marketable securities.

Cash Flows

The following table sets forth a summary of the net cash flow activity (in thousands):

	Nine Months Ended September 30,		Year Ended December 31,	
	2024	2023	2023	2022
Net cash used in operating activities	$ (41,006)	$(36,247)	$ (43,699)	$ (36,068)
Net cash (used in) provided by investing activities	(142,141)	20,224	27,352	(29,263)
Net cash provided by financing activities	180,375	31	31	110,855
Net (decrease) increase in cash, cash equivalents and restricted cash	$ (2,772)	$(15,992)	$ (16,316)	$ 45,524

Operating Activities

For the nine months ended September 30, 2024, net cash used in operating activities was $41.0 million primarily due to our net loss of $45.8 million and changes in operating assets and liabilities of $6.2 million, partially offset by $11.0 million of non-cash charges related to non-cash license expense, stock-based compensation, depreciation, non-cash operating lease expense and amortization of discounts on marketable securities.

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For the nine months ended September 30, 2023, net cash used in operating activities was $36.2 million primarily due to our net loss of $35.4 million and changes in our operating assets and liabilities of $3.9 million, partially offset by $3.0 million of non-cash charges related to stock-based compensation, depreciation and amortization of discounts on marketable securities.

For the year ended December 31, 2023, net cash used in operating activities was $43.7 million primarily due to our net loss of $47.3 million and changes in operating assets and liabilities of $0.8 million, partially offset by $4.4 million of non-cash charges related to stock-based compensation, depreciation, non-cash operating lease expense and amortization of discounts on marketable securities.

For the year ended December 31, 2022, net cash used in operating activities was $36.1 million primarily due to our net loss of $40.2 million and changes in our operating assets and liabilities of $2.7 million, partially offset by $1.4 million of non-cash charges related to stock-based compensation, depreciation and amortization of discounts on marketable securities.

Investing Activities

Net cash used in investing activities was $142.1 million during the nine months ended September 30, 2024, which was primarily driven by purchases of marketable securities of $175.6 million, partially offset by maturities of marketable securities of $33.5 million.

Net cash provided by investing activities was $20.2 million during the nine months ended September 30, 2023, which was primarily driven by maturities of marketable securities of $44.8 million, partially offset by purchases of marketable securities for $23.5 million and purchases of property and equipment of $1.0 million.

Net cash provided by investing activities was $27.4 million during the year ended December 31, 2023, which was primarily driven by maturities of marketable securities of $52.0 million, partially offset by purchases of marketable securities for $23.5 million and purchases of property and equipment of $1.1 million.

Net cash used in investing activities was $29.3 million during the year ended December 31, 2022, which was primarily driven by purchases of marketable securities for $40.4 million and property and equipment of $1.9 million, partially offset by cash provided from maturities of marketable securities of $13.0 million.

Financing Activities

Net cash provided by financing activities was $180.4 million during the nine months ended September 30, 2024, as compared to $31,000 during the nine months ended September 30, 2023. The increase in net cash provided by financing activities was primarily due to the Series C Financing which occurred in March 2024, compared to no financings completed during 2023, partially offset by $1.1 million of payments of offering costs.

Net cash provided by financing activities was $31,000 during the year ended December 31, 2023, as compared to $110.9 million during the year ended December 31, 2022. The decrease in net cash provided by financing activities was primarily due to the fact that the Series B financing occurred in 2022 and no financings occurred in 2023.

Future Funding Requirements

As of September 30, 2024, we had cash, cash equivalents and marketable securities of $180.9 million. Based upon our current operating plans, we believe that the estimated net proceeds

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from this offering, together with our existing cash, cash equivalents and investments in marketable securities, will be sufficient to fund our operations into 2028. However, our forecast for the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement that involves risks and uncertainties, and actual results could vary materially. Additionally, conducting preclinical studies and clinical trials is costly, and the timing of progress and expenses in these studies and trials is uncertain. We will need to raise substantial additional capital in the future.

Our future funding requirements will depend largely on:

- the type, number, scope, progress, expansions, results, costs and timing of, discovery, preclinical studies and clinical trials of our current and future product candidates;
- the costs and timing of manufacturing for our current and future product candidates and commercial manufacturing;
- the costs, timing and outcome of regulatory review of our current and future product candidates;
- the timing and amount of milestones, royalties, or other payments we may be required to make to third parties, including Sanofi, CFF and AbbVie, and the terms and timing of establishing and maintaining any other similar arrangements we may enter in the future;
- the legal costs of obtaining, maintaining and enforcing our patents and other intellectual property rights;
- our efforts to enhance operational systems and hire additional personnel to satisfy our obligations as a public company;
- the costs associated with hiring additional personnel and consultants as our clinical activities increase;
- the costs and timing of establishing or securing sales and marketing capabilities if any current or future product candidate is approved;
- our ability to achieve sufficient market acceptance, coverage and adequate reimbursement from third-party payors and adequate market share and revenue for any approved products;
- the financial terms of any such agreement that we may enter into, including if we in-license or acquire additional product candidates or intellectual property; and
- costs to add additional operational, financial, clinical, quality and management information systems.

We have no committed sources of capital. Until such time, if ever, as we can generate substantial product revenue to support our cost structure, we expect to finance our cash needs through equity offerings, debt financings or other capital sources, potentially including collaborations, licenses or other strategic arrangements. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our existing common stockholders. In addition, debt financing and equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, or declaring dividends. If we raise additional funds through a strategic agreement, we may have to grant rights to develop and market our current and future product candidates even if we would otherwise prefer to develop and market such product candidates ourselves. Our failure to raise capital or enter into such other arrangements when needed could have a negative impact on our financial condition and on our ability to pursue our business plans and strategies.

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If we are unable to raise additional funds, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts.

Contractual Obligations and Commitments

Research and Development and Manufacturing Agreements

We enter into contracts in the normal course of business with CROs, CDMOs and other vendors to assist in the research and development activities and other services and products for operating purposes. These contracts generally provide for termination at any time upon a certain amount of prior notice.

Leases

In May 2022, we executed a non-cancelable operating lease in Waltham, Massachusetts (the "Waltham Lease") to rent out 24,051 square feet of laboratory and office space and relocated our headquarters to Waltham in January 2023. Total expected cash payments in connection with the Waltham Lease will be $9.7 million over the term of the agreement ending in 2030. This excludes our share of facility operating expenses, real-estate taxes and other costs that are reimbursable to the landlord under the lease. For additional information regarding the lease obligations see Note 7, "Leases" in our consolidated financial statements included elsewhere in this prospectus.

License Agreements

Our agreements with certain third parties to license intellectual property include potential milestone fees, sublicense fees and royalty fees. The milestone fees are dependent upon the development of products using the intellectual property licensed under the arrangements and contingent upon the achievement of development or regulatory approval milestones, as well as commercial milestones. These potential obligations are contingent upon the occurrence of future events and the timing and likelihood of such potential obligations are not known with certainty. For further information regarding these agreements, please see "*Business—Licenses and Collaboration Agreements*."

Critical Accounting Estimates

Our management's discussion and analysis of our financial condition and results of operations are based on our consolidated financial statements, which are prepared in accordance with GAAP. The preparation of our consolidated financial statements requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities, costs and expenses and the disclosure of contingent assets and liabilities in our consolidated financial statements and accompanying notes. We base our estimates and assumptions on historical experience and other factors that we believe to be reasonable under the circumstances. We evaluate our estimates and judgments on an ongoing basis. We base our estimates on historical experience, known trends and events and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Our actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are more fully described in Note 2, *"Summary of Significant Accounting Policies"* in our consolidated financial statements and our unaudited interim condensed consolidated financial statements included elsewhere in this prospectus, we believe that our critical accounting estimates are as follows.

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Research and Development Expenses and Accruals

In preparing the consolidated financial statements, we are required to estimate our accrued research and development expenses as of each balance sheet date. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. This process involves reviewing open contracts, communicating with internal personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of the actual cost. We periodically confirm the accuracy of our estimates with our service providers and make adjustments, if necessary. The majority of our service providers invoice in arrears for services performed or when contractual milestones are met. The financial terms of agreements with these service providers are subject to negotiation, vary from contract-to-contract and may result in uneven payment flows. In circumstances where amounts have been paid in excess of costs incurred, we record a prepaid expense.

Contingent milestone payments, if any, are expensed when the milestone results are probable and estimable, which is generally upon the achievement of the milestone.

Although we do not expect our estimates to be materially different from amounts incurred, if our estimates of the status and timing of services performed differ from the actual status and timing of services performed, it could result in us reporting amounts that are too high or too low in any particular period. To date, there have been no material differences between our estimates of such expenses and the amounts incurred.

Stock-Based Compensation Expense

We measure stock-based awards granted to employees, directors and non-employees based on their fair value on the date of the grant. We recognize compensation expense for awards to employees and directors over the requisite service period, which is generally the vesting period of the respective award. Compensation expense for awards to non-employees with service-based vesting conditions is recognized in the same manner as if we had paid cash in exchange for the goods or services, which is generally over the vesting period of the award. For stock-based awards with service-based vesting conditions, we recognize compensation expense using the straight-line method. The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model, which requires inputs based on certain subjective assumptions, including the expected stock price volatility, the expected term of the option, the risk-free interest rate for a period that approximates the expected term of the option and our expected dividend yield. Changes in the assumptions can materially affect the fair value and ultimately how much stock-based compensation expense is recognized. These inputs are subjective and generally require judgment to develop. See Note 12, "*Stock-based Compensation*" in our annual consolidated financial statements included elsewhere in this prospectus for information concerning certain of the specific assumptions we used in applying the Black-Scholes option pricing model to determine the estimated fair value of our stock options granted for the nine-month periods ended September 30, 2024 and 2023 and the years ended December 31, 2023 and 2022. For awards to non-employees, the expected term of the option is equal to the contractual term of the non-employees' service agreement. The fair value of each restricted common stock award is estimated on the date of grant based on the fair value of our common stock on that same date.

Determination of Fair Value of Common Stock Valuations

As there has been no public market for our common stock to date, the estimated fair value of our common stock has been determined by our board of directors as of the date of each option grant with input from management, considering our most recently available third-party valuations of common

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stock, and our board of directors' assessment of additional objective and subjective factors that it believed were relevant and which may have changed from the date of the most recent valuation through the date of the grant. These third-party valuations were performed in accordance with the guidance outlined in the *American Institute of Certified Public Accountants' Accounting and Valuation Guide, Valuation of Privately-Held-Company Equity Securities Issued as Compensation*. Our common stock valuation was prepared using either the option-pricing method ("OPM") or the hybrid method, both of which used a market approach to estimate our equity value. The OPM treats common stock and convertible preferred stock as call options on the equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. Under the OPM, the common stock has value only if the funds available for distribution to stockholders exceeded the value of the convertible preferred stock liquidation preferences at the time of the liquidity event, such as a strategic sale or a merger. The hybrid method is a probability-weighted expected return method ("PWERM") where the equity value in one or more of the scenarios is calculated using an OPM. The PWERM is a scenario-based methodology that estimates the fair value of common stock based upon an analysis of future values for us, assuming various outcomes. The common stock value is based on the probability-weighted present value of expected future investment returns considering each of the possible outcomes available as well as the rights of each class of stock. The future value of the common stock under each outcome is discounted back to the valuation date at an appropriate risk-adjusted discount rate and probability weighted to arrive at an indication of value for the common stock. A discount for lack of marketability of the common stock is then applied to arrive at an indication of value for the common stock.

These third-party valuations were performed at various dates, which resulted in valuation of our common stock of $6.11 per share as of February 2024, $8.08 per share as of August 2024 and $10.33 per share as of October 2024. The fair value of our common stock was determined by our board of directors, with input from management and considering the independent third-party valuations and various objective and subjective factors as of each grant date, including:

- the prices at which we sold preferred stock and the superior rights and preferences of the preferred stock relative to our common stock at the time of each grant;
- our ability to raise future financings;
- the progress of our research and development efforts, including the status of clinical trials for our product candidates;
- the lack of liquidity of our equity as a private company;
- our stage of development and business strategy and the material risks related to our business and industry;
- the achievement of enterprise milestones, including entering into collaboration and license agreements;
- the valuation of publicly traded companies in the life sciences and biotechnology sectors, as well as recently completed mergers and acquisitions of peer companies;
- any external market conditions affecting the biotechnology industry and trends within the biotechnology industry;
- the likelihood of achieving a liquidity event for the holders of our preferred stock and holders of our common stock, such as an initial public offering, or a sale of our company, given prevailing market conditions; and
- the analysis of IPOs and the market performance of similar companies in the biopharmaceutical industry.

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The assumptions underlying these valuations were highly complex and subjective and represented management's best estimates, which involved inherent uncertainties and the application of management's judgment. As a result, if we had used significantly different assumptions or estimates, the fair value of our common stock and our stock-based compensation expense could be materially different.

Once a public trading market for our common stock has been established in connection with the completion of this offering, it will no longer be necessary for our board of directors to estimate the fair value of our common stock in connection with our accounting for granted stock options and other such awards we may grant, as the fair value of our common stock will be determined based on the quoted market price of our common stock.

Recent Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2, "*Summary of Significant Accounting Policies*" in our consolidated financial statements and our unaudited interim condensed consolidated financial statements included elsewhere in this prospectus.

Qualitative and Quantitative Disclosures about Market Risk

Interest Rate Risk

Our primary exposure to market risk is interest rate sensitivity, which is impacted by changes to the general level of U.S. interest rates, particularly because our cash equivalents and marketable securities are in the form of money market funds and marketable debt securities, which are classified as available for sale. As of September 30, 2024, we had cash, cash equivalents and marketable securities of $180.9 million and as of December 31, 2023, we had cash and cash equivalents of $38.5 million. Interest income is sensitive to changes in the general level of interest rates and our cash, cash equivalents and marketable securities are subject to interest rate risk and could fall in value if market interest rates increase.

As of September 30, 2024 and 2023 and December 31, 2023 and 2022, we had no debt outstanding, and therefore we are not subject to interest rate risk related to debt.

Foreign Currency Exchange Risk

We are not currently exposed to significant market risk related to changes in foreign currency exchange rates.

Effects of Inflation

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We believe that inflation has not had a material effect on our consolidated financial statements included elsewhere in this prospectus.

Emerging Growth Company and Smaller Reporting Company Status

We are an "emerging growth company," as defined in the JOBS Act, and we may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies. We may take advantage of these exemptions until we are no longer an emerging growth company. Section 107 of the JOBS Act provides that an

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"emerging growth company" can take advantage of the extended transition period afforded by the JOBS Act for the implementation of new or revised accounting standards. We have elected to use the extended transition period for complying with new or revised accounting standards and as a result of this election, our consolidated financial statements may not be comparable to companies that comply with public company effective dates. We may take advantage of these exemptions up until the time that we are no longer an "emerging growth company."

We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the completion of this offering, (b) in which we have total annual gross revenue of at least $1.235 billion or (c) in which we are deemed to be a "large accelerated filer" under the rules of the SEC, which means, among other things, the market value of our common stock that is held by non-affiliates exceeds $700.0 million as of the last business day of our most recently completed second fiscal quarter and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

We are also a "smaller reporting company" as defined in the Exchange Act. We may continue to be a smaller reporting company even after we are no longer an emerging growth company. We may take advantage of certain of the scaled disclosures available to smaller reporting companies until for so long as either (i) our voting and non-voting common stock held by non-affiliates is less than $250.0 million measured on the last business day of our second fiscal quarter, or (ii) our annual revenue is less than $100.0 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is less than $700.0 million measured on the last business day of our second fiscal quarter.

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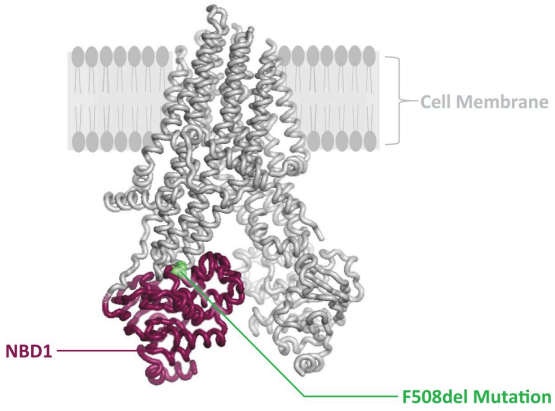
BUSINESS

Overview

We are a clinical-stage biopharmaceutical company on a mission to revolutionize the current treatment paradigm for cystic fibrosis ("CF") patients by developing novel medicines that normalize the function of the cystic fibrosis transmembrane conductance regulator ("CFTR") protein to deliver clinically meaningful benefit to CF patients. CF is a progressive and life-threatening genetic disease caused by inherited mutations in the CFTR gene, which lead to insufficient CFTR function. While advances in the discovery and development of CFTR modulators have significantly improved the lives of people living with CF, at least two-thirds of patients on the current standard of care do not have normal CFTR function, defined as sweat chloride levels below 30 mmol/L. Patients with reduced CFTR function can experience debilitating multi-system complications that lead to significantly reduced quality of life and shorter life expectancy. Our goal is to deliver differentiated medicines for people living with CF that can restore their CFTR function to as close to normal as possible by directly stabilizing CFTR's nucleotide-binding domain 1 ("NBD1"). Despite having long been identified as a critical component for proper CFTR function, NBD1 has been considered "undruggable," and none of the currently approved CF therapies directly stabilizes NBD1. Worldwide revenue for approved CFTR modulators was approximately $10 billion in 2023 and is expected to grow to $15 billion by 2029. Leveraging more than a decade of our co-founders' research on NBD1, we are advancing a pipeline of small molecules engineered to correct the defects caused by the F508del genetic mutation, which resides in the NBD1 domain. Approximately 90% of people with CF carry at least one copy of the F508del genetic mutation.

We believe stabilizing NBD1 is central to unlocking dramatic improvements in clinical outcomes and quality of life for CF patients. The NBD1 domain of the CFTR protein, as illustrated in Figure 1, plays a key role in the folding, stability and trafficking of CFTR to a cell's surface, where it normally functions to conduct chloride and other ions and regulate the flow of water. Within the NBD1 domain, F508del severely destabilizes CFTR, preventing normal folding and trafficking of CFTR to a cell's surface and impairing chloride channel function. We have employed biophysical, cell-based and virtual screening campaigns and extensive use of structural biology to guide the optimization of novel small molecule NBD1 stabilizers.

Figure 1. CFTR Structure



We are conducting ongoing Phase 1 trials of our two highly potent NBD1 stabilizers—SION-719 and SION-451—evaluating the safety, tolerability and PK of single and multiple ascending doses of

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each product candidate in healthy subjects. These trials are randomized (3:1 active:placebo), doubled-blinded, placebo-controlled and are being conducted in Australia. As of January 14, 2025, five SAD cohorts and three MAD cohorts of SION-719 have been completed, with over 60 healthy subjects dosed, and six SAD cohorts and three MAD cohorts of SION-451 have been completed, with over 70 subjects dosed. Both SION-719 and SION-451 have been generally well tolerated based on interim Phase 1 clinical data as of the cutoff date of January 14, 2025. In these trials, at both single and multiple doses, SION-719 and SION-451 exposures were achieved that have the potential, based on our preclinical CFHBE model, to provide clinically meaningful benefit if SION-719 or SION-451 were administered as part of a dual combination or as an add-on to the standard of care ("SOC"). We plan to continue enrolling healthy subjects in additional MAD cohorts.

We are also developing a portfolio of complementary CFTR modulators designed to work synergistically with our NBD1 stabilizers to improve CFTR function, as seen in preclinical models. In July 2024, we in-licensed three clinical-stage compounds from AbbVie Global Enterprises Ltd. ("AbbVie") to expand our portfolio of combination product opportunities, including galicaftor (SION-2222), which targets CFTR's transmembrane domain 1 ("TMD1"), and has completed Phase 2 clinical trials. In addition, we have recently completed a Phase 1 clinical trial evaluating SION-109, which targets CFTR's intracellular loop 4 ("ICL4") region.

Our vision is to build a CF franchise anchored by our NBD1 stabilizers to deliver clinically meaningful benefit to CF patients. We believe our robust pipeline of NBD1 stabilizers and complementary modulators provide multiple potential pathways to achieving that vision, either in combination with each other to produce a proprietary combination CF therapy, or in combination with the current standard of care. We plan to evaluate multiple NBD1 stabilizer candidates and complementary modulator candidates and select the most promising candidates to advance into later-stage development. Initially, we intend to evaluate the lead NBD1 stabilizer candidate in combination with the current standard of care in a proof-of-concept trial. In parallel, we will determine the proprietary dual combination that we believe is optimal to advance into a later-stage clinical trial in CF patients, as illustrated in Figure 2.

Figure 2. Our NBD1 Stabilizers Have Multiple Potential Pathways to Deliver Clinically Meaningful Benefit to CF Patients

Central to our development strategy is our use of the industry standard, clinically predictive preclinical cystic fibrosis human bronchial epithelial ("CFHBE") model to measure CFTR function. The CFHBE model uses lung cells from CF patients and has been highly predictive of clinical outcomes for approved CFTR modulators. Vertex Pharmaceuticals, Inc. ("Vertex"), the manufacturer of the five approved CFTR modulators, has demonstrated that increased chloride transport in the CFHBE model is strongly correlated with improved CFTR function in CF patients. In head-to-head preclinical studies using the CFHBE model, we evaluated several of our proprietary dual combinations (combining each of SION-719 and SION-451 with each of SION-2222 and SION-109) in direct comparison to elexacaftor/tezacaftor/ivacaftor ("ETI") (which we synthesized using methods described in publicly available sources), with all compounds at their respective highest effective dose ("E_{max}"). In other head-to-head preclinical studies using the CFHBE model, we evaluated "add-on" combinations (SION-719 and ETI, and 451 and ETI), in direct comparison to ETI alone, with all compounds at E_{max}. In all of these studies, whether evaluating a proprietary dual combination or an "add-on" combination of an

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NBD1 stabilizer and ETI, we observed a marked improvement in CFTR protein activity of more than 1.5-fold relative to ETI alone. The CFHBE model has helped us identify active compounds and predict the potential clinical exposures needed to achieve a target level of clinical activity in humans. We intend to continue to leverage insights from the CFHBE model as we make critical pipeline prioritization and development decisions.

Our Pipeline

Our proprietary portfolio includes NBD1 stabilizers and complementary modulators. There are two types of complementary modulators: correctors, which partially improve CFTR protein folding to aid its trafficking to the cell surface, and potentiators, which increase CFTR channel function by enabling chloride flow through the cell membrane. We believe the synergistic approach of combining NBD1 stabilizers with complementary modulators provides the highest probability of normalizing CFTR function for CF patients. Our portfolio includes:

- SION-719 and SION-451, our highly potent NBD1 stabilizers, are both in Phase 1 healthy volunteer trials in Australia to evaluate their pharmacokinetic ("PK") profile, safety and tolerability. We have completed SAD dosing and 3 MAD cohorts in each trial. Both NBD1 stabilizers have been generally well tolerated in these ongoing trials based on interim data to date. Topline results from our SION-719 and SION-451 Phase 1 clinical trials are expected in the first half of 2025.
- Galicaftor (SION-2222) and SION-2851 are TMD1-directed CFTR correctors. Galicaftor was generally well-tolerated in Phase 1 and Phase 2 trials, and improvement in sweat chloride as a monotherapy and improvements in sweat chloride and lung function as a combination therapy with navocaftor were observed. SION-2851 has completed a Phase 1 single ascending dose ("SAD") trial in healthy volunteers.
- SION-109, an ICL4-directed CFTR corrector, has been evaluated in a recently completed Phase 1 clinical trial in healthy volunteers. SION-109 was generally well tolerated at all dose levels administered in all parts of this Phase 1 trial. The target exposure for SION-109 as part of a dual combination with SION-451 or SION-719 was achieved with single and multiple doses.
- Navocaftor (SION-3067), a potentiator, has been evaluated in Phase 2 trials, in which it demonstrated potential as a combination therapy.

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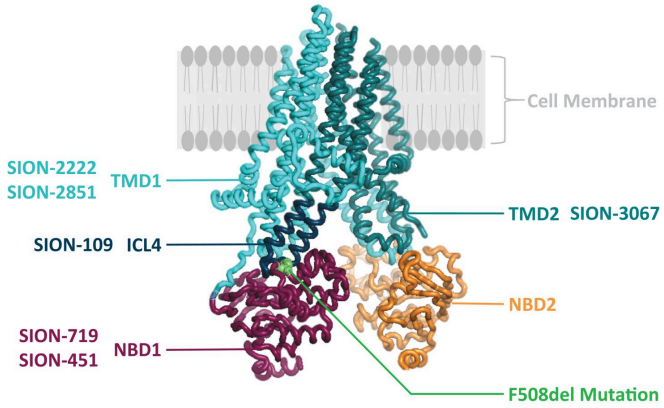
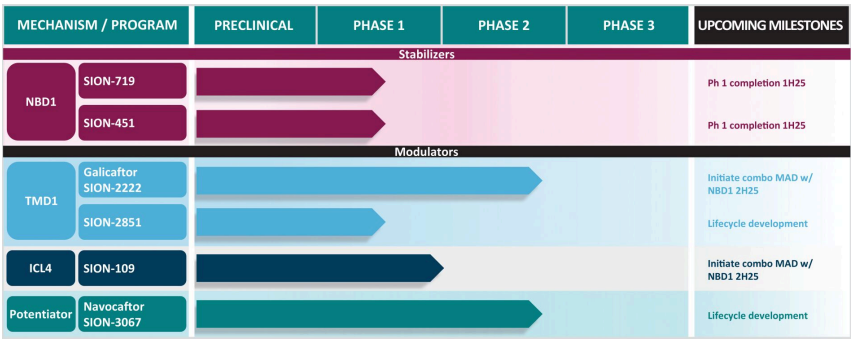
Figure 3 below captures the targeted binding locations within the CFTR structure for our pipeline of NBD1 stabilizers and complementary modulators.

Figure 3. Our Multi-Prong Approach to Potentially Improving CFTR Function



Our current portfolio of programs is summarized in Figure 4 below:

Figure 4. Our Proprietary Pipeline of Product Candidates for the Treatment of CF



Clinical trials for galicaftor, SION-2851, and navocaftor were conducted by AbbVie or Galapagos.

We have not obtained regulatory approval for any product candidate, and it is possible that we may never obtain regulatory approval for any product candidates we may seek to develop in the future.

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Our Strategy

Our mission is to revolutionize the current treatment paradigm for CF patients by developing novel medicines that normalize the function of the CFTR protein to deliver clinically meaningful benefit to CF patients. The key pillars of our strategy are:

- **Advance our novel NBD1 stabilizers**. We are evaluating SION-719 and SION-451 in ongoing Phase 1 clinical trials to assess their PK profile, safety and tolerability. In preclinical studies using the CFHBE model, the combination of SION-719 or SION-451 with one of our complementary modulators or ETI showed increased chloride transport, which indicates improvement in *in vitro* CFTR activity. Given the clinically predictive nature of the CFHBE model, we believe these data indicate the potential for an NBD1-anchored dual combination to improve CFTR activity, which we believe will deliver clinically meaningful benefit to CF patients. Following completion of our ongoing Phase 1 clinical trials of SION-719 and SION-451, expected in the first half of 2025, we plan to select a lead NBD1 stabilizer and conduct a drug-drug interaction trial before initiating a Phase 2a proof-of-concept trial in CF patients.
- **Develop and advance our pipeline of complementary modulators for proprietary combination product development.** We are developing a proprietary pipeline of complementary CFTR modulators designed to work synergistically with our NBD1 stabilizers to improve CFTR function, as seen in our CFHBE model. We have several CFTR modulators in development, including galicaftor (SION-2222), a TMD1-directed corrector, which has recently completed a Phase 1 clinical trial.
- **Build upon our NBD1-centric CF franchise through a data-driven dual combination path.** Our strategy is to advance multiple NBD1 stabilizers and complementary compounds through Phase 1 development, with the goal of selecting the combinations with the best potential, based on human data. Following the selection of our lead NBD1 stabilizer, we intend to begin MAD trials in healthy volunteers evaluating galicaftor and/or SION-109 in combination with our lead NBD1 stabilizer to assess the safety, tolerability and PK of each combination, with the goal of developing proprietary combination therapies that provide clinically meaningful benefit to CF patients. Based on the results of these trials, we plan to select the most promising lead proprietary dual combination to advance into Phase 2b dose-ranging trials in patients with CF.
- **Fortify our CF franchise through continued research efforts and utilization of the translational CFHBE model.** Our goal is to build a CF franchise anchored by our NBD1 stabilizers and remain focused on our mission to deliver clinically meaningful benefit to CF patients. The CFHBE model has been highly predictive of clinical outcomes for approved CFTR modulators, and our application of the model provides a key translational roadmap for us to prioritize compounds for further evaluation. We believe the model allows us to determine the target exposure needed to achieve a desired level of clinical activity, such as the level of improvement in a patient's forced expiratory volume in one second ("FEV_1"), a measure of lung function, and sweat chloride level, the clinical biomarker of CFTR function. We intend to invest in research activities and leverage insights from the model in our pursuit of delivering additional differentiated product candidates that meaningfully impact the lives of CF patients and facilitate our company's long-term growth. We also intend to opportunistically consider strategic in-licensing opportunities to maximize the value of our pipeline.

Our Company's History and Our Team

Sionna was founded in 2019 to continue to explore novel approaches to treating CF by targeting NBD1. Our co-founders, Greg Hurlbut, Ph.D., and Mark Munson, Ph.D., spent over a decade extensively researching the NBD1 target as research leaders at Sanofi SA (f/k/a Sanofi Genzyme) ("Sanofi"). Dr. Hurlbut served as Head of Protein Conformational Diseases and Rare Pulmonary

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Diseases Research, where he led a novel strategy focused on discovering small molecules that correct the F508del CFTR mutation. Dr. Munson served as U.S. Head of Medicinal Chemistry and oversaw a diverse group of scientists that advanced multiple development candidates in oncology and rare disease. Shortly after our inception, we entered into an exclusive license agreement with Sanofi to acquire exclusive worldwide rights to research, develop and commercialize certain compounds designed to stabilize NBD1, as well as an ICL4 corrector.

In addition, we have assembled a leadership team with deep expertise in drug discovery and developing CF and other rare disease therapies, launching and commercializing therapeutics globally, and building successful public pharmaceutical companies.

- ***Mike Cloonan***, our President and Chief Executive Officer, has more than 20 years of leadership experience at global organizations, most recently as Chief Operating Officer at Sage Therapeutics, Inc. and prior to that as Senior Vice President of U.S. Commercial at Biogen, Inc.
- ***Charlotte McKee, M.D.***, our Chief Medical Officer, is a pulmonologist with more than 20 years of drug development experience who, while serving as Vice President of CF and Alpha-1 Antitrypsin Deficiency Clinical Development at Vertex, was instrumental in the development and regulatory approvals of three of Vertex's five approved CFTR modulators, including Trikafta.
- ***Elena Ridloff, C.F.A.***, our Chief Financial Officer and Head of Corporate Development, has more than 20 years of experience in finance in the life sciences industry, most recently as Chief Financial Officer at ACADIA Pharmaceuticals Inc.

Our leadership team is supported by a dedicated team of employees with deep industry-leading expertise, our board of directors, scientific and clinical advisory boards and a group of premier life sciences investors. Since our inception, we have raised approximately $330 million from investors including RA Capital, TPG's the Rise Fund, Atlas Venture, OrbiMed and Enavate Sciences. We also received founding support from the Cystic Fibrosis Foundation ("CFF"), which has been a committed investor and supporter of our research and development work. Prior to our inception, the CFF spent more than a decade funding early-stage F508del corrector discovery work at Sanofi that contributed to our pipeline. Prospective investors should not rely on the past investment decisions of our investors, as our investors may have different risk tolerances and have received their shares in prior offerings at prices lower than the price offered to the public in this offering. Please see the section titled "*Certain Relationships and Related Person Transactions*" included elsewhere in this prospectus for a description of the financings we have conducted to date.

Overview of Cystic Fibrosis, CFTR Function and the F508del Mutation

An estimated 106,000 people have been diagnosed with CF across 94 countries, including approximately 33,000 adults and children living with CF in the U.S., according to the CFF. While life expectancy for CF patients has improved significantly over the years since the first CFTR modulator was approved, the median predicted survival age for individuals with CF born in the U.S. between 2019 and 2023 is still just 61 years, according to the 2023 CFF patient registry. The majority of people who have been diagnosed with CF live in the U.S., the United Kingdom and Europe. CF is the most common fatal inherited disease in the U.S., and it can affect people of every racial and ethnic group. CF is caused by mutations to the CFTR gene that result in reduced or no function of the CFTR protein. The disease is autosomal recessive, meaning that two copies of a CFTR mutation are required to cause the disease, either two copies of the same mutation ("homozygous") or two different mutations ("heterozygous"). Approximately 90% of people with CF carry at least one copy of the F508del mutation, and approximately 44% of people with CF are homozygous for F508del. The F508del mutation (a deletion of the amino acid phenylalanine at position 508, in NBD1) is considered a severe CF mutation, and individuals with this mutation tend to fall at the worst end of the CF severity spectrum because they have little or no CFTR function in epithelial cells.

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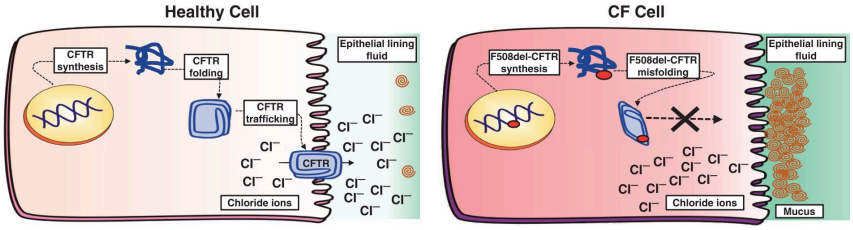
In people with CF, mutations in the CFTR gene cause the CFTR protein to become dysfunctional. The CFTR protein is found on the apical membrane, or surface, of epithelial cells throughout the body, including in the lungs, pancreas, sweat glands, biliary tract and intestines. The CFTR protein is critical for proper salt and water balance in the cell, which drives production of freely flowing mucus for tissue hydration in the airways, digestive system and other organs. When the CFTR protein is not working properly, chloride—a component of salt—gets trapped in cells, as illustrated in Figure 5. Without chloride to attract water to the cell surface, thick mucus accumulates in vital organs such as the lungs, pancreas and gastrointestinal tract and causes multisystem complications, including respiratory infections, chronic lung inflammation, poor nutrient absorption and often progressive respiratory failure, which is the primary cause of death in people with CF.

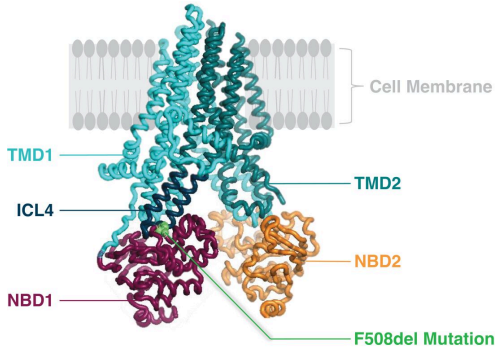
Figure 5. CFTR Regulates Chloride Transport in Epithelial Cells



Source: Favia, 2019

As illustrated in Figure 6, the CFTR protein includes two nucleotide binding domains (NBD1 and NBD2) and two transmembrane domains (TMD1 and TMD2). The transmembrane domains form the ion channel across an epithelial cell's membrane. The nucleotide binding domains facilitate the ion channel's opening and closing by binding and hydrolyzing adenosine triphosphate. There are also four intracellular loops that link the nucleotide binding domains to the transmembrane domains and are important to regulating ion channel gating.

Figure 6. The CFTR Protein and the F508del Mutation



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The F508del mutation resides in the NBD1 domain near an interface with the fourth intracellular loop, ICL4, which is particularly critical to the folding of CFTR. When the ICL4 interface is disrupted, as it is by the F508del mutation, CFTR can neither fold nor function properly. The F508del mutation severely destabilizes CFTR's NBD1 domain, preventing normal folding and trafficking of CFTR to a cell's surface and impairing chloride channel function. Support for NBD1 as a key target is based in part on *in vitro* studies that introduced mutations at other sites on NBD1 that suppressed the effect of the F508del mutation.

Current Unmet Need and Market Opportunity

While advances in the treatment of patients with CF have improved the lives of patients and resulted in a large commercial market, we believe significant opportunity remains to provide clinically meaningful benefit to CF patients through the development of NBD1-anchored treatments. NBD1 has long been considered an important target to normalize CFTR function because it is the site where the F508del mutation—the most common mutation that causes CF—resides. However, attempts by others to stabilize NBD1 have fallen short, leading to the view that NBD1 is "undruggable." The current standard of care, Trikafta, as well as the recently approved Alyftrek, are made up of three components that target certain domains of CFTR, but not NBD1. At least two-thirds of patients on Trikafta do not have normal CFTR function, defined as sweat chloride levels below 30 mmol/L. Even treated CF patients can continue to experience the ongoing effects of reduced CFTR function over time, including respiratory infections, pulmonary exacerbations, or "lung attacks," and continued lung function decline. More than 6,000 patients have discontinued use of approved CFTR modulators, none of which target NBD1. Additionally, some patients on Trikafta reduce dosages due to tolerability issues, including elevated liver function tests and mental health effects such as mood disturbances, depression, and mental fogginess. In December 2024, the Trikafta label was updated to include a boxed warning for the risks of drug-induced liver injury and liver failure, and the Alyftrek label includes the same boxed warning. Seven to eight percent of patients on Trikafta have experienced significant mental health effects, and depression, including suicidal ideation and attempt, is listed in the warnings and precautions section of the summary of product characteristics for Kaftrio, the brand name for Trikafta in Europe. Patients who discontinue use of Trikafta or experience tolerability challenges have limited or no alternative treatments available to improve their clinical outcomes or quality of life. Currently, the alternatives for these patients are limited to less efficacious combination products that include one or more components of Trikafta, or Alyftrek, which demonstrated non-inferiority to Trikafta in the primary endpoint of two Phase 3 clinical trials, providing patients with similar FEV_1 as Trikafta and sweat chloride level improvements of 3 to 8 mmol/L. Approximately 69% of Alyftrek patients in two Phase 3 clinical trials did not achieve normal CFTR function, defined as sweat chloride levels below 30 mmol/L. Our research with key opinion leaders has indicated the desire for more treatment options for CF patients, support for a new mechanism of action that could provide clinically meaningful benefit for people living with CF, and need for an alternative for those patients who experience tolerability issues on Trikafta. We aim to expand the current treatment paradigm through a proprietary dual combination or as an add-on to the standard of care.

Worldwide revenue for approved CFTR modulators was approximately $10 billion in 2023, and it is expected to grow to $15 billion by 2029. Vertex, which markets all five of the currently approved CFTR modulators, reported revenues in 2023 from global sales of its then-approved CFTR modulators of approximately $9.9 billion, more than $8.9 billion of which the company attributed to Trikafta. Vertex's approved CFTR modulators target approximately 92,000 CF patients in North America, Europe and Australia, and more than 20% of eligible patients are currently not on CFTR modulators. CF screening of newborn infants has served to identify CF patients as early as possible in their lives. For example, newborn screening for CF has been required in the U.S. since 2010, and in 2021, 64.4% of newly diagnosed people with CF in the U.S. were identified by newborn screening, based on CFF registry data. The availability of CFTR modulators has also increased the use of genetic testing to determine eligibility for treatment.

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Current Standard of Care and its Limitations

The approved CFTR modulators are oral small molecule therapies that improve CFTR function either by potentiating channel gating or by improving cellular processing and trafficking of the CFTR protein. The current standard of care for people with the F508del mutation is a triple combination product marketed by Vertex as Trikafta (elexacaftor, tezacaftor, ivacaftor and ivacaftor). In addition, in December 2024, Vertex received approval from the FDA for a second-generation, triple modulator combination, Alyftrek, for the treatment of CF in patients aged six years and older who have at least one F508del mutation or another responsive mutation in the CFTR gene. Vertex also markets three other approved CFTR modulators. None of the approved modulators directly stabilize NBD1. The drugs and their approved indications in the U.S. are summarized in Figure 7.

Figure 7. FDA-Approved CFTR Modulators and Summary Indications

U.S. Drug Name	Mechanism of Action	Indication	Approved Age Group in the U.S.	2023 Worldwide Revenues
Alyftrek (vanzacaftor/tezacaftor/deutivacaftor)	TMD1, TMD2, ICL4	At least one F508del mutation or another responsive mutation based on *in vitro* data	6 years and older	n/a
Trikafta (elexacaftor, tezacaftor, ivacaftor and ivacaftor)	TMD1, TMD2, ICL4	At least one F508del mutation or other responsive mutations based on *in vitro* data	2 years and older	$8.95 billion
Symdeko (tezacaftor/ivacaftor and ivacaftor)	TMD1, TMD2	Homozygous F508del or at least one tezacaftor/ivacaftor responsive mutation based on *in vitro* assay data and/or clinical evidence	6 years and older	$123 million
Orkambi (lumacaftor/ivacaftor)	TMD1, TMD2	Homozygous F508del mutation	1 year and older	$326 million
Kalydeco (ivacaftor)	TMD2	At least one ivacaftor responsive mutation based on clinical and/or in vitro assay data	1 month and older	$476 million

Despite the clinical benefits Trikafta provides CF patients, including improved lung function and quality of life, at least two-thirds of people with CF being treated on Trikafta or another approved CFTR modulator do not have normal CFTR function. CF progression is most commonly assessed through a patient's mean lung function, as measured by FEV_1 improvement. In addition, sweat chloride level, the clinical biomarker of CFTR function, has been used for decades as a diagnostic test for CF and has served as a highly useful tool in the development of approved CFTR modulators. A sweat chloride level greater than or equal to 60mmol/L indicates that CF is likely, while a sweat chloride level under 30 mmol/L is normal and indicates that CF is unlikely. Sweat chloride levels between 30 mmol/L and 59 mmol/L are considered abnormal, indicating partial CFTR dysfunction or "residual function" in diagnostic settings. An observational study of 3,131 individuals with CF from the CFF Registry found that, while treatment with Trikafta resulted in improvements in sweat chloride level to below 60 mmol/L in most patients, two-thirds of patients still had sweat chloride levels above normal levels (*i.e.*, above 30 mmol/L) (Figure 8).

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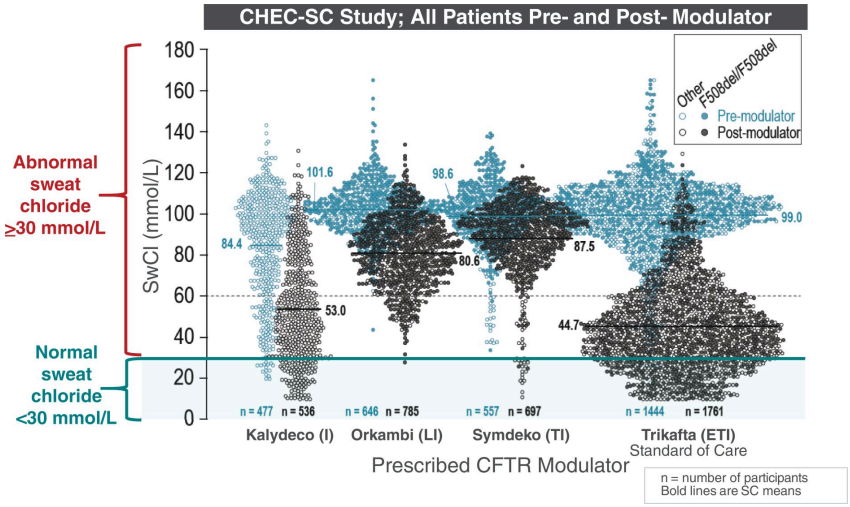
Figure 8. Approximately Two-Thirds of Patients on Approved Therapies Do Not Have Normal CFTR Function as Measured by Sweat Chloride Levels



(Sweat chloride levels less than or equal to 29 mmol/L indicate that CF is unlikely; levels of 30 - 59 mmol/L indicate that CF is possible and additional testing is needed; and levels greater than or equal to 60 mmol/L indicate that CF is likely.)

Two-year interim data from a five-year post-marketing real-world observational trial of patients taking Trikafta showed that mean rates of pulmonary exacerbations and the presence of bacterial pathogens improved but were not normalized after initiating treatment on Trikafta. The three-year interim data, presented at the European CF Society meeting in Glasgow in 2024, showed numerical increases in mean rates of pulmonary exacerbations and decreases in mean lung function compared to the two-year interim data, supporting the opportunity for clinical improvements over the current standard of care. In addition to continued pulmonary complications, patients also experienced negative mental health side effects, including numerical increases in rates of depression, anxiety disorder and hypertension after initiating treatment on Trikafta. Another side effect associated with ivacaftor is cataracts, which can complicate use and requires monitoring, especially in children. In addition, in December 2024, the Trikafta label was updated to include a boxed warning for the risks of drug-induced liver injury and liver failure. The Alyftrek label includes the same boxed warning.

Alyftrek demonstrated non-inferiority to Trikafta in the primary endpoint of two Phase 3 clinical trials, providing patients with similar FEV_1 as Trikafta and sweat chloride level improvements of 3 to 8 mmol/L. Approximately 69% of Alyftrek patients in two Phase 3 clinical trials did not achieve normal CFTR function. These results support our beliefs that NBD1 stabilization is required to meaningfully improve upon the current standard of care and that a high unmet need remains for an alternative therapy that can provide clinically meaningful benefit to CF patients.

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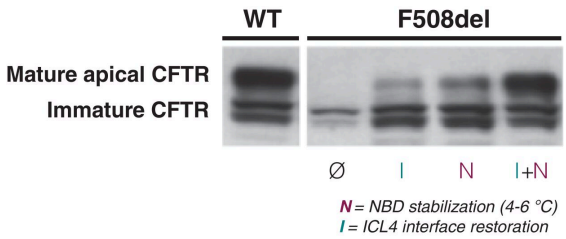
Research Findings Support NBD1 as a Key Target for Stabilizing F508del-CFTR

Multiple studies by third parties have concluded that NBD1 is a key drug target for correcting the F508del mutation, including *in vitro* studies that introduced mutations at other sites on NBD1 that suppressed the effect of the F508del mutation. For example, researchers at Utrecht University and University of Texas Southwestern Medical Center identified a second site NBD1 mutation ("I539T") that rescued the misfolding and instability of F508del-CFTR. They concluded the "co-translational rescue of F508del NBD1 misfolding in CFTR by I539T advocates this domain as the most important drug target for cystic fibrosis."

At University of Texas Southwestern Medical Center, University of Alabama Birmingham, and McGill University, a second group of researchers identified additional mutations that suppressed the effect of F508del and which, in *in vitro* studies, stabilized NBD1 and the NBD1-ICL4 interface and fully restored F508del-CFTR maturation and function to wild-type levels, as shown in the western blot in Figure 9. A western blot is an assay that uses gel electrophoresis to separate a mixture of proteins, which are then transferred to a solid membrane, and then an antibody is used to detect a specific protein in the sample. The mature apical CFTR band is seen with wild-type CFTR. Partial restoration is observed with NBD1 stabilization and ICL4 interface restoration individually; full restoration of CFTR function occurred in the presence of both NBD1 and ICL4 suppressor mutations. Without a suppressor mutation, no F508del-CFTR is produced (Ø).

Figure 9. NBD1 Stabilization Synergizes with Improved Domain-Domain Assembly to Fully Correct F508del-CFTR



Source: Thibodeau, 2010

Research Findings Link Further Improvements in CFTR Function to Improved Clinical Outcomes

Clinical evidence obtained by third parties has illustrated that further reductions in sweat chloride, the clinical biomarker of CFTR function, towards wild-type levels, are associated with improved clinical outcomes, even for patients who have already experienced some benefits from an approved modulator therapy. According to a trial published in the *New England Journal of Medicine*, when CF patients heterozygous for F508del and a "gating" mutation were switched from Kalydeco, which does not target F508del, to Trikafta, which targets F508del, they experienced significant improvements in CFTR function, as measured by improvements in the mean sweat chloride levels, and in mean lung function, as measured by FEV_1. Patients with a gating mutation represent approximately 6% of the CF patient population. Their mean sweat chloride level at baseline was 50.9 mmol/L. After eight weeks of treatment with Trikafta, the patients' mean sweat chloride level was 32.7 mmol/L, representing an improvement of approximately 20 mmol/L, and their mean lung function level, as measured by FEV_1, improved by 5.8 percentage points.

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Our Approach and Leveraging the CFHBE Model

Our programs leverage an industry standard, clinically predictive CFHBE model to measure CFTR protein function *in vitro*. Activity in this model has been shown to be correlated to chloride transport activity, which in turn, has been shown to be correlated to improved lung function in clinical trials designed to evaluate product candidates in CF patients. We have used, and plan to continue to use, insights from the CFHBE model to inform critical pipeline prioritization and development decisions. For example, we selected SION-719 and SION-451 to advance based on their preclinical profiles, including potency in the CFHBE model. We assessed SION-719 and SION-451 in our CFHBE model in direct, head-to-head comparison to ETI, the components of Trikafta. When evaluated in our CFHBE model at E_{max} concentrations, both SION-719 and SION-451, in dual combination with one of our complementary modulators, improved *in vitro* CFTR protein activity to wild-type, or normal, levels. This was a more than 1.5-fold improvement in CFTR protein activity compared to the improvement in such activity observed with ETI at E_{max} in the same experiment. We believe that we can leverage the reproducible correlation between chloride transport at different drug exposure levels in the CFHBE model and clinical outcomes to predict the target level of exposure to achieve clinically meaningful benefit in CF patients.

The CFHBE model uses electrophysiology to evaluate CFTR function by measuring CFTR protein activity in an Ussing Chamber, which is commonly used in CF drug discovery efforts. In this model, human bronchial cells derived from the lungs of CF patients are cultured in a manner to resemble the lung epithelium and to increase their numbers. These cultured cells are then placed in the Ussing Chamber, which uses electrodes to measure ion movement across the membranes of the cultured epithelial cells grown into a monolayer with tight junctions. Cultured CFHBE cells exhibit many of the structural and functional attributes believed to be associated with CF airway disease.

Vertex has successfully applied a variation of the CFHBE model with 20% human serum for multiple CFTR modulators advanced to clinical trials, including their five approved modulators. Importantly, the CFHBE model provided key preclinical data on CFTR function to support the clinical evaluation of elexacaftor as part of Trikafta. Based on publications detailing Vertex's use of the CFHBE model, we believe we conducted our CFHBE model using similar methods and under similar experimental conditions to those Vertex employed. Similar to Vertex, in our model, cell culture media is supplemented with human serum to 20% by volume to estimate the amount of free drug available to engage CFTR in CFHBE cells. This adjustment is designed to simulate the *in vivo* environment, where much of a drug is bound to serum proteins and not available to enter epithelial cells.

We conduct detailed dose-response studies in our CFHBE model to estimate the level of clinical improvement we believe we can achieve with our modulators at specific levels of drug exposure in clinical trials. The addition of human serum helps correct for the high levels of protein binding that are characteristic of our modulators, similar to approved modulators, which we believe enables our model to more accurately predict the clinical exposure required for efficacy. Our CFHBE model accurately predicted the required total plasma concentration of lumacaftor (a component of Orkambi) at its efficacious clinical exposures, as shown in Figure 10 below. We compared lumacaftor's CFTR-dependent chloride transport activity in two CFHBE model variants—with 20% human serum (like our model) and without 20% human serum—and benchmarked these against published Phase 2 clinical results regarding sweat chloride levels for lumacaftor. Figure 10 shows that the predicted lumacaftor dose response, as depicted by the red line, closely matched lumacaftor's clinical dose response, as depicted by the black line. The CFHBE model without 20% human serum, depicted by the blue line, failed to accurately predict the clinical exposure of lumacaftor required for efficacy, even if adjusted for lumacaftor's measured free fraction in human plasma, depicted by the dotted green line.

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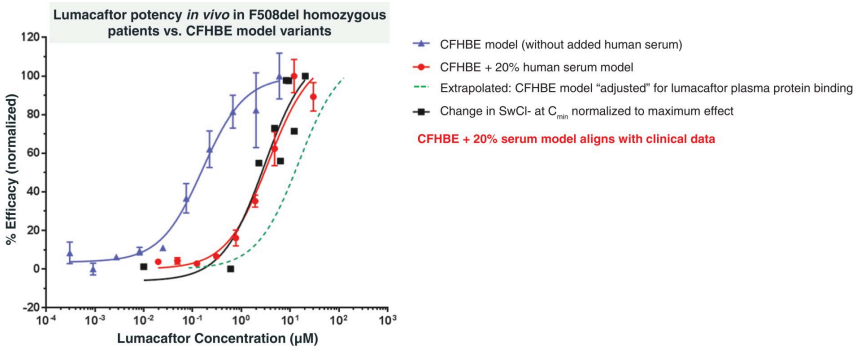
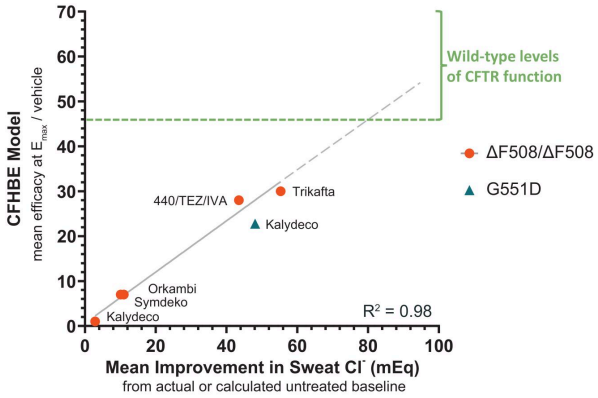
Figure 10. Our CFHBE Model is Designed to More Accurately Predict the Required Clinical Exposure than Standard CFHBE Model



Using published data from the clinical trials of Vertex's approved CFTR modulators, we have validated internally our CFHBE model by assessing the relationship between *in vitro* CFTR protein function, as measured by chloride transport in our CFHBE model, and the mean sweat chloride levels seen in Vertex's clinical trials of these approved modulators.

As shown in Figure 11 below, improvements in CFTR function we observed in our CFHBE model for approved and investigational CFTR modulators have been highly correlated with improvements in sweat chloride measurements from clinical trials for these therapies. Notably, we have used the CFHBE model to predict negative clinical trial outcomes, too. For example, we modeled other third-party modulators previously in development and independently determined that these compounds had insufficient activity to demonstrate target clinical efficacy, which was supported by published clinical trial data.

Figure 11. CFHBE Model Has Been Predictive of Sweat Chloride Improvement



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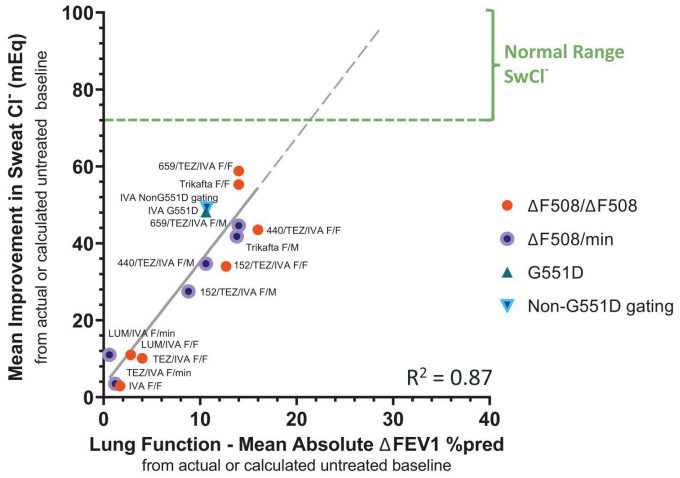
Mean improvements in sweat chloride levels in clinical trials have been shown to be correlated with mean improvements in lung function as measured by FEV_1, as shown in Figure 12 below.

Figure 12. Sweat Chloride Has Been Correlated With Lung Function Benefit



We consider our CFHBE model a translational roadmap because of its ability to correlate with sweat chloride improvement in clinical trials, as seen in Figure 11 above, which in turn has been shown to correlate to improved lung function in clinical trials, as shown in Figure 12 above. We have observed this correlation with our CFHBE data and clinical lung function data for approved and investigational CFTR modulators that demonstrated activity in clinical trials, including galicaftor and navocaftor, as shown in Figure 13 below.

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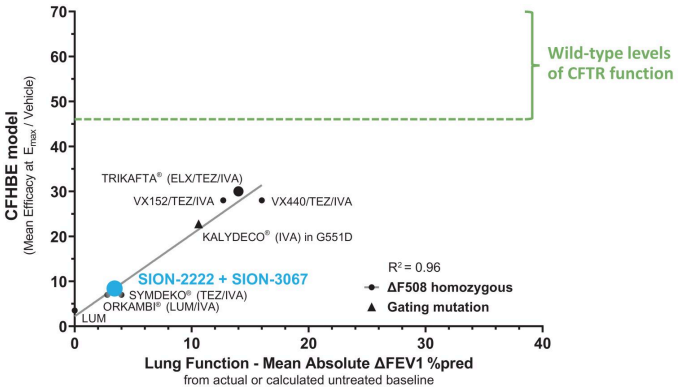
Figure 13. CFHBE Model Correlates to Improved Lung Function



We believe our CFHBE model allows us to predict the target exposure needed for our product candidates to achieve a desired level of clinical activity, such as the level of improvement in patients' lung function, as measured by FEV_1 improvement, and sweat chloride levels. We have tested our product candidates in the CFHBE model at E_{max} and generated a prediction of sweat chloride and FEV_1 changes, based on the documented correlation, observed in clinical trials of approved and investigational modulators, between improvements in sweat chloride levels and improvements in lung function. We believe that the CFTR function improvement observed in our CFHBE model of our product candidates has the potential to translate clinically to deliver clinically meaningful benefit to CF patients.

Our CF Programs

We have a portfolio of NBD1 stabilizers and complementary modulators that target other regions of the CFTR protein, with the goal of advancing a combination therapy that has the potential to deliver clinically meaningful benefit to CF patients. We are pursuing an approach to select the most promising NBD1 stabilizer candidate and NBD1 dual combination to advance into Phase 2 clinical trials in CF patients based on initial human data on safety, tolerability and PK in healthy volunteers.

NBD1 Stabilizer Program

NBD1 is recognized as a key target in treating CF patients with the F508del mutation, as the mutation drives NBD1 instability and defects in its interface assembly. However, NBD1 has presented significant challenges as a drug target due to the attributes of the region's binding sites. Our NBD1 program builds on more than a decade of our cofounders' research, which has combined biophysical, cell-based and virtual screening campaigns, along with extensive use of structural biology. We believe the technical challenge associated with NBD1-targeted drug discovery, along with our intellectual property rights, represent a substantial competitive barrier.

We selected SION-719 and SION-451 for clinical evaluation based on their potency, PK profiles and safety as observed in preclinical studies. We assessed SION-719 and SION-451 in our CFHBE model in direct, head-to-head comparison to ETI, the components of Trikafta. When evaluated in our CFHBE model at their respective E_{max} concentrations, both SION-719 and SION-451, in dual combination with one of our complementary modulators, improved *in vitro* CFTR protein activity of

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F508del-CFTR to wild-type, or normal, levels. This was a more than 1.5-fold improvement in CFTR protein activity compared to the improvement in such activity observed with ETI at E_{max} in the same experiment. Based on predicted exposure levels in the CFHBE model, we believe co-administering SION-719 or SION-451 with a complementary modulator has the potential to achieve clinically meaningful benefit in CF patients.

In addition, we have nominated two NBD1 stabilizers with differentiated profiles as development candidates.

SION-719 and SION-451

Phase 1 Clinical Development

We initiated Phase 1 SAD and MAD clinical trials of SION-719 and SION-451 in healthy subjects in July 2024 and August 2024, respectively. These trials are randomized, doubled-blinded, placebo-controlled trials designed to evaluate safety, tolerability and PK of each product candidate. Both trials are being conducted in Australia. In a Part C of each Phase 1 trial, we plan to evaluate the effect of food on the PK of each product candidate and the bioequivalence of a tablet formation compared to the oral suspension administered in the Phase 1 SAD and MAD trials. We intend to enroll up to 120 healthy volunteers in each trial. We expect topline data for these trials in the first half of 2025.

Interim Phase 1 Trial Data for SION-719

As of January 14, 2025, over 60 healthy subjects have been dosed in the Phase 1 clinical trial of SION-719. The trial was designed to enroll eight subjects, randomized 3:1 active:placebo, in each dosing cohort. Five SAD cohorts have been completed, evaluating single doses of 20 mg, 40 mg, 80 mg, 160 mg and 20 mg taken with food to provide a preliminary assessment of the effect of food on PK. Three MAD cohorts have been completed, evaluating 20 mg, 40 mg and 80 mg of SION-719 twice daily over 10 dosing days, and the next MAD cohort will evaluate 160 mg twice daily over 10 dosing days. We plan to continue enrolling healthy subjects in the trial. All data remain blinded to individual subject treatment assignment, with the exception of selected individual subjects unblinded for administrative and study planning purposes according to the clinical trial protocol.

SION-719 was generally well tolerated at all dose levels administered based on interim Phase 1 clinical data as of the data cutoff date of January 14, 2025. There were no serious adverse events (“SAEs”). Most treatment-emergent adverse events (“TEAEs”) were mild to moderate (Grade 1 or Grade 2). No TEAEs led to the discontinuation of trial drug. The most common TEAEs, occurring in >1 subject, were headache, hypoglycemia and diarrhea. All TEAEs occurring in >1 subject were Grade 1 or Grade 2, except a single Grade 4 hypoglycemia TEAE in a placebo subject in a SAD cohort. There were no TEAEs related to liver function tests. No dose-limiting TEAEs or safety trends of concern have been observed.

Increasing exposure was observed with increasing single and multiple doses. The concentration targets for SION-719 as an add-on to SOC and as part of a dual combination with SION-2222 or SION-109 were achieved with single and multiple doses.

A PK summary of SION-719 is shown in Figures 14 and 15 below. The observed PK was consistent with twice daily (“BID”) dosing.

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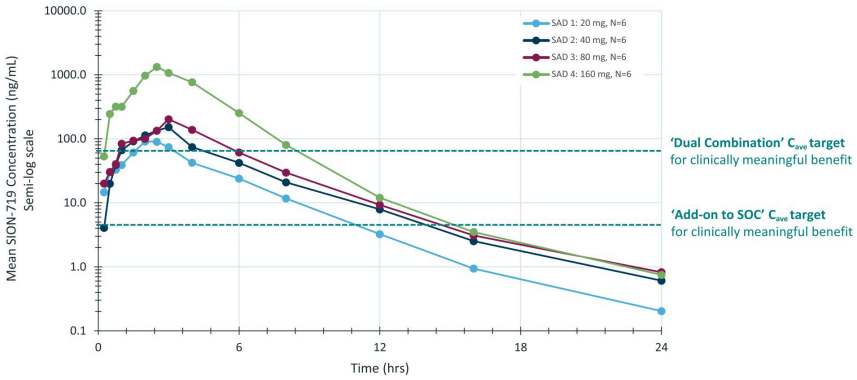
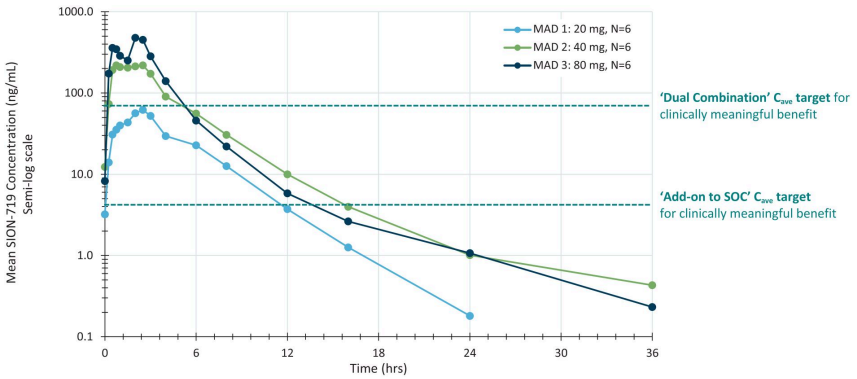
Figure 14. Preliminary Phase 1 PK Summary for SION-719 in the SAD Portion of the Trial



(Each solid line shows mean concentration data from a dosing cohort. Dotted lines represent average PK concentration exposure targets that have the potential, based on our preclinical CFHBE model, to provide clinically meaningful benefit, if SION-719 is administered in a proprietary dual combination with either SION-2222 or SION-109, or as an add-on to SOC.)

Figure 15. Preliminary Phase 1 PK Summary for SION-719 in the MAD Portion of the Trial



(Each solid line shows mean concentration data from a dosing cohort on Day 10. Dotted lines represent average PK concentration exposure targets that have the potential, based on our preclinical CFHBE model, to provide clinically meaningful benefit, if SION-719 is administered in a proprietary dual combination with either SION-2222 or SION-109, or as an add-on to SOC.)

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Interim Phase 1 Trial Data for SION-451

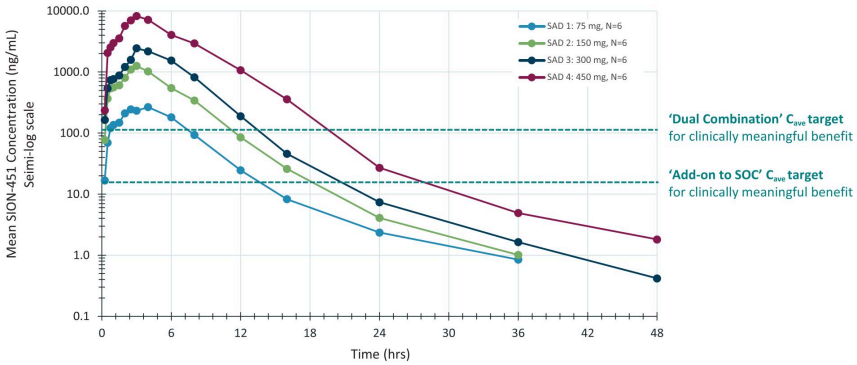
As of January 14, 2025, over 70 healthy subjects have been dosed in the Phase 1 clinical trial of SION-451. The trial was designed to enroll eight subjects, randomized 3:1 active:placebo, in each dosing cohort. Four SAD cohorts have been completed, evaluating single doses of 75 mg, 150 mg, 300 mg and 450 mg. Two additional SAD cohorts have completed dosing with 75 mg or 25 mg with food to provide a preliminary assessment of the effect of food on PK. Three MAD cohorts have been completed, evaluating 75 mg, 150 mg and 300 mg of SION-451 twice daily over 10 dosing days, and the next MAD cohort will evaluate 225 mg twice daily over 10 dosing days. We plan to continue enrolling healthy subjects in the trial. All data remain blinded to individual subject treatment assignment.

SION-451 was generally well tolerated at all dose levels administered based on interim Phase 1 clinical data as of the data cutoff date of January 14, 2025. There were no SAEs, and most TEAEs were mild to moderate (Grade 1 or Grade 2). No TEAEs led to the discontinuation of trial drug. The most common TEAEs, occurring in more than one subject, were headache, abdominal pain, contact dermatitis, influenza, presyncope, fatigue and upper respiratory tract infection. All TEAEs occurring in >1 subject were Grade 1 or Grade 2, and many were in an isolated dose cohort that was impacted by an outbreak of respiratory infection in the Phase 1 unit. One Grade 1 TEAE of increased transaminases has been observed, in a subject with influenza A infection. No dose limiting TEAEs or safety trends of concern have been observed.

Increasing exposure was observed with increasing single and multiple doses. The concentration targets for SION-451 as both an add-on to SOC and as part of a dual combination with SION-2222 or SION-109 were achieved with single and multiple doses.

A PK summary of SION-451 is shown in Figures 16 and 17 below. The observed PK was consistent with BID dosing.

Figure 16. Preliminary Phase 1 PK Summary for SION-451 in the SAD Portion of the Trial



(Each solid line shows mean concentration data from a dosing cohort. Dotted lines represent average PK concentration exposure targets that have the potential, based on our preclinical CFHBE model, to provide clinically meaningful benefit, if SION-451 is administered in a proprietary dual combination with either SION-2222 or SION-109, or as an add-on to SOC.)

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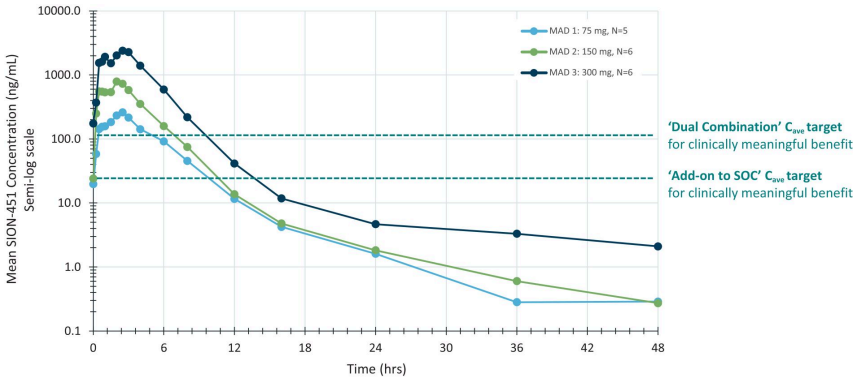
Figure 17. Preliminary Phase 1 PK Summary for SION-451 in the MAD Portion of the Trial

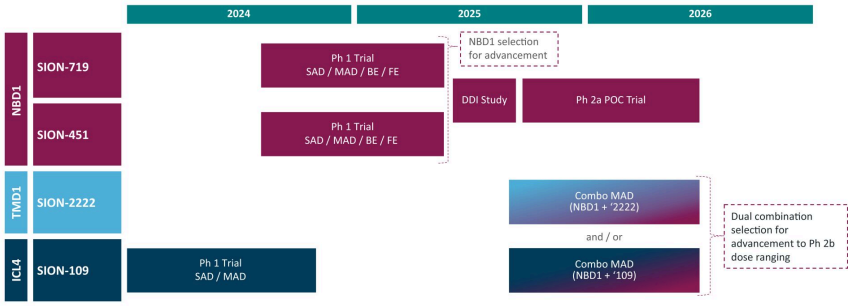


(Each solid line shows mean concentration data from a dosing cohort on Day 10. Dotted lines represent average PK concentration exposure targets that have the potential, based on our preclinical CFHBE model, to provide clinically meaningful benefit, if SION-451 is administered in a proprietary dual combination with either SION-2222 or SION-109, or as an add-on to SOC.)

Phase 2 Clinical Development Plans

Based on our current clinical development strategy, we anticipate conducting a Phase 2a add-on to Trikafta proof-of-concept trial and a Phase 2 dual combination treatment trial after the completion of our ongoing Phase 1 trials of SION-719 and SION-451. Our current expectations for our clinical development strategy are depicted in Figure 18 below.

Figure 18. Our Strategic Development Plan to Advance Our Pipeline of NBD1 Stabilizers and Complementary Modulators



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Phase 2a Proof-of-Concept Clinical Trial—NBD1 stabilizer + Trikafta

Following completion of our ongoing Phase 1 clinical trials of SION-719 and SION-451, we plan to select a lead NBD1 stabilizer and conduct a drug-drug interaction trial before initiating a Phase 2a proof-of-concept trial in CF patients. We expect the Phase 2a trial to be a two-way crossover trial in which we enroll up to 20 trial subjects with CF who are stable on physician-prescribed Trikafta. All trial subjects would be randomly allocated to one of two trial arms and continue taking Trikafta throughout the trial. Trial subjects in "Arm A" would first receive Trikafta in combination with our lead NBD1 stabilizer for 14 days, and, after a 28-day washout period, would then receive Trikafta in combination with placebo for 14 days. Trial subjects in "Arm B" would receive the same treatments in reverse order. All subjects would have a 28-day safety follow-up period. We expect to select safety as the primary endpoint, and PK and improvements to sweat chloride levels as the secondary endpoints. We expect to initiate the Phase 2a clinical trial in the second half of 2025.

Combination MAD Clinical Trials—NBD1 stabilizer + galicaftor or SION-109

Following completion of our ongoing Phase 1 clinical trials of SION-719 and SION-451 and completion of combination toxicology studies, and our selection of a lead NBD1 stabilizer, we intend to begin one or two separate MAD trials of dual combinations, assessing the safety, tolerability and PK of our lead NBD1 stabilizer in dual combination with galicaftor and/or SION-109 in healthy volunteers. We anticipate initiating these combination MAD trials in the second half of 2025. Following the completion of such trials, we intend to select one dual combination to advance into Phase 2b dose-ranging trials in CF patients.

Preclinical Studies

We have used numerous complementary methods to validate and characterize the activity of our compounds. In addition to the CFHBE model, we have utilized differential static light scattering ("DSLS") experiments to measure the thermal stability of the NBD1 protein, surface plasmon resonance ("SPR") studies to evaluate the interaction of SION-719 and SION-451 with the NBD1 domain, and CFTR western blot analysis to assess the impact of NBD1 stabilizers on the folding, maturation and stability of F508del-CFTR. Given the results of these preclinical studies, we believe that we have identified highly potent NBD1 stabilizers with robust preclinical activity.

SION-719 and SION-451 Increased NBD1 Thermal Stability

In preclinical studies, SION-719 and SION-451 increased the stability of the NBD1 domain as measured using DSLS experiments. Light scattering provides a measure of the temperature at which a protein unfolds. As the temperature rises, the CFTR protein unfolds and creates aggregates that interfere with the passage of light. These preclinical studies evaluated the change in temperature that the protein aggregated as the ratio of the compound to CFTR protein increased, which is an indicator of stability. We also evaluated ETI in these preclinical studies, and we found that they had no effect on NBD1 thermal stability, as measured by light scattering techniques. Restoration of NBD1 thermal stability has been highlighted as being both necessary and sufficient to correct F508del CFTR folding and assembly. Both SION-719 (Figure 19, left) and SION-451 (Figure 19, right) increased F508del-NBD1 stability by approximately 16°C. These preclinical models show the ability of SION-719 and SION-451 to improve the stability of NBD1 as compared to ETI, as measured by light scattering techniques.

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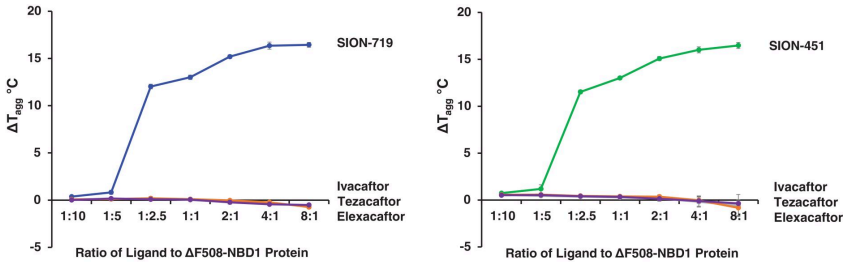
Figure 19. SION-719 and SION-451 Increased Stability of the NBD1 Domain



ΔT_{agg} °C = Change in the temperature at which F508del-NBD1 protein unfolds and aggregates
***N=4,** error bars represent standard deviation*

SION-719 and SION-451 Bound to NBD1 with High Affinity

To evaluate the binding ability of our stabilizer candidates, we employed SPR studies to evaluate the interaction of SION-719 and SION-451 with the NBD1 domain. SPR studies measure changes in the mass of biomolecules immobilized on a metal film. When a small molecule ligand binds to the immobilized target protein, the refractive index of the metal film changes, resulting in a changed reflection angle of light. In these studies, we found that the strength of the binding interaction was high; the binding affinity of SION-719 to F508del-NBD1 was approximately 4.3 nM and the binding affinity of SION-451 to F508del-NBD1 was approximately 2.4 nM, each expressed as a K_D value.

SION-719 and SION-451 Restored F508del Folding and Maturation

In preclinical studies, we evaluated SION-719 and SION-451 in various combinations, including with ETI, galicaftor and SION-109, to assess these combinations' ability to improve CFTR folding, maturation and stability and thereby correct F508del-CFTR. Preclinical studies with both SION-719 and SION-451-based combinations resulted in F508del-CFTR maturation to levels that are similar to wild-type CFTR. We believe these results demonstrated potential synergy between an NBD1 stabilizer and these complementary modulators.

The results of studies of CFTR maturation in human CF submucosal gland epithelial ("CFSMEo") cells by western blot with SION-719 are shown in Figure 20 (top), and the results with SION-451 combinations are shown in Figure 20 (bottom). Together, the western blots illustrate F508del-CFTR protein in a submucosal-gland epithelial cell line that expresses CFTR, treated with various combinations of CFTR modulators. The high molecular weight bands, labeled C-band, indicate the presence of the active, mature apical glycoform of CFTR (dark bands), which is responsible for CFTR channel function. The compounds were evaluated at their E_{max}.

In Figure 20, the far-left column in the C-band is F508del in dimethylsulfoxide ("DMSO") alone, showing no mature protein, and the far-right column is wild-type CFTR in DMSO with a dark bank indicating the presence of mature CFTR protein. As seen in Figure 20 (top), dual combinations of SION-719 with galicaftor or SION-109, or the addition of SION-719 to ETI, resulted in wild-type levels of corrected F508del-CFTR protein. Treatment with SION-719 as a single agent demonstrated a greater effect on F508del maturation than ETI at E_{max}. On the other hand, there was little improvement in the maturation of F508del-CFTR protein with galicaftor alone, SION-109 alone or ETI at their respective E_{max} concentrations, as indicated by the light gray bands. The results with SION-451

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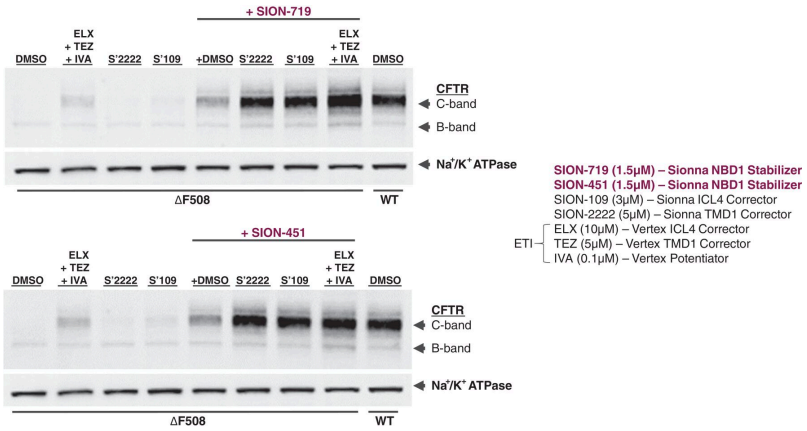
presented in Figure 20 (bottom) are similar. The western blots below demonstrate the importance of NBD1 stabilization to the correction of F508del maturation.

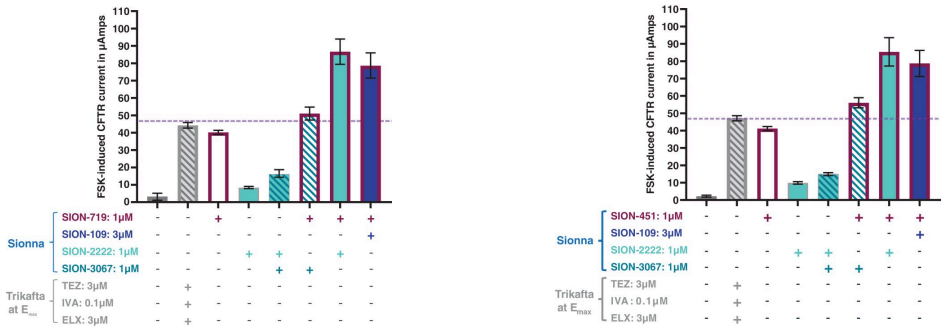
Figure 20. F508del-CFTR Maturation in CFSMEo- Cells Was Fully Restored with Our SION-719 and SION-451 Corrector Combinations



SION-719 and SION-451 Normalized CFTR Function in CFHBE Model at E_{max}

In our CFHBE model, SION-719 and SION-451 as single agents at E_{max} improved F508del-CFTR activity to levels observed near those with triple combination ETI at E_{max}. The results of the SION-719 and SION-451 studies are shown in Figure 21. In Figure 21, CFTR activity is expressed as a ratio relative to ETI at E_{max}. The vertical bars represent the mean CFTR channel activity (+/- standard error of eight replicates) from a representative study in homozygous F508del CFHBE cells in response to the treatments indicated. The dashed line represents the average response to ETI in each study.

Figure 21. SION-719 and SION-451 Were Highly Active in the CFHBE Model at E_{max}



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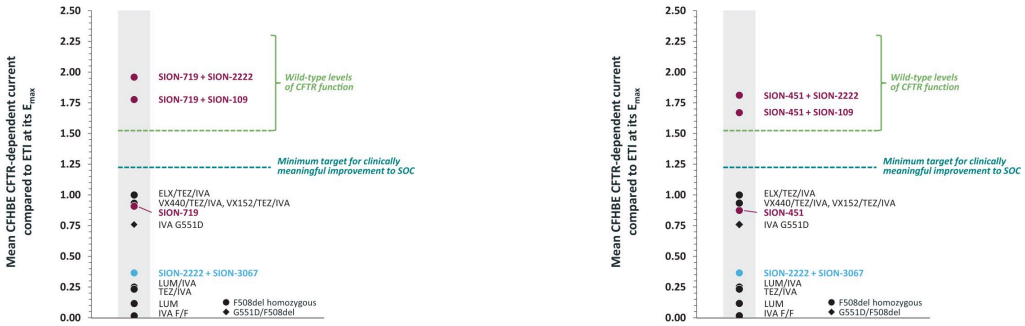
In our CFHBE model, both SION-719 and SION-451, in dual combinations with galicaftor or SION-109, improved *in vitro* F508del-CFTR activity to wild-type levels, when administered at E_{max}. This was a more than 1.5-fold improvement in CFTR protein activity compared to the improvement in such activity observed with ETI at E_{max} in the same experiment. As noted above, improvements in CFTR function we observed in our CFHBE model have been highly correlated with mean improvements in sweat chloride levels and mean improvements in lung function.

Figure 22. SION-719 and SION-451 Combinations with Galicaftor or SION-109 Were Highly Active in the CFHBE Model at E_{max}



Based on our findings in these preclinical studies and the strong correlation between chloride conductance and improved clinical activity, we believe that both SION-719 and SION-451, in combination with a complementary CFTR modulator, have the potential to achieve significant improvement in CFTR function, as measured by sweat chloride levels and lung function, and thereby have the potential to lead to clinically meaningful benefit for CF patients. However, the results observed from our preclinical studies may not necessarily be predictive of clinical outcomes, and actual outcomes may differ. We will need to complete additional clinical studies to determine the safety and efficacy of SION-719 and SION-451.

Setting Initial Clinical Exposure Targets Based on CFHBE Model

To set total plasma concentration targets in our Phase 1 clinical trials with SION-719 and SION-451 in healthy volunteers, we conducted multiple dose response studies of both compounds in our CFHBE model that assessed F508del-CFTR activity as a function of each compound's concentration in cell culture media supplemented with human serum to 20% by volume. We selected a target exposure for our Phase 1 trials based on an average of these preclinical dose response studies along with studies to define an adequate safety margin. Figure 23 below presents illustrative dose responses of SION-719, as a single agent and in combination with ETI, SION-109 or galicaftor (SION-2222), as a function of the fold efficacy (CFTR activity) of ETI at increasing dose concentrations, in CFHBE cells from single donors. The black dotted horizontal line indicates ETI's improvement in F508del-CFTR activity at E_{max}, the teal horizontal line indicates our minimum target for clinically meaningful improvement in F508del-CFTR activity based on our CFHBE assay, and the green horizontal line represents the lower bound of the CFTR activity range observed across a panel of eight CFTR wild-type CFHBE donors. The X-axis shows increasing drug concentrations on a logarithmic scale. A roughly two-fold increase over ETI, as seen with SION-719 treatment at its E_{max} in each of the three combinations, is in the range of wild-type channel activity.

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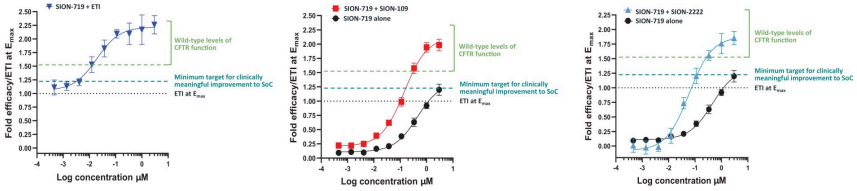
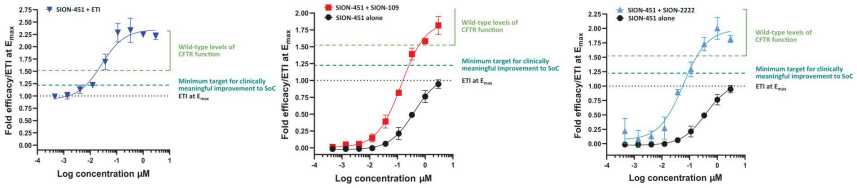
Figure 23. Representative ΔF508/ΔF508 CFHBE Dose-Response of SION-719 as Single Agent and in Combination with ETI, SION-109 or SION-2222



(ETI = 3 μM Elexacaftor + 45 μM Tezacaftor + 0.3 μM Ivacaftor. SION-109 and SION-2222 were used at 3 μM.)

Figure 24 below presents illustrative dose responses of SION-451, as a single agent and in combination with ETI, SION-109 or galicaftor (SION-2222), as a function of the fold efficacy (CFTR activity) of ETI at increasing dose concentrations, in CFHBE cells from single donors. A roughly two-fold increase over ETI, as seen with SION-451 treatment at its E_{max} in each of the three combinations, is in the range of wild-type channel activity.

Figure 24. Representative ΔF508/ΔF508 CFHBE Dose-Response of SION-451 as Single Agent and in Combination with ETI, SION-109 or SION-2222



(ETI = 3 μM Elexacaftor + 45 μM Tezacaftor + 0.3 μM Ivacaftor. SION-109 and SION-2222 were used at 3 μM.)

These dose response curves illustrate that our NBD1 stabilizers work synergistically with complementary modulators, and with the standard of care, to significantly improve CFTR function in preclinical models. Given the correlation seen in our preclinical studies between CFTR function and clinical activity, we believe that achieving target exposure levels for our product candidates in our ongoing and future clinical trials has the potential to translate to significant improvements in sweat chloride and lung function, as measured by FEV_1, in CF patients.

Preclinical Safety/Pharmacology and Toxicology to Support Clinical Trials

For both SION-719 and SION-451, we conducted standard *in vitro* and *in vivo* toxicology and safety pharmacology studies necessary to support first in human studies. The preclinical study results supported early clinical testing above exposure levels that we have predicted can be effective.

Additional NBD1 Stabilizer Candidates

Our portfolio includes additional NBD1 stabilizer candidates with differentiated profiles from SION-719 and SION-451.

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We have nominated two additional NBD1 stabilizers as development candidates.

Complementary Programs

We have established a pipeline of proprietary complementary modulators, representing three different mechanisms of action, TMD1-directed correctors, an ICL4-directed corrector, and a potentiator, that can potentially be combined with our NBD1 stabilizers. Our two prioritized clinical-stage complementary modulators are galicaftor (SION-2222) and SION-109. Following completion of our NBD1 stabilizer Phase 1 clinical trials, we plan to evaluate galicaftor and/or SION-109 in dual combination with our selected lead NBD1 stabilizer in Phase 1 MAD trials in healthy volunteers.

Our portfolio includes additional complementary compounds that we may advance for future combination development with an NBD1 stabilizer, including navocaftor, which has been evaluated in Phase 1 and Phase 2 clinical trials, including in combination with galicaftor, provides our pipeline with a third mechanism of action complementary to NBD1 stabilizers and future opportunities to develop additional combination products to potentially expand our CF franchise.

Clinical Data — Complementary Modulators

TMD1 Programs—Galicaftor (SION-2222) and SION-2851

In a Phase 2 clinical trial conducted by AbbVie prior to our in-licensing transaction, galicaftor demonstrated clinical activity in improving sweat chloride and lung function as part of a combination trial with navocaftor. Galicaftor has been evaluated in Phase 1, Phase 1b and Phase 2 clinical trials involving healthy subjects and CF patients. Galicaftor was generally well-tolerated at all doses administered. The majority of adverse events were mild to moderate in severity. No serious adverse events were reported in healthy volunteers, and among CF patients, serious adverse events were reported infrequently and consisted of common manifestations of the underlying CF disease. Based on galicaftor's preclinical profile, we do not expect significant PK or drug/drug interactions with our NBD1 stabilizers or other complementary modulators. The PK profile of galicaftor in CF patients was similar to that observed in healthy volunteers. The activity of galicaftor in patients with CF has been evaluated in three randomized, double-blind, placebo-controlled Phase 2 trials, as summarized below.

Trials GLPG-2222-CL-201 and GLPG-2222-CL-202 had at least 80% power to detect selected changes in sweat chloride. In clinical trials, least squares means, or "LS means," represent the average predicted values of the outcome variable in a statistical model and estimate the effect of a treatment while controlling for other covariates that may influence the outcome. These means provide a clearer comparison between treatment groups by minimizing potential biases from imbalanced covariates. A confidence interval ("CI") is a range of values, derived from the sample data, that is believed to contain the treatment effect with a specified level of confidence, usually 95%. In clinical trials, the p-value quantifies the probability of observing the trial results (or more extreme results) assuming there is no effect or no difference between treatment groups. A p-value of < 0.05 is generally considered statistically significant, meaning that the probability of the results occurring by chance alone is less than five percent.

- Trial M19-530 was a Phase 2 dose-ranging trial conducted by AbbVie in Europe and the U.S. to evaluate the safety, tolerability and efficacy of navocaftor alone and in combination with galicaftor in 76 CF patients that were homozygous for F508del mutation. The primary efficacy endpoint of this trial was absolute change in lung function ($ppFEV_1$) from baseline (day 1) through day 29. The trial was completed in June 2022.
  - This trial included eight total arms: a single placebo arm, two arms with different doses of navocaftor in combination with placebo for galicaftor, and five treatment arms across a range

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of doses of galicaftor (doses 10 mg, 30 mg, 100 mg, 200 mg, 300 mg once daily (“QD”)) in combination with navocaftor 150 mg for 28 days.

- The mean absolute change from baseline in $ppFEV_1$ from baseline through Day 29 ranged from -0.1 to 3.5 percentage points for the five dual combination dose groups (navocaftor 150mg and galicaftor across a range of doses). The least squares means (“LS means”) change from baseline in ppFEV1 was statistically significant in the 200mg and 300mg galicaftor combination groups, 3.5 $ppFEV_1$ ($p<0.05$) and 3.1 $ppFEV_1$ ($p<0.05$), respectively. These data are from the analysis in which all spirometry values were used, regardless of usage status of bronchodilator or airway clearance regimen before spirometry collection. LS means with standard errors (“SE”) and p-values are from mixed-effect model repeat measures analysis.
- AbbVie also assessed sweat chloride improvement as a secondary efficacy endpoint. Mean SwCl improvement occurred in all groups with galicaftor 30 mg or higher, with improvement of 18.6 mmol/L in the 200 mg group ($p<0.001$) and 19.9 mmol/L in the 300 mg group ($p<0.001$).
- Galicaftor was generally well-tolerated in combination with navocaftor in this trial. Most reported AEs were mild to moderate in severity. Two serious adverse events (“SAEs”) occurred in two subjects receiving galicaftor in combination with navocaftor (ileus and cholecystitis acute); both were considered unrelated to the trial drugs by the investigator.

- Trial GLPG-2222-CL-202 was a Phase 2 dose-ranging trial conducted by Galapagos in Europe and the U.S. to evaluate the safety and tolerability and the effect on CFTR function (as assessed by sweat chloride), pulmonary function and the Cystic Fibrosis Questionnaire—Revised (“CFQ-R”), which measures health-related quality of life, of galicaftor in 59 CF patients that were homozygous for F508del mutation. The trial was completed in October 2017.
  - Four doses of galicaftor were tested in this trial (50 mg, 100 mg, 200 mg, 400 mg QD) over 29 days.
  - Galicaftor was generally well-tolerated in this trial. The majority of reported treatment-emergent AEs were mild or moderate in severity. A total of four SAEs were reported (two after galicaftor, two after placebo) in two subjects in the pooled placebo and one subject in the galicaftor 100 mg QD treatment group, respectively. The three subjects experienced one or two events of infective pulmonary exacerbation of CF, all of which were considered not related to trial drug.
  - Sweat chloride levels, lung function ($ppFEV_1$) and CFQ-R were also assessed as secondary endpoints. Mean sweat chloride concentrations decreased dose-dependently with increasing doses of galicaftor, with a maximum decrease observed in the 200 mg QD treatment group on days 15 and 29, with statistically significant LS means differences compared to placebo of -11.2 (95% CI, -19.1; -3.3; $p=0.0062$) and -15.8 (95% CI, -23.2; -8.3; $p<0.0001$) mmol/L, respectively. After termination of the trial drug, mean sweat chloride concentrations returned to baseline values in all treatment groups. Percent predicted FEV_1 and CFQ-R did not significantly improve in any group.
- Trial GLPG-2222-CL-201 was a Phase 2 trial conducted by Galapagos in Europe and Australia that evaluated the safety and tolerability and the effect on CFTR function (sweat chloride), lung function ($ppFEV_1$) and CFQ-R of galicaftor in 37 CF patients that were heterozygous for F508del and a gating mutation, receiving ivacaftor. The trial was completed in August 2017.
  - Two doses of galicaftor were tested in this trial (150 mg or 300 mg QD) over 29 days.
  - Galicaftor was well-tolerated in this trial. Most treatment-emergent AEs were mild in severity, and there were no SAEs.

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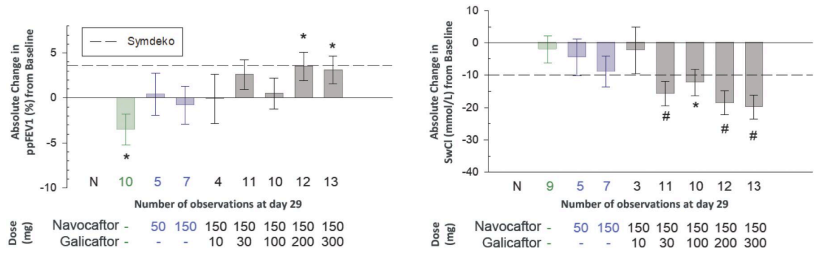
- Changes from baseline in sweat chloride, ppFEV$_1$ and CFQ-R at day 29 were also assessed as secondary endpoints. The galicaftor 300 mg QD treatment group had a statistically significant LS means with CI difference compared to placebo of -11.7 (95% CI, -21.1; -2.2) mmol/L (p=0.0170). There were no statistically significant changes in sweat chloride in the 150 mg and 300 mg treatment groups. Lung function and CFQ-R did not significantly improve in any group. All results are described for the modified intent to treat population, which excluded one subject who received an incorrect trial kit.

These Phase 2 results demonstrated that galicaftor as a single agent increased CFTR activity in patients with the F508del mutation. The activity of galicaftor in combination with navocaftor in Trial M19-530 was similar to the activity of approved dual combination modulators, as seen via indirect, cross-trial comparisons and as predicted based on our CFHBE model (Figure 25). These data supported the selection of a 200 mg dose QD of galicaftor for subsequent trials in combination with navocaftor.

Figure 25. Galicaftor + Navocaftor Combination Showed Similar Activity to Symdeko in Phase 2 Cross-Trial Comparison



Data presented as LS mean ± standard error. * p < 0.05; # p < 0.001

In addition to galicaftor, we licensed SION-2851 from AbbVie in July 2024. SION-2851 is a potent TMD1-directed corrector that has completed a Phase 1 SAD trial in 16 healthy volunteers. The trial was conducted by AbbVie and Galapagos in Belgium in 2018. The primary endpoint was safety and tolerability, assessed by the number of subjects with adverse events. Based on its mechanism of action and preclinical studies, we believe it may be potentially synergistic with NBD1 stabilizers.

ICL4 Program—SION-109

We are also advancing SION-109, which targets the ICL4 interface, for development in combination with an NBD1 stabilizer.

In December 2024, we completed our evaluation of SION-109 in a randomized, double blind, placebo-controlled Phase 1 clinical trial in healthy subjects, following clearance of its Investigational New Drug application (“IND”) by the U.S. Food and Drug Administration (the “FDA”) in June 2023. The trial was conducted in the U.S. and was designed to evaluate the safety, tolerability and PK profile of single and multiple ascending doses of SION-109, administered as an oral suspension. In a Part C of the Phase 1 trial, we also evaluated the effect of food on PK and the bioequivalence of a tablet formation compared to the oral suspension used in the Phase 1 SAD and MAD trials.

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102 healthy adult subjects were dosed in this Phase 1 trial. The trial was designed to enroll 8 subjects, randomized 3:1 active:placebo, to each dosing cohort. Six SAD cohorts evaluated single doses from 50 mg to 400 mg. Five MAD cohorts evaluated from 50 mg to 150 mg twice daily over 10 dosing days. 15 subjects enrolled in the open-label food effect and tablet bioequivalence part evaluating 100 mg single doses each of suspension fasted, tablet fasted, and tablet fed.

SION-109 was generally well tolerated at all dose levels administered in all parts of this Phase 1 trial. A summary table of reported TEAEs is shown in Figure 26 below. There were no SAEs, and most TEAEs were mild to moderate (Grade 1 or Grade 2). No TEAEs led to the discontinuation of trial drug, and no dose-limiting TEAEs were observed. Sporadic increases in potassium were observed that were determined to be related to sample collection and/or processing. A 150 mg MAD cohort was repeated with revised sampling guidance, and no increased potassium values were observed. Isolated and transient increases in transaminases were observed associated with four AEs in three subjects in the MAD and Part C (one Grade 1 AE, two Grade 2 AEs and one Grade 3 AE) whose values returned to the normal range in follow-up. Other liver function tests, including bilirubin, were unremarkable. There were no clinically meaningful, treatment-emergent trends in other safety parameters, vital signs or electrocardiograms.

Figure 26. Phase 1 TEAEs for SION-109 in the MAD Portion of the Trial

	Placebo *(n=9)*	Cohort 1 50 mg *(n=6)*	Cohort 2 100 mg *(n=6)*	Cohort 3 150 mg *(n=6)*	Cohort 4 75 mg *(n=7)*	Cohort 5 150 mg *(n=6)*	MAD Total *(n=40)*
Any TEAE, n (%)	**4 (44)**	**4 (67)**	**3 (50)**	**3 (50)**	**0**	**1 (17)**	**15 (38)**
Mild (Grade 1)	2 (22)	-	-	1 (17)	-	-	3 (8)
Moderate (Grade 2)	2 (22)	4 (67)	2 (33)	1 (17)	-	1 (17)	10 (25)
Severe (Grade 3)	-	-	1 (17)	1 (17)	-	-	2 (5)
Life-threatening (Grade 4)	-	-	-	-	-	-	-
Leading to treatment discontinuation	-	-	-	-	-	-	-
Serious TEAEs, n (%)	**-**	**-**	**-**	**-**	**-**	**-**	**-**
Most frequent TEAEs (>1 subject), n (%)							
Headache	2 (22)	2 (33)	-	-	-	1 (17)	5 (13)
Abdominal pain	-	-	1 (17)	1 (17)	-	-	2 (5)
Alanine aminotransferase increased	-	-	1 (17)	1 (17)	-	-	2 (5)
Constipation	-	1 (17)	1 (17)	-	-	-	2 (5)
Diarrhea	-	-	1 (17)	1 (17)	-	-	2 (5)
Myalgia	1 (11)	-	1 (17)	-	-	-	2 (5)

(Safety observations in the SAD and Part C portions of the trial were generally consistent with the MAD findings shown.)

Increasing exposure was observed with increasing single and multiple doses. The target exposure for SION-109 as part of a dual combination with SION-451 or SION-719 was achieved with multiple doses of 75 mg BID and higher doses.

A PK summary of SION-109 is shown in Figure 27 below. The PK observed was consistent with BID dosing.

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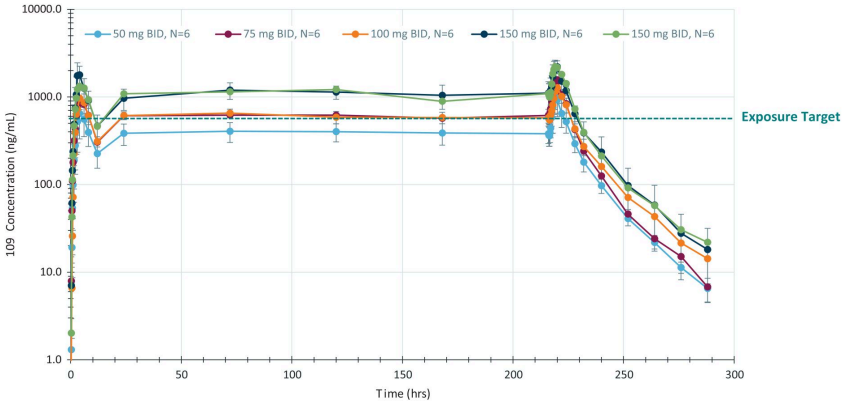
Figure 27. Phase 1 PK Summary for SION-109 in the MAD Portion of the Trial



(Each solid line shows mean concentration data for a dosing cohort over 10 days of dosing. Data points for Day 2 through Day 9 are trough (pre-dose) concentrations. The dotted line represents the $C_{minimum}$ (trough) PK exposure target for SION-109, with the aim to achieve targeted exposure to deliver clinically meaningful benefit when administered in a proprietary dual combination with SION-451 or SION-719, based on CFHBE assay data.

PK observations in the SAD and Part C portions of the trial were generally consistent with the MAD findings shown.)

Potentiator Program—Navocaftor (SION-3067)

Navocaftor is a clinical-stage potentiator of CFTR gating activity that we have licensed from AbbVie. Navocaftor provides our pipeline with a third mechanism of action complementary to NBD1 stabilizers and future opportunities to develop additional combination products to potentially expand our CF franchise. Navocaftor has completed Phase 1, Phase 1b and Phase 2 trials in over 300 subjects and was generally well-tolerated in CF subjects and healthy volunteers, with improvements observed in sweat chloride levels in combination with galicaftor.

In Phase 1 trials evaluating safety and tolerability in healthy subjects, navocaftor was generally well-tolerated in combination with galicaftor, in each case at doses up to 500 mg twice daily for 14 days. These trials were conducted in Europe and the U.S. In addition, navocaftor has been generally well-tolerated in both CF subjects and healthy volunteers. When navocaftor was given as a monotherapy or in combination with galicaftor, all AEs were mild to moderate. The PK profile in CF patients was similar in healthy volunteers.

The activity of navocaftor in patients with CF was evaluated in a randomized, double-blind, placebo-controlled Phase 2 trial in combination with a galicaftor dose range from 10 mg to 300 mg QD, as summarized above. Combination treatment of navocaftor 150 mg QD with galicaftor resulted in improvements in FEV_1 and sweat chloride levels in the homozygous F508del population. As expected, treatment with 50 mg QD or 150 mg QD navocaftor monotherapy for 28 days did not result in improvements in FEV_1 or sweat chloride levels.

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Manufacturing

We have leveraged multiple third-party manufacturers to support the manufacturing of our product candidates for clinical trials and, if we receive regulatory approval, we intend to rely on third parties for commercial manufacture. We do not own or operate, and currently have no plans to establish, any manufacturing facilities. We expect this strategy will enable us to maintain a more efficient infrastructure, outsourcing instead of building manufacturing and supply chain capabilities, while simultaneously enabling us to focus our expertise on the clinical development of our product candidates. We expect to enter into commercial supply agreements with such manufacturers prior to any potential approval of our product candidates.

Commercialization

We have exclusive worldwide commercial rights to our product candidates. Given our stage of development, we have not yet established a commercial organization or distribution capabilities. The CF patient populations are well-characterized and clearly identified in the U.S., Canada, Europe and several other regions around the world, with highly active and informed CF patient advocacy groups. Most CF patients are treated at a limited number of centralized CF patient care centers by a team of healthcare professionals who are experts in and dedicated to treating CF.

We plan to independently commercialize our products, if approved, in the U.S. and other regions where we determine it makes commercial sense to do so. Given the established CF patient care centers and identified teams of healthcare professionals, we believe we could commercialize our product(s) for CF with a relatively small specialty sales force that calls on a limited and focused group of prescribing healthcare professionals. At the appropriate time, we will recruit a sales force and a medical affairs team and take other steps to establish the necessary commercial infrastructure. As product candidates advance through our pipeline, our plans may change.

Competition

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. While we believe we have competitive advantages, we face substantial competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic research institutions and governmental agencies, and public and private research institutions. This may include other small-molecule drug discovery companies using similar approaches or other types of therapies, such as small molecule, gene therapy, gene editing and/or mRNA therapies.

In particular, we expect to compete with Vertex, which has multiple approved products, as well as additional product candidates in development, for the treatment of CF that would compete with our product candidates, if approved. Vertex is the manufacturer of the five approved CFTR modulators, including the standard of care, Trikafta, a triple combination therapy approved for patients with at least one F508del mutation or responsive mutations based on *in vitro* data. Vertex's marketed products generated approximately $10 billion in sales in 2023. In addition, in December 2024, Vertex received approval from the FDA for a second-generation, triple modulator combination, Alyftrek, for treatment of CF in patients aged six years and older who have at least one F508del mutation or another responsive mutation in the CFTR gene. Alyftrek demonstrated non-inferiority to Trikafta in the primary endpoint of two Phase 3 clinical trials, providing patients with similar FEV_1 as Trikafta and sweat chloride improvements of 3 to 8 mmol/L. Any product candidates that we successfully develop and commercialize will compete with these existing therapies, as well as any new therapies that may become available in the future that are approved to treat the same diseases for which we may obtain approval for our product candidates.

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We may also face competition from other companies attempting to develop therapeutics targeting CF. For example, in August 2023, HIT-CF Europe announced plans to initiate a 52-patient trial of Fair Therapeutics, Inc.'s CFTR modulators in select European CF patients based on organoid response to the experimental combination. Multiple companies are developing CF-utilizing nucleic acid therapies, which are compounds that allow expression of a functional CFTR protein and are relevant for the more than 5,000 people with CF who cannot make full-length CFTR protein and are not eligible for CFTR modulators. In addition, multiple companies are developing candidates for gene therapy approaches to treat CF.

Some of our competitors have significantly greater financial resources than we do and an established presence in the market. Our competitors may have greater expertise in research and development, manufacturing, obtaining regulatory approvals and marketing approved products and may obtain regulatory approvals for their products more rapidly than we can, if at all. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. We also compete with these companies in recruiting, hiring and retaining qualified scientific and management talent, establishing clinical trial sites and patient registration for clinical trials and obtaining manufacturing slots at contract manufacturing organizations.

If our product candidates do not offer advantages over available products, we may not be able to successfully compete against current and future competitors. The key factors affecting the success of our products, if approved, are likely to be their potential efficacy, safety, convenience and availability of reimbursement.

Intellectual Property

We strive to protect and enhance the intellectual property and proprietary technology that we consider important to our business, including by pursuing patent applications that cover our product candidates and methods of using the same, as well as any other relevant inventions and improvements that we believe to be commercially important to the development of our business. We also rely on trade secrets relating to our proprietary know-how, continuing innovation and in-licensing opportunities to develop, strengthen and maintain our proprietary and intellectual property position. We additionally may rely on regulatory protection afforded through data exclusivity, market exclusivity and patent term extensions, where available.

Our commercial success may depend in part on our ability to: obtain, maintain, enforce and protect our intellectual property and other proprietary rights for commercially important technology, inventions and know-how related to our business; defend and enforce any patents we may own or in-license in the future; prevent others from infringing any patents we may own or in-license in the future; preserve the confidentiality of our trade secrets; and operate without infringing the valid enforceable patents and proprietary rights of third parties. Our ability to limit third parties from making, using, selling, offering to sell or importing our products may depend on the extent to which we have rights under valid and enforceable licenses, patents or trade secrets that cover these activities. In some cases, enforcement of these rights may depend on third party licensors. With respect to both licensed and company-owned intellectual property, we cannot be sure that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications filed by us in the future, nor can we be sure that any of our existing patents or any patents that may be granted to us in the future will be commercially useful in protecting our commercial products and methods of manufacturing the same.

Patent expiration dates noted in the following paragraphs refer to statutory expiration dates and do not take into account any potential patent term adjustment or extension that may be available.

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NBD1 Stabilizers

We co-own with Sanofi a patent family that discloses and covers each of SION-719 and SION-451 and methods of using SION-719 and SION-451 for the treatment of CF. The patent family is in the Patent Cooperation Treaty ("PCT") stage and is also pending in Argentina and Taiwan. The statutory expiration for this family is September 2043. We own a provisional patent application that discloses and claims the use of SION-719 in combination with other agents for the treatment of CF. This provisional patent application, if converted to a non-provisional application, will have a statutory expiration of March 2045.

Complementary Modulators

We exclusively license from Sanofi one patent family that discloses and covers SION-109 and methods of using SION-109 for the treatment of CF. The patent family has entered national phase and is pending in the U.S., the European Patent Office, African Regional Industrial Property Organization, African Intellectual Property Organization, Algeria, Australia, Bahrain, Brazil, Canada, Chile, China, Colombia, Eurasian Patent Office, Ecuador, Egypt, Guatemala, Hong Kong, Honduras, Indonesia, Israel, India, Jordan, Japan, Kuwait, Mexico, Malaysia, New Zealand, Nigeria, Oman, Panama, Peru, Philippines, Saudi Arabia, Singapore, South Africa, South Korea, Sri Lanka, Thailand, the United Arab Emirates and Vietnam. The statutory expiration for this family is November 2040.

We exclusively license from AbbVie one patent family that discloses and covers galicaftor and methods of using galicaftor for the treatment of CF. The patent family includes granted patents in the U.S., Albania, Argentina, Austria, Australia, Belgium, Bulgaria, Brazil, Canada, Chile, China, Colombia, Costa Rica, Croatia, Czech Republic, Cyprus, Denmark, Dominican Republic, Estonia, Finland, France, Germany, Greece, Hong Kong, Hungary, Iceland, India, Indonesia, Ireland, Israel, Italy, Japan, Latvia, Liechtenstein, Lithuania, Luxembourg, Malta, Malaysia, Mexico, Monaco, Netherlands, Norway, Panama, Peru, Poland, Portugal, Republic of North Macedonia, Romania, Russia, San Marino, Serbia, Singapore, Slovakia, Slovenia, Spain, South Africa, South Korea, Sweden, Switzerland, Turkey, Taiwan, Ukraine, the United Kingdom and Uruguay, and has a statutory expiration date of October 2035. We exclusively license from AbbVie one patent family that discloses and covers SION-2851 and methods of using SION-2851 for the treatment of CF. The patent family includes granted patents in the U.S., Australia, Brazil, Canada, Chile, China, Colombia, Costa Rica, Dominican Republic, France, Germany, India, Indonesia, Israel, Italy, Japan, Malaysia, Mexico, Panama, Peru, Pakistan, Russia, Singapore, Spain, South Africa, Taiwan, Ukraine, the United Kingdom, Uruguay and Vietnam, and has a statutory expiration date of July 2036.

We exclusively license from AbbVie one patent family that discloses and covers SION-3067 and methods of using SION-3067 for the treatment of CF. The patent family includes granted patents in the U.S., Albania, Austria, Australia, Belgium, Bulgaria, Chile, China, Colombia, Costa Rica, Croatia, Czech Republic, Cyprus, Denmark, Dominican Republic, Estonia, Finland, France, Germany, Greece, Hong Kong, Hungary, Iceland, India, Ireland, Israel, Italy, Japan, Latvia, Liechtenstein, Lithuania, Luxembourg, Macao, Malta, Malaysia, Mexico, Monaco, Netherlands, Norway, Panama, Peru, Philippines, Portugal, Poland, Republic of North Macedonia, Romania, Russia, San Marino, Serbia, Singapore, Slovakia, Slovenia, Spain, South Africa, South Korea, Sweden, Switzerland, Turkey and the United Kingdom, a pending application in Canada, and has a statutory expiration date of May 2037. Additionally, the patent family includes granted patents in Argentina and Taiwan, each of which has a statutory expiration date of June 2037.

We own two patent applications that each disclose and contain claims that recite the use of our NBD1 stabilizer product candidates in combination with SION-109, galicaftor, SION-3067, Trikafta and/or other modulators for the treatment of CF. The first is a PCT international application and has a statutory expiration of October 2043, and the second is a provisional patent application, which, if converted to a non-provisional application, will have a statutory expiration of March 2045.

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Licensing and Collaboration Agreements

Sanofi License Agreement

On December 20, 2019, we entered into a license agreement, as amended by Amendment No. 1 dated May 14, 2020, Amendment No. 2 on June 8, 2020, Amendment No. 3 on December 14, 2021, Amendment No. 4 on January 28, 2022, Amendment No. 5 on February 21, 2023 and Amendment No. 6 on October 28, 2024 (as amended, the "Sanofi License Agreement"), with Sanofi, pursuant to which we have been granted an exclusive, worldwide license to develop, commercialize, manufacture, use, hold, keep, register or dispose of certain compounds, patents and proprietary information and inventions, in each case for therapeutic, prophylactic, prognostic and diagnostic purposes in or for humans, subject to retained rights. The licensed and derived rights are directed, among other things, to CFTR modulator therapies which are being utilized in SION-719, SION-109 and SION-451.

Pursuant to the terms of the Sanofi License Agreement, we must use commercially reasonable efforts to develop, pursue regulatory approval for and commercialize a licensed product. Sanofi and its affiliates retain the right to practice under the licensed patents and use the licensed know-how solely to conduct non-clinical research for all therapeutic, prophylactic, prognostic and diagnostic uses in or for humans, other than for CF; provided, however, that Sanofi will not exercise these retained rights until after December 20, 2024 and will not file any patents that claim a licensed compound.

As initial consideration for the license, we paid a non-refundable, upfront payment of $1.5 million, as well as a reimbursement of $0.3 million for Sanofi's research and development expenses. In addition, we are required to pay Sanofi a total of up to $40 million upon achievement of certain late-stage developmental and commercial milestones. None of such milestones have been achieved to date. We are also required to pay royalties to Sanofi in the low single-digit percentage range based on net sales of licensed products, subject to customary reductions and offsets. Such royalty payments shall be reduced for products covered by derived patents. The royalty term will terminate on a product-by-product and country-by-country basis upon the later of (i) the expiration of the last-to-expire valid claim within the relevant licensed patent rights, (ii) the expiration of regulatory exclusivity in such country for such licensed product and (iii) the tenth anniversary of the first commercial sale of a licensed product in such country.

We are entitled to sublicense the rights granted to us under the Sanofi License Agreement under certain circumstances, provided that any such sublicense must be consistent with the terms of the Sanofi License Agreement. If we receive sublicense income from any such sublicense, we are required to pay Sanofi a low double digit percentage of such sublicense income.

We have also granted Sanofi an exclusive option to purchase, at a defined price, any priority review voucher ("PRV") granted to us as a result of the development of the licensed compounds or products. In the event that Sanofi does not exercise its option with respect to any PRV, we may (x) use the PRV, in which case we must pay Sanofi a high seven-digit amount or (y) sell the PRV to a third party, in which case we must share a sub-teen double-digit percentage of the sale consideration with Sanofi.

We have the right, but not the obligation, to prepare, file, prosecute and maintain the licensed patents and product trademarks at our own cost. We have the first right to enforce and defend any licensed patents, with Sanofi having back-up enforcement and defense rights. We also have the sole right to enforce and defend any product trademarks at our sole cost and expense.

We have the right to terminate the Sanofi License Agreement for convenience, subject to a 90-day notice period. Sanofi has customary termination rights under the Sanofi License Agreement, including for our material breach, payment default, bankruptcy or challenge of the validity of any patent

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right, subject to specified notice and cure periods. Unless earlier terminated, the Sanofi License Agreement will expire in each country upon the expiration of the last-to-expire royalty term in such country and, with respect to the Sanofi License Agreement in its entirety, upon the expiration of the royalty term for the last licensed product for which there has been a first commercial sale. Upon expiration of the Sanofi License Agreement, the license granted to us will become non-exclusive, royalty-free, fully paid-up and perpetual.

CFF Payment Agreement

On December 20, 2019, we entered into a payment agreement (the "CFF Payment Agreement") with CFF, pursuant to which we agreed to provide CFF with compensation in exchange for the grant of, or forbearance from exercising, certain of CFF's rights existing under the Research, Development and Commercialization Agreement, dated October 1, 2011, by and between CFF (through an assignment by Cystic Fibrosis Foundation Therapeutics, Inc.) and Genzyme Corporation, an affiliate of Sanofi (the "CFFT-Genzyme Agreement"). As described above, we have been granted a license to certain compounds, patents and know-how pursuant to the Sanofi License Agreement, some of which were generated as a result of a research plan under the CFFT-Genzyme Agreement. Under the CFF Payment Agreement, we are obligated to compensate CFF in connection with our development and commercialization of licensed products under the Sanofi License Agreement. Concurrent with the execution of the CFF Payment Agreement, Sanofi and CFF terminated the CFFT-Genzyme Agreement.

As initial consideration for CFF's grant of, and forbearance from exercising, its rights under the CFFT-Genzyme Agreement, we issued CFF 300,300 shares of our Series Seed preferred stock. In addition, we agreed to pay CFF a sub-teen double-digit percentage of any amounts paid by us to Sanofi under the Sanofi License Agreement, other than milestone, royalty or reimbursement payments. As of December 31, 2023, we have paid CFF a total of approximately $0.2 million in accordance with the terms of the CFF Payment Agreement. In addition, we are required to pay CFF a total of up to $40 million upon achievement of certain late-stage developmental and commercial milestones. None of such milestones have been achieved to date. We are also required to pay revenue-shares of royalty payments to CFF in the low single-digit percentage range based on net sales of licensed products, subject to customary reductions and offsets. Such milestone and royalty payments shall be reduced for products covered by derived patents. The royalty term will terminate on a product-by-product and country-by-country basis upon the later of (i) the expiration of the last-to-expire valid claim within the relevant patent rights that claims such product or its exploitation in such country, (ii) the tenth anniversary of the first commercial sale of a product in such country and (iii) expiration of regulatory exclusivity of a product in such country. Further, a side letter was executed between us and Sanofi, which clarifies the relationship between us, Sanofi and CFF, under which we are obligated to pay Sanofi 20% of the milestones it would have been obligated to pay CFF, net of the milestone amounts it is obligated to pay under the Sanofi License Agreement.

If at any time prior to the first commercial sale of a product developed as a result of the CFF Payment Agreement, we cease to use commercially reasonably efforts to develop, and obtain and maintain regulatory approvals for, at least one of our products for therapeutic, prophylactic, prognostic or diagnostic uses in or for humans in specified major markets for a continuous period of 365 days, CFF has the option to exercise rights to an exclusive, irrevocable, worldwide interruption license under our patents, the licensed patents and the licensed know-how, to develop, manufacture, use, sell, offer to sell and import any of our products containing a licensed compound or any compound covered by a licensed patent or incorporating licensed know-how.

Either party may terminate the CFF Payment Agreement for a material breach by the other party, subject to a specified notice and cure period. Unless earlier terminated, the CFF Payment Agreement

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will expire (a) with respect to each product in each country, upon the expiration of the last-to-expire royalty term in such country, and (b) with respect to the CFF Payment Agreement in its entirety, upon the later of (i) the expiration of the royalty term for the last licensed product for which there has been a first commercial sale or (ii) the expiration or termination of our obligation to pay consideration to Sanofi under the Sanofi License Agreement.

AbbVie License Agreement

On July 11, 2024, we entered into a license agreement (the "AbbVie License Agreement") with AbbVie, pursuant to which we have been granted an exclusive worldwide, royalty-bearing, sublicensable license to certain patent and other intellectual property rights to research, develop, commercialize, make, manufacture, use, import and sell products for prophylactic or therapeutic use in humans for all indications, subject to certain limitations and retained rights. The licensed rights are directed, among other things, to three clinical-stage CFTR modulator therapies: galicaftor (ABBV-2222, now referred to as SION-2222), a TMD1-directed CFTR corrector, navocaftor (ABBV-3067, now referred to as SION-3067), a CFTR potentiator, and ABBV-2851 (now referred to as SION-2851), a TMD1-directed corrector. Under the AbbVie License Agreement, we have assumed all global development and commercialization responsibilities for such therapies. Pursuant to the terms of the AbbVie License Agreement, we must use commercially reasonable efforts to develop, pursue regulatory approval for and commercialize a licensed product. We are also required to achieve certain development milestones within specified time periods for products incorporating intellectual property covered by the AbbVie License Agreement.

The license granted to us under the AbbVie License Agreement is subject to certain preexisting rights held by AbbVie and Galapagos NV ("Galapagos"). In particular, certain of the licensed patents and other intellectual property rights were developed by or on behalf of Galapagos and are sublicensed to us subject to the terms of the second amended and restated collaboration agreement between Galapagos and AbbVie dated as of October 24, 2018 (the "Galapagos License Agreement"), as amended by a side letter between Galapagos and AbbVie dated as of July 11, 2024. We are also entitled to sublicense the rights granted to us under the AbbVie License Agreement under certain circumstances, provided that any such sublicense must be consistent with the terms of the AbbVie License Agreement and the Galapagos License Agreement.

As initial consideration for the license, we paid a non-refundable, upfront payment of $5 million and issued 1,414,445 shares of our common stock to AbbVie. In addition, we are required to pay AbbVie a total of up to $360 million upon achievement of certain development and commercial milestones, consisting of up to $70.0 million in late-stage development milestones and up to $290.0 million in commercial milestones. None of such milestones have been achieved to date. We are also required to pay royalties to AbbVie in the low to mid single-digit percentage range based on net sales of licensed products, subject to customary reductions and offsets, with the percentage range depending in part on the compounds used. The royalty term will terminate on a product-by-product and country-by-country basis upon the later of (i) the expiration of the last-to-expire valid claim within the relevant patent rights that covers the manufacture, use, sale or other exploitation of a product in such country, (ii) the tenth anniversary of the first commercial sale of a product in such country and (iii) expiration of regulatory exclusivity of a product in such country. In addition, we are required to pay AbbVie up to $130 million in commercial and sales-based milestone payments, mid to high single-digit royalties on the licensed products or other payments due to Galapagos pursuant to the Galapagos License Agreement, to the extent such payments are triggered by our use of the licensed rights under the AbbVie License Agreement. To date, no payments have been triggered.

We have also granted AbbVie a right of first negotiation (the "ROFN") if we decide to pursue a license or sublicense to commercialize a licensed product (a "Commercial License Transaction") prior to initiating Phase 3 clinical trials. If AbbVie timely exercises the ROFN, then it will have an exclusive

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period to negotiate in good faith the terms of a Commercial License Transaction. In the event (x) AbbVie does not timely exercise the ROFN or notifies us that it does not intend to pursue a Commercial License Transaction (including after timely exercising the ROFN) or (y) the parties fail to reach agreement or enter into a definitive agreement for the Commercial License Transaction within the exclusive negotiation period, then the ROFN regarding all licensed products will terminate. We have also granted AbbVie a right of participation, whereby AbbVie may participate in a private placement concurrent with this offering in an amount in the low to mid single-digit percentage range of the number of shares offered in this offering. Such right has been waived in connection with this offering.

We are responsible for the prosecution and maintenance of the licensed patents at our own cost. We have the first right to enforce and defend any licensed patent at our own cost, with AbbVie having back-up enforcement and defense rights.

We have the right to terminate the AbbVie License Agreement for convenience, subject to a prescribed notice period. AbbVie has customary termination rights under the AbbVie License Agreement, including for our material breach, payment default, bankruptcy, challenge of the validity of any patent right or shelving of a product for a specified time period, subject to specified notice and cure periods. Unless earlier terminated, the AbbVie License Agreement will expire upon the expiration of the last-to-expire royalty term. Upon expiration of the AbbVie License Agreement, the license granted to us will become non-exclusive, royalty-free, fully paid-up and perpetual.

In the event the AbbVie License Agreement is terminated, we and AbbVie shall agree upon a transition plan to revert the licensed compounds or licensed products containing such licensed compounds to AbbVie. Within a prescribed time period of such termination, we are obligated to (i) assign and transfer all of our rights and interests in the documentation and data related to the reverting compounds or products to AbbVie, (ii) grant to AbbVie a non-exclusive, royalty-free license right of reference for AbbVie to develop or commercialize any of the reverting compounds or products, (iii) grant to AbbVie an exclusive, royalty-bearing worldwide license to exploit any of the reverting compounds or products and (iv) transfer to AbbVie control of all clinical studies being conducted for any of the reverting compounds or products.

Government Regulation

The FDA and comparable regulatory authorities in federal, state and local jurisdictions and in other foreign countries impose extensive requirements upon companies involved in the clinical development, manufacture, marketing and distribution of drugs, such as those we are developing. These agencies and other federal, state and local entities extensively regulate, among other things, the research and development, testing, manufacture, quality control, safety, effectiveness, labeling, packaging, storage, record keeping, approval, advertising and promotion, distribution, post-approval monitoring and reporting, sampling and export and import of drugs. The process of obtaining regulatory approvals in the U.S. and in foreign countries and jurisdictions, along with subsequent compliance with applicable federal, state, local and foreign statutes and regulations, requires the expenditure of substantial time and financial resources. Failure to comply with the applicable requirements at any time during the product development process, approval process or after approval, may subject an applicant and/or sponsor to a variety of sanctions. For example, failure to comply with the applicable U.S. requirements may result in administrative or judicial sanctions including refusal by FDA to approve pending applications, withdrawal of an approval, imposition of a clinical hold, issuance of warning letters and untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal investigations and penalties brought by the FDA and the Department of Justice or other governmental entities.

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Review and Approval of Drugs in the United States

In the U.S., the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act ("FDCA"), and its implementing regulations. Drugs are also subject to other federal, state and local statutes and regulations.

The process required by the FDA before a drug may be marketed in the U.S. generally involves the following:

- completion of extensive nonclinical, or preclinical, laboratory tests, animal studies and formulation studies in compliance with the FDA's Good Laboratory Practice ("GLP") regulations;
- submission to the FDA of an IND, which must become effective before human clinical trials may begin and must be updated annually and when certain changes are made;
- approval by an institutional review board ("IRB") or independent ethics committee ("IEC") at each clinical site before each trial may be initiated at that site;
- performance of adequate and well-controlled human clinical trials in accordance with Good Clinical Practices ("GCP") requirements and other clinical trial-related regulations to establish the safety and efficacy of the investigational drug product for each proposed indication;
- preparation and submission to the FDA of an NDA after completion of all pivotal trials, together with the payment of application user fees, as applicable;
- a determination by the FDA within 60 days of its receipt of an NDA to accept the marketing application for review;
- satisfactory completion of an FDA advisory committee review, if applicable;
- satisfactory completion of one or more FDA inspections of the manufacturing facility or facilities at which the product is produced to assess compliance with cGMP requirements and to assure that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity;
- satisfactory completion of FDA audits of clinical trial sites to assure compliance with GCPs and the integrity of the clinical data; and
- FDA review and approval of the NDA.

Preclinical Studies

Before testing any drug product candidate, including our product candidates, in humans, the product candidate must undergo rigorous preclinical testing. Preclinical studies include laboratory evaluations of the product's chemistry, purity, toxicity, formulation, and stability as well as *in vitro* and animal studies to assess potential safety and efficacy and in some cases to establish the rationale for therapeutic use. The conduct of preclinical studies is subject to federal regulations and requirements, including GLP regulations for safety and toxicology studies.

The IND and IRB Process

Prior to beginning the first clinical trial with a product candidate in the U.S., we must submit an IND to the FDA. An IND sponsor must submit a protocol for each clinical trial, the results of the preclinical tests, manufacturing information, analytical data and any available clinical data or literature and plans for clinical studies, among other things, to the FDA as part of an IND. An IND is a request for authorization from the FDA to grant an exemption that allows an unapproved drug to be shipped in

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interstate commerce for use and administration in an investigational clinical trial for humans. The IND must become effective before human clinical trials may begin in the U.S. Some preclinical testing, such as animal tests of reproductive adverse events and carcinogenicity, may continue even after the IND is submitted. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to one or more proposed clinical trials and places the clinical trial on a clinical hold. A clinical hold is an order issued by the FDA to the sponsor to delay a proposed clinical investigation or to suspend an ongoing clinical trial. Clinical holds may be imposed by the FDA when there is concern for patient safety, and may be a result of new data, findings or developments in clinical, nonclinical, and/or chemistry, manufacturing and controls or where there is non-compliance with regulatory requirements. A partial clinical hold is a delay or suspension of only part of the clinical work requested under the IND. Following issuance of a clinical hold or partial clinical hold, an investigation (or full investigation in the case of a partial clinical hold) may only resume after the FDA has notified the sponsor that the investigation may proceed. As a result, submission of an IND may not result in the FDA allowing clinical trials to initiate.

A separate submission to an existing IND must also be made for each successive clinical trial to be conducted, and the FDA must grant permission, either explicitly or implicitly by not objecting, before each clinical trial can begin.

A sponsor may choose, but is not required, to conduct a foreign clinical trial under an IND. When a foreign clinical trial is conducted under an IND, all FDA IND requirements must be met unless waived. When the foreign clinical trial is not conducted under an IND, such as our ongoing Phase 1 clinical trials of SION-719 and SION-451 being conducted in Australia, the sponsor must ensure that the study is conducted in accordance with GCP, including review and approval by an IEC and informed consent from subjects. FDA must be able to validate the data from the study through an on-site inspection if necessary.

In addition to the foregoing IND requirements, an IRB representing each institution participating in the clinical trial must review and approve the plan for any clinical trial before it commences at that institution, and the IRB must conduct continuing review of the study. The IRB is charged with protecting the welfare and rights of trial participants and considers whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB must review and approve, among other things, the study protocol and informed consent information to be provided to study subjects and must monitor the clinical trial until completion. An IRB can suspend or terminate approval of a clinical trial at its institution, or an institution it represents, if the clinical trial is not being conducted in accordance with the IRB's requirements or if the product candidate has been associated with unexpected serious harm to patients.

Clinical Trials in Support of an NDA

Clinical trials involve the administration of the investigational new drug to human subjects under the supervision of qualified investigator in accordance with GCP requirements, which include the requirement that all research subjects, or their legal representative, provide their informed consent in writing for their participation in any clinical trial. Clinical trials are conducted under written study protocols detailing, among other things, the exclusion and inclusion criteria, the objectives of the trial, dosing procedures, subject selection, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. As part of an IND, a protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA.

Regulatory authorities, the IRB or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the subjects are being exposed to an unacceptable health risk or that the trial is unlikely to meet its stated objectives. Some studies also include oversight by an independent

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group of qualified experts organized by the clinical study sponsor, known as a data and safety monitoring board ("DSMB"), which provides authorization for whether or not a study may move forward at designated check points based on access to certain data from the study and may halt the clinical trial if it determines that there is an unacceptable safety or health risk for subjects or other grounds, such as no demonstration of efficacy.

Information about certain clinical trials must be submitted within specific timeframes to the National Institutes of Health ("NIH") for public dissemination on their *www.clinicaltrials.gov* website. Information related to the investigational product, patient population, phase of investigation, study sites and investigators and other aspects of the clinical trial is made public as part of the registration of the clinical trial. Although sponsors are obligated to disclose the results of their clinical trials after completion, disclosure of the results can be delayed in some cases for some time. Failure to timely register a covered clinical study or to submit study results as provided for in the law can give rise to civil monetary penalties and also prevent the non-compliant party from receiving future grant funds from the federal government.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

- Phase 1: The investigational drug is initially introduced into a limited population of healthy human subjects or, in certain indications such as cancer, patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion, side effects, and, if possible, to gain an early indication of its effectiveness or determine optimal dosage.
- Phase 2: The investigational drug is administered to a limited patient population with a specified disease or condition to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage. Multiple Phase 2 clinical trials may be conducted to obtain information prior to beginning Phase 3 clinical trials.
- Phase 3: The investigational drug is administered to an expanded patient population, generally at geographically dispersed clinical trial sites, in well-controlled clinical trials to generate enough data to statistically evaluate the efficacy and safety of the product for approval, to establish the overall risk/benefit profile of the product, and to provide adequate information for product approval and labeling of the product. Generally, two adequate and well-controlled Phase 3 clinical trials are required by the FDA for approval of an NDA.

Post-approval trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication and are commonly intended to generate additional safety data regarding use of the product in a clinical setting. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval on an NDA.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if serious adverse events occur. In addition, within 15 calendar days after the sponsor determines that the information qualifies for reporting, written IND safety reports must be submitted to the FDA and investigators for serious and unexpected suspected adverse events, findings from other studies or animal or *in vitro* testing that suggest a significant risk for human subjects and any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information. The FDA will typically inspect one or more clinical sites to assure compliance with GCP and the integrity of the clinical data submitted.

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During the development of a new drug, sponsors are given opportunities to meet with the FDA at certain points. These points may be prior to submission of an IND, at the end of Phase 2, and before an NDA is submitted. Meetings at other times may be requested. These meetings can provide an opportunity for the sponsor to share information about the data gathered to date and for the FDA to provide advice.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the product candidate and finalize a process for manufacturing the drug product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and manufacturers must develop, among other things, methods for testing the identity, strength, quality and purity of the final drug product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

FDA Review of an NDA Submission and FDA Approval

Assuming successful completion of the required clinical testing, the results of the preclinical and clinical studies, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA requesting approval to market the product for one or more indications. An NDA is a request for approval to market a new drug for one or more specified indications and must contain proof of the drug's safety and efficacy for the requested indications. FDA must approve an NDA before a drug may be marketed in the U.S. For companies, the marketing application is required to include both negative and ambiguous results of preclinical studies and clinical trials, as well as positive findings. Data may come from company-sponsored clinical trials intended to test the safety and effectiveness of a use of a product, or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and effectiveness of the investigational drug product for the proposed indication to the satisfaction of the FDA. In most cases, the submission of an NDA is subject to a significant application user fee. Fee exceptions or fee waivers may be obtained under certain limited circumstances.

The FDA conducts a preliminary review of all NDAs within the first 60 days of its receipt, before accepting them for filing, to determine whether they are sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA reviews an NDA to determine, among other things, whether the drug is safe and effective and whether the facility in which it is manufactured, processed, packaged or held meets standards designed to assure the product's continued safety, quality and purity.

During its review of an NDA, the FDA typically will inspect the facility or facilities where the product is or will be manufactured. These pre-approval inspections may cover all facilities associated with an NDA, including drug component manufacturing (such as APIs), finished drug product manufacturing and control testing laboratories. The FDA will not approve an NDA unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications.

In addition, as a condition of approval, or post-approval if it becomes aware of a serious risk associated with the use of the product, the FDA may require the submission of a Risk Evaluation and

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Mitigation Strategy ("REMS"), if it determines that a REMS is necessary to ensure that the benefits of the drug outweigh its risks and to assure the safe use of the drug. If the FDA concludes a REMS is needed, the sponsor of the NDA must submit a proposed REMS. The FDA will not approve the NDA without a REMS, if required. A REMS may include one or more elements, including medication guides, physician communication plans, patient package insert and/or elements to assure safe use, such as special training or certification for prescribing or dispensing, restricted distribution methods, special monitoring, patient registries or other risk minimization tools.

Under the Prescription Drug User Fee Act ("PDUFA") guidelines that are currently in effect, the FDA has a goal of ten months from the date of "filing" of a standard NDA, for a new molecular entity, to review and act on the submission, and six months from the filing date of a new molecular entity NDA with priority review. Accordingly, this review process typically takes 12 months and eight months, respectively, from the date the NDA is submitted to the FDA. The FDA does not always meet its PDUFA goal dates for standard or priority NDAs, and the review process is often extended by FDA requests for additional information or clarification.

In addition, under the Pediatric Research Equity Act of 2003, as amended ("PREA"), certain NDAs or supplements to an NDA must contain data that are adequate to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. A sponsor who is planning to submit a marketing application for a drug that includes a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration must submit an initial Pediatric Study Plan ("PSP"), within 60 days of an end-of-Phase 2 meeting or, if there is no such meeting, as early as practicable before initiation of the Phase 3 or Phase 2/3 study. The initial PSP must include an outline of the pediatric study or studies that the sponsor plans to conduct, including study objectives and design, age groups, relevant endpoints and statistical approach, or a justification for not including such detailed information, and any request for a deferral of pediatric assessments or a full or partial waiver of the requirement to provide data from pediatric studies along with supporting information. The FDA and the sponsor must reach an agreement on the PSP. A sponsor can submit amendments to an agreed-upon initial PSP at any time if changes to the pediatric plan need to be considered based on data collected from preclinical studies, early phase clinical trials and/or other clinical development programs.

The FDA may refer an application for a novel drug or a drug that presents difficult questions of safety or efficacy to an advisory committee. An advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

After evaluating the NDA and all related information, including the advisory committee recommendation, if any, and inspection reports regarding the manufacturing facilities and clinical trial sites, the FDA may issue an approval letter, or, in some cases, a Complete Response Letter. A Complete Response Letter indicates that the review cycle of the application is complete, and the application will not be approved in its present form. A Complete Response Letter generally outlines the deficiencies in the submission and contains a statement of specific conditions that must be met in order to secure final approval of the NDA and it may require additional clinical or preclinical testing in order for FDA to reconsider the application. If a Complete Response Letter is issued, the applicant may resubmit the NDA, addressing all of the deficiencies identified in the letter, withdraw the application, or request a hearing. Even with submission of this additional information, the FDA ultimately may decide

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that the application does not satisfy the regulatory criteria for approval. If and when those conditions have been met to the FDA's satisfaction, the FDA will typically issue an approval letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications.

Even if the FDA approves a product, depending on the specific risk(s) to be addressed, it may limit the approved indications for use of the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Orphan Drug Designation and Exclusivity

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug product intended to treat a rare disease or condition, which is generally a disease or condition that affects either (i) fewer than 200,000 individuals in the U.S. or (ii) more than 200,000 individuals in the U.S. and for which there is no reasonable expectation that the cost of developing and making the product available in the U.S. for this type of disease or condition will be recovered from sales of the product. A company must request orphan drug designation before submitting an NDA. If the request is granted, the FDA will disclose the identity of the therapeutic agent and its potential use. Orphan drug designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation or for a select indication or use within the rare disease or condition for which it was designated, the product is entitled to orphan product exclusivity. Orphan product exclusivity means that the FDA may not approve any other applications to market the same product for the same indication for seven years, except in certain limited circumstances, including if a subsequent product with the same active ingredient for the same indication is shown to be clinically superior to the approved product on the basis of greater efficacy or safety, or providing a major contribution to patient care, or if the company with orphan drug exclusivity is not able to meet market demand. Further, the FDA may approve more than one product for the same orphan indication or disease as long as the products contain different active ingredients. Moreover, competitors may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan drug has exclusivity. Orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and user-fee waivers.

A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. In addition, orphan drug exclusive marketing rights in the U.S. may be lost if the FDA later determines that the request for designation was materially defective or, as noted above, if a second applicant demonstrates that its product is clinically superior to the approved product with orphan exclusivity or the manufacturer of the approved product is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

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The FDA has historically taken the position that the scope of orphan exclusivity aligns with the approved indication or use of a product, rather than the disease or condition for which the product received orphan designation. However, in *Catalyst Pharms., Inc. v. Becerra*, 14 F.4th 1299 (11th Cir. 2021), the court disagreed with this position, holding that orphan-drug exclusivity blocked the FDA's approval of the same drug for all uses or indications within the same orphan-designated disease. On January 24, 2023, the FDA published a notice in the Federal Register to clarify that the FDA intends to continue to apply its longstanding interpretation of the regulations to all matters outside of the scope of the *Catalyst* order and will continue tying the scope of orphan drug exclusivity to the uses or indications for which a drug is approved. It is unclear how future litigation, legislation, agency decisions and administrative actions will impact the scope of orphan drug exclusivity.

Expedited Development and Review Programs for Drugs

The FDA maintains several programs intended to facilitate and expedite development and review of new drugs that were intended to address unmet medical needs in the treatment of serious or life-threatening diseases or conditions. Some of these programs are referred to as Fast Track designation, Breakthrough Therapy designation, Priority Review and Accelerated Approval, and the purpose of these programs is to either expedite the development or review of important new drugs to get them to patients earlier than under standard FDA development and review procedures.

The FDA has a Fast Track designation program that is intended to expedite or facilitate the process for reviewing new drugs that meet certain criteria. Specifically, new drugs are eligible for Fast Track designation if they are intended, whether alone or in combination with one or more other products, to treat a serious or life-threatening condition and preclinical or clinical data demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to both the product and the specific indication for which it is being studied. The sponsor can request that the FDA grant the product Fast Track designation any time before receiving NDA approval but ideally no later than the pre-NDA meeting. Fast Track designation provides increased opportunities for sponsor interactions with the FDA review team to expedite development and review of the product. The FDA may also review sections of the NDA for a Fast Track designated product on a rolling basis before the completed application is submitted, if the sponsor provides a schedule for the submission of the sections of the application, the FDA agrees to accept sections of the application and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the application. The FDA's goal for reviewing a Fast Track application does not begin until the last section of the NDA is submitted. Fast Track designation may be lost if the designation is no longer supported by data emerging in the clinical trial process.

Additionally, a drug may be eligible for designation as a Breakthrough Therapy if the product is intended, alone or in combination with one or more other products, to treat a serious or life-threatening condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over currently approved therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The benefits of Breakthrough Therapy designation include the same benefits as Fast Track designation, plus intensive guidance from the FDA to ensure an efficient drug development program. Breakthrough Therapy designation comes with all of the benefits of Fast Track designation, which means that the sponsor may file sections of the NDA for review on a rolling basis if certain conditions are satisfied, including an agreement with the FDA on the proposed schedule for submission of portions of the application and the payment of applicable user fees before the FDA may initiate a review. The FDA may take certain actions with respect to Breakthrough Therapies, including holding meetings with the sponsor throughout the development process; providing timely advice to the product sponsor regarding development and approval; involving more senior staff in the review process; assigning a cross-disciplinary project lead for the review team; or taking other steps to design the clinical trials in an efficient manner.

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A product may also be eligible for priority review if it treats a serious or life-threatening condition and, if approved, would provide a significant improvement in safety and effectiveness compared to available therapies. The FDA determines at the time that the marketing application is submitted, on a case-by-case basis, whether the proposed drug represents a significant improvement in treatment, prevention or diagnosis of disease when compared with other available therapies. Significant improvement may be demonstrated by evidence of increased effectiveness in the treatment of a condition, elimination or substantial reduction of a treatment-limiting product reaction, documented enhancement of patient compliance that may lead to improvement in serious outcomes and evidence of safety and effectiveness in a new subpopulation. A priority review designation is intended to direct overall attention and resources to the evaluation of such applications and to shorten the FDA's goal for taking action on a marketing application from ten months to six months for an NDA for a new molecular entity from the date of filing. If criteria are not met for priority review, the application for a new molecular entity is subject to the standard FDA review period of ten months after FDA accepts the application for filing. Priority review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

A product may also be eligible for accelerated approval if it treats a serious or life-threatening disease or condition, generally provides a meaningful advantage over available therapies and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality ("IMM") that is reasonably likely to predict an effect on IMM or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. Accelerated approval by the FDA is generally contingent on a sponsor's agreement to conduct, in an adequate and diligent manner, additional post-approval confirmatory studies to verify and describe the product's clinical benefit. These confirmatory trials must be completed with due diligence, and, under the Food and Drug Omnibus Reform Act of 2022 ("FDORA"), the FDA is now permitted to require, as appropriate, that such trials be underway prior to approval or within a specific time period after the date of approval for a product granted accelerated approval. Under FDORA, the FDA has increased authority for expedited procedures to withdraw the product from the market (and withdraw its approval). As a result, a product candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, could result in the FDA's withdrawal of the approval and require the withdrawal of the product from the market on an expedited basis. All promotional materials for product candidates approved under accelerated regulations are subject to prior review by the FDA.

Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened. Fast Track designation, Breakthrough Therapy designation, priority review and accelerated approval do not change the standards for approval and may not ultimately expedite the development or approval process.

Hatch-Waxman Amendments

Section 505 of the FDCA describes three types of marketing applications that may be submitted to the FDA to request marketing authorization for a new drug. A Section 505(b)(1) NDA is an application that contains full reports of investigations of safety and efficacy. A 505(b)(2) NDA is an application that contains full reports of investigations of safety and efficacy but where at least some of the information required for approval comes from investigations that were not conducted by or for the applicant and for which the applicant has not obtained a right of reference or use from the person by or for whom the investigations were conducted. This regulatory pathway enables the applicant to rely, in part, on the FDA's prior findings of safety and efficacy for an existing product, or published literature, in

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support of its application. Section 505(j) establishes an abbreviated approval process for a generic version of approved drug products through the submission of an Abbreviated New Drug Application ("ANDA"). An ANDA provides for marketing of a generic drug product that has the same active ingredients, dosage form, strength, route of administration, labeling, performance characteristics and intended use, among other things, to a previously approved product, known as a reference listed drug ("RLD"). ANDAs are termed "abbreviated" because they are generally not required to include preclinical (animal) and clinical (human) data to establish safety and efficacy. Instead, generic applicants must scientifically demonstrate that their product is bioequivalent to, or performs in the same manner as, the innovator drug through *in vitro*, *in vivo* or other testing.

U.S. Non-Patent Exclusivity

Under the Hatch-Waxman Amendments, the FDA may not approve (or in some cases accept) an ANDA or 505(b)(2) application until any applicable period of non-patent exclusivity for the RLD has expired. Market exclusivity provisions under the FDCA can delay the submission or the approval of certain follow-on applications. The FDCA provides a five-year period of non-patent data exclusivity within the U.S. to the first applicant to gain approval of an NDA for a new chemical entity ("NCE"). A drug is an NCE if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the physiological or pharmacological action of the drug substance. In cases where such NCE exclusivity has been granted, the FDA may not accept for review an ANDA, for a generic version of the drug or a 505(b)(2) NDA for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval, until the expiration of five years unless the submission is accompanied by a paragraph IV certification, which states the proposed generic drug will not infringe one or more of the already approved product's listed patents or that such patents are invalid or unenforceable, in which case the applicant may submit its application four years following the original product approval.

The FDCA also provides three years of market exclusivity for non-NCE NDAs, 505(b)(2) NDA or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example, new indications, dosages or strengths of an existing drug. This three-year exclusivity period covers only the conditions of use associated with the new clinical investigations and often protects changes to a previously approved drug product, such as a new dosage form, route of administration, combination or indication, but it generally would not protect the original, unmodified product from generic competition. In other words, it does not prohibit the FDA from approving follow-on applications that do not reference the protected clinical data. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Pediatric exclusivity is another type of regulatory market exclusivity in the U.S. Pediatric exclusivity, if granted, adds six months to existing regulatory exclusivity periods for all formulations, dosage forms, indications of the active moiety, and listed patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric trial in accordance with an FDA-issued "Written Request" for such a trial, provided that at the time pediatric exclusivity is granted there is not less than nine months of term remaining. The issuance of a Written Request does not require the sponsor to undertake the described clinical trials.

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Hatch-Waxman Patent Certification and the 30-Month Stay

In seeking approval of an NDA or a supplement thereto, NDA sponsors are required to list with the FDA each patent with claims that cover the applicant's product or an approved method of using the product. Upon approval, each of the patents listed by the NDA sponsor is published in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Upon submission of an ANDA or 505(b)(2) NDA, an applicant is required to certify to the FDA concerning any patents listed for the RLD in the Orange Book that:

- no patent information on the drug product that is the subject of the application has been submitted to the FDA;
- such patent has expired;
- the date on which such patent expires; or
- such patent is invalid, unenforceable or will not be infringed upon by the manufacture, use or sale of the drug product for which the application is submitted.

Generally, the ANDA or 505(b)(2) NDA cannot be approved until all listed patents have expired, except where the ANDA or 505(b)(2) NDA applicant challenges a listed patent through the last type of certification, also known as a paragraph IV certification. If the applicant does not challenge the listed patents or indicates that it is not seeking approval of a patented method of use, the ANDA or 505(b)(2) NDA application will not be approved until all of the listed patents claiming the referenced product have expired. If the ANDA or 505(b)(2) NDA applicant has provided a paragraph IV certification the applicant must send notice of the paragraph IV certification to the NDA and patent holders once the application has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the paragraph IV certification. If the paragraph IV certification is challenged by an NDA holder or the patent owner(s) asserts a patent challenge to the paragraph IV certification, the FDA may not approve that application until the earlier of 30 months from the receipt of the notice of the paragraph IV certification, the expiration of the patent, when the infringement case concerning each such patent was favorably decided in the applicant's favor or settled, or such shorter or longer period as may be ordered by a court. This prohibition is generally referred to as the 30-month stay. In instances where an ANDA or 505(b)(2) NDA applicant files a paragraph IV certification, the NDA holder or patent owner(s) regularly take action to trigger the 30-month stay, recognizing that the related patent litigation may take many months or years to resolve. Thus, approval of an ANDA or 505(b)(2) NDA could be delayed for a significant period of time depending on the patent certification the applicant makes and the reference drug sponsor's decision to initiate patent litigation. If the drug has NCE exclusivity and the ANDA is submitted four years after approval, the 30-month stay is extended so that it expires seven and a half years after approval of the innovator drug, unless the patent expires or there is a decision in the infringement case that is favorable to the ANDA applicant before then.

Patent Term Restoration and Extension

A patent claiming a new drug product may be eligible for a limited patent term extension under the Hatch-Waxman Amendments, which permits a patent term restoration of up to five years for patent term lost during product development and the FDA regulatory review. The restoration period granted is typically one-half the time between the effective date of an IND and the submission date of an NDA, plus the time between the submission date of an NDA and the ultimate approval date, provided the sponsor acted with diligence. Patent term restoration cannot be used to extend the remaining term of a patent past a total of 14 years from the product's approval date. Only one patent applicable to an approved drug product is eligible for the extension, and the application for the extension must be submitted prior to the expiration of the patent in question and within 60 days of drug approval. A patent

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that covers multiple drugs for which approval is sought can only be extended in connection with one of the approvals. The U.S. Patent and Trademark Office reviews and approves the application for any patent term extension or restoration in consultation with the FDA.

Post-Approval Requirements

Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims are subject to prior FDA review and approval. There are also annual prescription drug product program fee requirements for marketed products.

The FDA may impose a number of post-approval requirements as a condition of approval of an NDA. For example, the FDA may require post-marketing testing, including Phase 4 clinical trials, and surveillance to further assess and monitor the product's safety and effectiveness after commercialization.

FDA regulations require that products be manufactured in specific facilities and in accordance with cGMP regulations which require, among other things, quality control and quality assurance, the maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP.

In addition, drug manufacturers and other entities involved in the manufacture and distribution of approved drugs and those supplying products, ingredients and components of them are required to register their establishments with the FDA and state agencies and are subject to periodic unannounced inspections by the FDA and these state agencies for compliance with cGMP requirements. Manufacturers and other parties involved in the drug supply chain for prescription drug products must also comply with product tracking and tracing requirements and for notifying FDA of counterfeit, diverted, stolen and intentionally adulterated products or products that are otherwise unfit for distribution in the U.S. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP requirements and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance.

Once an approval of a drug is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in mandatory revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Any of these limitations on approval or marketing could restrict the commercial promotion, distribution, prescription or dispensing of products. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or clinical holds on post-approval clinical trials;

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- refusal of the FDA to approve pending NDAs or supplements to approved NDAs, or suspension or withdrawal of product approvals;
- product seizure or detention, or refusal to permit the import or export of products;
- consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs;
- mandated modification of promotional materials and labeling and the issuance of corrective information;
- issuance of safety alerts, Dear Healthcare Provider letters, press releases and other communications containing warnings or other safety information about the product; and
- injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Drugs may be promoted by a manufacturer and any third parties acting on behalf of a manufacturer only for the approved indications and in a manner consistent with the approved label for the product. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

In addition, the distribution of prescription pharmaceutical products is subject to the Prescription Drug Marketing Act ("PDMA"), which regulates the distribution of drugs and drug samples at the federal level and sets minimum standards for the registration and regulation of drug distributors by the states. Both the PDMA and state laws limit the distribution of prescription pharmaceutical product samples and impose requirements to ensure accountability in distribution.

Failure to comply with any of these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Physicians may prescribe, in their independent professional medical judgment, legally available products for uses that are not described in the product's labeling and that differ from those evaluated by us and approved by the FDA. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer's communications on the subject of off-label use of their products. The federal government has levied large civil and criminal fines against companies for alleged improper promotion of off-label use and has enjoined companies from engaging in off-label promotion. The FDA and other regulatory agencies have also required that companies enter into consent decrees and/or imposed permanent injunctions under which specified promotional conduct is changed or curtailed.

Review and Approval of Drugs in the European Union

In the EU, the research, development and commercialization of medicinal products are also subject to extensive regulatory requirements. As in the U.S., medicinal products can only be marketed if a marketing authorization ("MA") from the competent regulatory agencies has been obtained.

Clinical Trial Approval

In April 2014, the European Union adopted a Clinical Trials Regulation (EU) No 536/2014 (the "Clinical Trials Regulation") which replaced the previous Clinical Trials Directive 2001/20/EC on January 31, 2022. The Clinical Trials Regulation is directly applicable in all European Union Member States, meaning no national implementing legislation in each European Union Member State is

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required. The legislation aims at simplifying and streamlining the approval of clinical trials in the European Union. For instance, the Clinical Trials Regulation provides for a streamlined application procedure via a single entry point and strictly defined deadlines for the assessment of clinical trial applications.

Drug Review and Approval

In the European Union, medicinal products can only be commercialized after obtaining an MA. There are two types of MA:

The centralized MA, which is issued by the European Commission through the centralized procedure, based on the opinion of the Committee for Medicinal Products for Human Use of the EMA. A centralized MA is valid throughout the entire territory of the European Union and in the additional Member States of the European Economic Area ("EEA"), which is comprised of the Member States of the European Union plus Norway, Iceland and Liechtenstein. The centralized procedure is mandatory for certain types of products, including medicines produced by certain biotechnological processes, advanced therapy medicinal products (gene-therapy, somatic cell-therapy or tissue-engineered medicines), products designated as orphan medicinal products and products containing a new active substance indicated for the treatment of HIV, AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune and other immune dysfunctions and viral diseases. The centralized procedure is optional for products containing a new active substance not yet authorized in the European Union, or for products that constitute a significant therapeutic, scientific or technical innovation or which are in the interest of public health in the European Union.

National MAs, which are issued by the competent authorities of the Member States of the European Union and only cover their respective territory, are available for products not falling within the mandatory scope of the centralized procedure. Where a product has already been authorized for marketing in a Member State of the European Union, this national MA can be recognized in another Member State through the mutual recognition procedure. If the product has not received a national MA in any Member State at the time of application, it can be approved simultaneously in various Member States through the decentralized procedure.

Under the above described procedures, before granting the MA, the EMA or the competent authorities of the Member States of the European Union make an assessment of the risk-benefit balance of the product on the basis of scientific criteria concerning its quality, safety and efficacy.

Data and Market Exclusivity

In the European Union, innovative medicinal products approved on the basis of a complete and independent data package qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. The data exclusivity, if granted, prevents generic applicants from referencing the innovator's preclinical and clinical trial data contained in the dossier of the reference product when applying for a generic (abbreviated) MA, for eight years from the date on which the reference product was first authorized in the European Union. During an additional two-year period of market exclusivity, a generic marketing authorization application can be submitted, and the innovator's data may be referenced, but no generic or medicinal product can be placed on the European Union market until the expiration of the market exclusivity. The overall ten-year period may be extended to a maximum of 11 years if, during the first eight years of those ten years, the MA holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are determined to bring a significant clinical benefit in comparison with existing therapies. There is no guarantee that a product will be considered by the EMA to be an innovative medicinal product, and products may not qualify for data exclusivity. Even if a

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product is considered to be an innovative medicinal product so that the innovator gains the prescribed period of data exclusivity, another company could nevertheless also market another version of the product if such company obtained an MA based on an application with a complete and independent data package of pharmaceutical tests, preclinical tests and clinical trials.

Orphan Drug Designation and Exclusivity

A product can be designated as an orphan medicinal product by the European Commission if its sponsor can establish that: (1) the product is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (2) either (a) such condition affects no more than five in 10,000 persons in the European Union when the application is made or (b) it is unlikely that the product, without the benefits derived from orphan status, would generate sufficient return in the European Union to justify the necessary investment in its development; and (3) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the European Union or, if such method exists, the product will be of significant benefit to those affected by that condition. Orphan medicinal products are eligible for financial incentives such as reduction of fees or fee waivers. The application for orphan designation must be submitted before the application for marketing authorization. The applicant will receive a fee reduction for the MA application if the orphan designation has been granted, but not if the designation is still pending at the time the MA application is submitted. Orphan designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

Products receiving orphan designation in the European Union can receive ten years of market exclusivity, during which time no "similar medicinal product" may be placed on the market in the European Union. A "similar medicinal product" is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. However, an MA may be granted to a similar medicinal product with the same indication as an authorized orphan product during the ten-year period with the consent of the MA holder for the original orphan medicinal product or if the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities. Marketing authorization may also be granted to a similar medicinal product with the same indication as an authorizes orphan product if the applicant can establish that its similar product is safer, more effective or otherwise clinically superior to the original orphan medicinal product. The period of market exclusivity may, in addition, be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation, for example, if the product is sufficiently profitable not to justify maintenance of market exclusivity.

The aforementioned European Union rules are generally applicable in the EEA, which consists of the European Union Member States, plus Iceland, Liechtenstein and Norway.

Reform of the Regulatory Framework in the European Union

The European Commission introduced legislative proposals in April 2023 that, if implemented, will replace the current regulatory framework in the European Union for all medicines (including those for rare diseases and for children). The European Commission has provided the legislative proposals to the European Parliament and the European Council for their review and approval and, in April 2024, the European Parliament proposed amendments to the legislative proposals. Once the European Commission's legislative proposals are approved (with or without amendment), they will be adopted into European Union law.

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Other U.S. Healthcare Laws and Compliance Requirements

Healthcare providers, including physicians, and third-party payors play a significant role in determining what drug products are used by patients. Our current and future arrangements with healthcare providers, third party payors, patients and other parties within the healthcare industry as well as our business operations more generally may implicate broadly applicable fraud and abuse and other healthcare laws and regulations. Within the U.S., restrictions under applicable federal and state healthcare laws and regulations, including certain laws and regulations applicable only if we have marketed products, include the following:

- the federal healthcare anti-kickback statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the anti-kickback statute or specific intent to violate it in order to have committed a violation;
- the federal False Claims Act ("FCA"), which imposes criminal and civil penalties on individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government and actions under the FCA may be brought by private whistleblowers as well as the government. In addition, the government may assert that a claim including items and services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the FCA;
- the federal civil monetary penalties laws, which impose civil fines for, among other things, the offering or transfer of remuneration to a Medicare or state healthcare program beneficiary if the person knows or should know it is likely to influence the beneficiary's selection of a particular provider, practitioner or supplier of services reimbursable by Medicare or a state healthcare program;
- the federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), as amended, which imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and also establishes requirements related to the privacy, security, and transmission of individually identifiable health information which apply to many healthcare providers, physicians and third-party payors with whom we interact;
- the federal false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;
- the FDCA, which, among other things, strictly regulates drug product and medical device marketing, prohibits manufacturers from marketing such products for off-label use and regulates the distribution of samples;
- federal laws, such as the Medicaid Drug Rebate Program, that require pharmaceutical manufacturers to report certain calculated product prices to the government or provide certain discounts or rebates to government authorities or private entities, often as a condition of reimbursement under governmental healthcare programs;
- federal and state consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers;
- the so-called federal "sunshine law" or Open Payments which requires manufacturers of drugs, devices, biologics, and medical supplies to report to the Centers for Medicare & Medicaid Services information related to payments and other transfers of value to teaching hospitals,

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physicians, and other healthcare practitioners, as well as ownership and investment interests held by physicians and their immediate family members; and

- analogous state laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non- governmental third-party payors, including private insurers, and state laws which regulate interactions between pharmaceutical companies and healthcare providers, require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, require pharmaceutical companies to report information on transfers of value to other healthcare providers, marketing expenditures or pricing information and/or require licensing of sales representatives. State laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive record-keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products. Some states also impose requirements on manufacturers and distributors to establish the pedigree of product in the chain of distribution, including some states that require manufacturers and others to adopt new technology capable of tracking and tracing product as it moves through the distribution chain.

Given the breadth of the laws and regulations and narrowness of any exceptions, limited guidance for certain laws and regulations and evolving government interpretations of the laws and regulations, it is possible that some of our business activities could be subject to challenge under one or more of such laws. Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant penalties, including, without limitation, civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from participating in federal and state funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, diminished profits and future earnings, reputational harm and the curtailment or restructuring of our operations, any of which could harm our business.

Payments made to physicians in certain European Union Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization, and/or the regulatory authorities of the individual European Union Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct applicable in the European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, exclusion from participation in governmental healthcare programs, disgorgement, fines or imprisonment.

Healthcare Reform

In the U.S. and some foreign jurisdictions, there have been and continue to be ongoing efforts to implement legislative and regulatory changes regarding the healthcare system. Such changes could prevent or delay marketing approval of any product candidates that we may develop, restrict or

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regulate post-approval activities and affect our ability to profitably sell any product candidates for which we obtain marketing approval. Although we cannot predict what healthcare or other reform efforts will be successful, such efforts may result in more rigorous coverage criteria, in additional downward pressure on the price that we, or our future collaborators, may receive for any approved products or in other consequences that may adversely affect our ability to achieve or maintain profitability.

Within the U.S., the federal government and individual states have aggressively pursued healthcare reform. For example, the Affordable Care Act ("ACA"), implemented in 2010, substantially changed the way healthcare is financed by both governmental and private insurers and contains a number of provisions that affect coverage and reimbursement of drug products and/or that could potentially reduce the demand for pharmaceutical products such as increasing drug rebates under state Medicaid programs for brand name prescription drugs and extending those rebates to Medicaid managed care and assessing a fee on manufacturers and importers of brand name prescription drugs reimbursed under certain government programs, including Medicare and Medicaid. Other aspects of healthcare reform, such as expanded government enforcement authority, could also affect our business.

Beyond the ACA, there have been ongoing health care reform efforts. Drug pricing and payment reform was a focus of the Trump Administration and has been an ongoing focus of the Biden Administration. For example, federal legislation enacted in 2021 eliminated the statutory cap on Medicaid drug rebate program rebates (currently set at 100% of a drug's "average manufacturer price") effective January 1, 2024. As another example, the Inflation Reduction Act of 2022 ("IRA") includes a number of changes intended to address rising prescription drug prices in Medicare Parts B and D. These changes, which have varying implementation dates, include caps on Medicare Part D out-of-pocket costs for beneficiaries, Medicare Part B and Part D drug price inflation rebates, a new Medicare Part D manufacturer discount drug program (replacing the ACA Medicare Part D coverage gap discount program) and a drug price negotiation program for certain high spend Medicare Part B and D drugs (with the first set of negotiated prices going into effect January 1, 2026). The focus on health care reform, including reform of drug pricing and payment, has continued in the wake of the IRA. President Biden has announced initiatives, including executive orders. that have sought to reduce prescription drug and other health care costs. Although a number of these and other proposed measures may require authorization through additional legislation to become effective, and the Biden administration may reverse or otherwise change these measures, both the Biden Administration and Congress have indicated that they will continue to seek new legislative measures to control drug costs. For example, in 2022, subsequent to the enactment of the IRA, the Biden administration released an executive order directing the HHS to report on how the Center for Medicare and Medicaid Innovation ("CMMI") could be leveraged to test new models for lowering drug costs for Medicare and Medicaid beneficiaries, which report proposed various models that CMMI is currently developing. Individual states in the U.S. have also become increasingly active in implementing regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Healthcare reform efforts have been and may continue to be subject to scrutiny and legal challenge. For example, with respect to the ACA, tax reform legislation was enacted that eliminated the tax penalty established for individuals who do not maintain mandated health insurance coverage beginning in 2019 and, in 2021, the U.S. Supreme Court dismissed the latest judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. As another example, revisions to regulations under the federal anti-kickback statute would remove protection for traditional Medicare Part D discounts offered by pharmaceutical manufacturers to pharmacy benefit managers and health plans. Pursuant to court order, the removal was delayed and subsequent legislation imposed a moratorium on implementation of the rule until January 2032. As another

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example, the IRA drug price negotiation program has been challenged in litigation filed by various pharmaceutical manufacturers and industry groups.

In the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize our potential product candidates. Recently, many countries in the European Union have increased the amount of discounts required on pharmaceuticals and these efforts could continue as countries attempt to manage healthcare expenditures, especially in light of the severe fiscal and debt crises experienced by many countries in the European Union. The downward pressure on healthcare costs in general, particularly prescription products, has become intense. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various Member States, and parallel trade, i.e., arbitrage between low-priced and high-priced Member States, can further reduce prices. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products, if approved in those countries.

Coverage and Reimbursement

The regulations that govern marketing approvals, pricing, coverage and reimbursement for new drug products vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Our ability to commercialize any product candidates successfully also will depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and other third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. Coverage and reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor’s determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

In the U.S., there is no uniform policy of coverage and reimbursement for products among third-party payors. As a result, obtaining coverage and reimbursement approval of a product from a government or other third-party payor is a time-consuming and costly process that could require us to provide to each payor supporting scientific, clinical and cost-effectiveness data for the use of our products on a payor-by-payor basis, with no assurance that coverage and adequate reimbursement

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will be obtained. The availability and adequacy of coverage and reimbursement by governmental healthcare programs such as Medicare and Medicaid, private health insurers and other third-party payors are essential for most patients to be able to afford our product candidates, if approved, and so will significantly affect our ability to successfully commercialize any such product candidates.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Coverage may not be available for any product that we commercialize or may be subject to controls imposed by third party payors to manage utilization (e.g., requiring specific approval for use of a product in a particular patient for coverage). Even if coverage is available, the resulting reimbursement payment rates may not be adequate or may require patient out-of-pocket costs that patients find unacceptably high. Reimbursement may affect the demand for, or the price of, any product candidate for which we obtain marketing approval. Obtaining and maintaining adequate reimbursement for our products may be difficult. We may be required to conduct expensive pharmacoeconomic studies to justify coverage and reimbursement or the level of reimbursement relative to other therapies. If coverage and adequate reimbursement are not available or reimbursement is available only to limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval. Additionally, we, or our collaborators, will be required to obtain coverage and reimbursement for any companion diagnostic tests developed separate and apart from the coverage and reimbursement we may seek for our product candidates, once approved.

There may be significant delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the FDA or similar regulatory authorities outside of the U.S. Moreover, eligibility for coverage and reimbursement does not imply that a drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution expenses. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or requested by private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the U.S. Our inability to promptly obtain coverage and adequate reimbursement rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

In some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, the European Union provides options for its Member States to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost effectiveness of a particular product candidate to currently available therapies. A Member State may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. Historically, products launched in the European Union do not follow price structures of the U.S. and generally prices tend to be significantly lower.

There can be no assurance that our product candidates, even if they are approved for sale in the U.S. or in other countries, will be considered medically reasonable and necessary for a specific

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indication or cost-effective by third-party payors, or that coverage and an adequate level of reimbursement will be available or that third-party payors' reimbursement policies will not adversely affect our ability to sell our product candidates profitably.

Data Privacy and Security Laws

Numerous state, federal and foreign laws, regulations, and standards govern the collection, use, access to, confidentiality and security of health-related and other personal information and could apply now or in the future to our operations or the operations of our partners. In the U.S., numerous federal and state laws and regulations, including data breach notification laws, health information privacy and security laws and consumer protection laws and regulations govern the collection, use, disclosure, and protection of health-related and other personal information. In addition, certain foreign laws govern the privacy and security of personal data, including health-related data. Privacy and security laws, regulations and other obligations are constantly evolving, may conflict with each other to complicate compliance.

Employees and Human Capital Resources

As of December 31, 2024, we had 35 full-time employees, of which 13 had M.D. or Ph.D. degrees. Within our workforce, 21 employees were engaged in research and development and 14 were engaged in business development, finance, legal and general management and administration as of December 31, 2024. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and new employees, advisors and consultants. The principal purposes of our equity incentive plans are to attract, retain and reward personnel through the granting of equity-based compensation awards in order to increase shareholder value and the success of our company by motivating such individuals to perform to the best of their abilities and achieve our objectives.

We believe that our future success largely depends upon our continued ability to attract and retain highly skilled employees. We provide our employees with competitive salaries and bonuses, opportunities for equity ownership, development programs that enable continued learning and growth and a robust employment package that promotes well-being across all aspects of their lives, including health care, retirement planning and paid time off.

We believe that much of our success is rooted in the diversity of our teams and our commitment to inclusion. We value diversity at all levels and focus on extending our diversity and inclusion initiatives across our entire workforce.

Facilities

Our corporate headquarters is located in Waltham, Massachusetts, where we lease and occupy approximately 24,051 square feet of laboratory and office space at 21 Hickory Drive, Suite 500, Waltham, Massachusetts 02451. We sublease approximately 6,399 square feet of such laboratory and office space. The current term of our lease expires in November 2030.

We believe that our facilities are adequate for our current needs and for the foreseeable future. To meet the future needs of our business, we may lease additional or alternate space. We believe that suitable additional or substitute space at commercially reasonable terms will be available as needed to accommodate any future expansion of our operations.

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Legal Proceedings

From time to time, we may become involved in legal proceedings arising from the ordinary course of business. We record a liability for such matters when it is probable that future losses will be incurred and that such losses can be reasonably estimated. Significant judgment by us is required to determine both probability and the estimated amount. Our management is currently not aware of any legal matters that could have a material effect on our financial position, results of operations or cash flows.

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MANAGEMENT

Executive Officers, Key Employees and Directors

The following table sets forth information regarding our executive officers, key employees and directors as of December 31, 2024.

Name	Age	Position
Executive Officers:		
Michael Cloonan, M.B.A.	53	Chief Executive Officer, President and Director
Charlotte McKee, M.D.	60	Chief Medical Officer
Elena Ridloff, C.F.A.	45	Chief Financial Officer and Head of Corporate Development
Non-Employee Directors:		
Paul Clancy, M.B.A.(2)(3)	63	Chairperson of the Board
Bruce Booth, D.Phil.(1)	50	Director
H. Edward Fleming, Jr., M.D.(2)	61	Director
Lucian Iancovici, M.D.(1)	42	Director
Joshua Resnick, M.D., M.B.A.	50	Director
Marcella Kuhlman Ruddy, M.D.(3)	62	Director
Laurie Stelzer, M.B.A.(1)(2)	57	Director
Peter A. Thompson, M.D.(3)	65	Director
Joanne Louise Viney, Ph.D.(1)	59	Director

(1) Member of the compensation committee.
(2) Member of the audit committee.
(3) Member of the nominating and corporate governance committee.

Executive Officers

Michael Cloonan, M.B.A., has served as our President, Chief Executive Officer and a member of our board of directors since May 2021. Previously, Mr. Cloonan served as the Chief Operating Officer of Sage Therapeutics, Inc. (NASDAQ: SAGE) ("Sage") from May 2020 to May 2021, and as the chief business officer of Sage from April 2017 to May 2020. Prior to joining Sage, Mr. Cloonan served in various leadership positions at Biogen, Inc. (NASDAQ: BIIB) ("Biogen") in various business and commercial roles in his fourteen years at Biogen. His most recent role at Biogen was as senior vice president, U.S. Commercial, where he was the general manager of the multi-billion dollar MS, hemophilia and SMA franchises. Mr. Cloonan has also served on the board of directors of bluebird bio, Inc. (NASDAQ: BLUE) since June 2024. Mr. Cloonan earned a B.A. in economics and accounting from the College of the Holy Cross and an M.B.A. from the Darden Graduate School of Business Administration at the University of Virginia. We believe Mr. Cloonan's extensive leadership experience in the biotechnology industry and experience as a public company board member provides him with the appropriate set of skills to serve as a member of our board of directors.

Charlotte McKee, M.D., has served as our Chief Medical Officer since June 2021. Previously, Dr. McKee served as vice president, Cystic Fibrosis and Alpha-1 Antitrypsin Deficiency Clinical Development at Vertex (NASDAQ: VRTX), from June 2014 to March 2020. Dr. McKee earned a B.A. in history and science from Harvard University, an M.A. in history from Columbia University and an M.D. from Columbia University Vagelos College of Physicians and Surgeons, and completed internal medicine training and a pulmonary and critical care fellowship at Johns Hopkins University School of Medicine.

Elena Ridloff, C.F.A., has served as our Chief Financial Officer and Head of Corporate Development since September 2021. Previously, Ms. Ridloff served in multiple roles at Acadia

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Pharmaceuticals Inc. (NASDAQ: ACAD) from 2018 to 2021, including most recently as chief financial officer from October 2018 to September 2021. Ms. Ridloff has also served on the board of directors of Kymera Therapeutics, Inc. (NASDAQ: KYMR) ("Kymera"), since March 2021 and Kronos Bio, Inc. (NASDAQ: KRON) since September 2020. Ms. Ridloff earned a B.A. in history and sociology of science from the University of Pennsylvania and is a Chartered Financial Analyst.

Non-Executive Directors

Bruce Booth, D.Phil., has served as a member of our board of directors since June 2020. Dr. Booth joined Atlas Venture in 2005 and currently serves as general partner. He also serves, or has previously served, as an advisor in various capacities to Takeda Pharmaceuticals Company Limited (NYSE: TAK), UCB Biopharmaceuticals and the Bill & Melinda Gates Foundation. Dr. Booth co-founded Kymera and has served as chairman of its board of directors since April 2016. He has also served as chairman of Vigil Neuroscience, Inc. (NASDAQ: VIGIL) since June 2020. Dr. Booth is chair or a member of the boards of directors of several private companies, including Nimbus Therapeutics, LLC, HotSpot Therapeutics, Inc., Arkuda Therapeutics, Inc. and Matchpoint Therapeutics, Inc. Dr. Booth previously served on the boards of directors of AVROBIO, Inc. (now Tectonic Therapeutic, Inc. (NASDAQ: TECX)) from February 2016 to June 2024, Magenta Therapeutics, Inc. (now Dianthus Therapeutics, Inc. (NASDAQ: DNTH)) from February 2016 to September 2023 and Unum Therapeutics, Inc. (now Cogent Biosciences, Inc. (NASDAQ: COGT)) from October 2014 to July 2020 and several private companies. Dr. Booth earned a B.S. in biochemistry, summa cum laude, from Pennsylvania State University. Dr. Booth was a British Marshall Scholar and earned a D.Phil. in molecular immunology from Oxford University's Nuffield Department of Medicine. We believe Dr. Booth's extensive leadership, executive, managerial and business experience with life sciences companies and experience as a public company board member provides him with the appropriate set of skills to serve as a member of our board of directors.

Paul Clancy, M.B.A., has served as chair of our board of directors since June 2022. Mr. Clancy is currently a visiting senior lecturer of Finance at Cornell University's Graduate School of Management, where he has been employed since August 2020, and has been an executive fellow at Harvard Business School since July 2020. Previously, Mr. Clancy served as executive vice president and senior advisor at Alexion Pharmaceutical, Inc. (NASDAQ: ALXN) from November 2019 to July 2020 and executive vice president and chief financial officer from July 2017 to October 2019. Mr. Clancy also served as executive vice president and chief financial officer of Biogen, Inc. (NASDAQ: BIIB) from August 2007 to June 2017. Mr. Clancy also serves on the boards of directors of Exact Sciences Corporation (NASDAQ: EXAS), since March 2021, Xilio Therapeutics, Inc. (NASDAQ: XLO), since July 2020, and Incyte Corporation (NASDAQ: INCY), since 2015. Mr. Clancy previously served on the board of directors of Agios Pharmaceuticals, Inc. (NASDAQ: AGIO) from September 2013 to June 2023. Mr. Clancy earned a B.S. in finance from Babson College and an M.B.A. from Columbia University. We believe Mr. Clancy's significant financial background in the biopharmaceutical industry and experience as a public company board member provides him with the appropriate set of skills to serve as a member of our board of directors.

H. Edward Fleming, Jr., M.D., has served as a member of our board of directors since March 2024. Dr. Fleming joined Enavate Sciences in November 2022 and serves as executive vice president of commercialization, investing in and building therapeutic companies. Previously, in 2022, Dr. Fleming retired from McKinsey & Company, where he worked for 26 years in various roles, including senior partner, global leader of research and development and senior advisor. Dr. Fleming has also served on the boards of directors of Upstream Bio, Inc. (NASDAQ: UPB) since June 2023 and of CRISPR Therapeutics AG (NASDAQ: CRSP) since June 2021. In addition, Dr. Fleming is a member of the boards of directors of several private companies, including Komodo Health, Inc., Orso Bio, Inc., Sudo Biosciences, Inc. and Egnite, Inc., and serves on the board of visitors of Vanderbilt's School of Basic

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Sciences. Dr. Fleming earned a B.A. in chemistry from Harvard University and an M.D. from Vanderbilt University, and completed internal medicine training at Johns Hopkins Hospital and subspecialty training in pulmonary and critical care medicine at the University of California, San Francisco. We believe Dr. Flemings's extensive background in the healthcare industry and experience working closely with biopharmaceutical companies provides him with the appropriate set of skills to serve as a member of our board of directors.

Lucian Iancovici, M.D., has served as a member of our board of directors since December 2019. Dr. Iancovici is currently a managing director with TPG, a private equity investment firm, where he has worked since January 2018. From September 2012 to October 2017, Dr. Iancovici served as the head of the Qualcomm Life Fund ("Qualcomm"), a venture fund focused on investing in digital health technologies. From January 2015 to October 2017, Dr. Iancovici was a general partner at dRx Capital, a joint venture investment company launched by Novartis and Qualcomm. From 2011 to 2012, Dr. Iancovici was an associate at McKinsey & Company. Dr. Iancovici has served on the board of directors of Rallybio Corp (NASDAQ: RLYB) since May 2020 and serves on the boards of the following private companies: K2 Medical Research, Ceribell, Inc., Ellodi Pharmaceuticals and AnovoRx Holdings, Inc. Dr. Iancovici is a board certified internal medicine doctor who trained at Columbia University Medical Center in New York prior to joining McKinsey & Company. Dr. Iancovici received a B.A. in economics and an M.D., both from Tufts University. We believe that Dr. Iancovici's extensive experience in the venture capital industry and his medical and scientific background and training provide him with the appropriate set of skills to serve as a member of our board of directors.

Joshua Resnick, M.D., M.B.A., has served as a member of our board of directors since December 2019. Dr. Resnick is a senior managing director at RA Capital Management, L.P., where he previously served as a Managing Director from October 2018 to March 2023. Dr. Resnick is also a faculty member at Harvard Medical School and continues to practice medicine as an attending physician in the Department of Emergency Medicine at Massachusetts General Hospital. Dr. Resnick has served on the boards of directors of PepGen Inc. (NASDAQ: PEPG) since November 2020, Aerovate Therapeutics, Inc. (NASDAQ: AVTE) since August 2020, as well as from October 2018 to February 2020, and Vor Biopharma Inc. (NASDAQ: VOR) since February 2019. Dr. Resnick received a B.A. in chemistry from Williams College, an M.D. from the University of Pennsylvania Perelman School of Medicine and an M.B.A. from The Wharton School of the University of Pennsylvania. We believe Dr. Resnick's biopharmaceutical industry and investor experience and medical background provides him with the appropriate set of skills to serve as a member of our board of directors.

Marcella Kuhlman Ruddy, M.D., has served as a member of our board of directors since January 2025. Dr. Ruddy has over 20 years of experience in drug development. Dr. Ruddy has served as the chief medical officer of Tectonic Therapeutic, Inc. (NASDAQ: TECX) since July 2021. From June 2016 to June 2021, Dr. Ruddy served as vice president of clinical development, immunology at Regeneron Pharmaceuticals, Inc. (NASDAQ: REGN), where she oversaw the clinical development of Dupixent. She is a trained pulmonologist who founded and led the Adult Cystic Fibrosis center at Massachusetts General Hospital, Boston from 1999-2004 prior to starting her drug development career at Merck & Co Inc. (NYSE: MRK). Dr. Ruddy has served on the board of directors of Upstream Bio, Inc. (NASDAQ: UPB) since January 2023. Dr. Ruddy previously served on the board of directors of Polarean, Inc. from August 2022 to June 2024. Dr. Ruddy received her B.A. in history from Princeton University and her M.D. and M.S. from Washington University, School of Medicine, St. Louis. We believe Dr. Ruddy's extensive experience in drug development and background in the biopharmaceutical industry provides her with the appropriate set of skills to serve as a member of our board of directors.

Laurie Stelzer, M.B.A., has served as a member of our board of directors since November 2024. Ms. Stelzer has served as the Chief Financial Officer of Kailera Therapeutics, Inc. ("Kailera") since January 2025. Prior to Kailera, Ms. Stelzer served as the Chief Financial Officer of Orna

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Therapeutics (“Orna”) from May 2024 to January 2025. Prior to Orna, Ms. Stelzer served as the Chief Financial Officer of ReNAgade Therapeutics, Inc. (“ReNAgade”) from September 2023 until the completion of its acquisition by Orna in May 2024. Prior to joining ReNAgade, Ms. Stelzer served as the Chief Financial Officer of Mirati Therapeutics, Inc. (Nasdaq: MRTX) (“Mirati Therapeutics”) from May 2022 to September 2023. Prior to joining Mirati Therapeutics, Ms. Stelzer served as the Executive Vice President and Chief Financial Officer of Arena Pharmaceuticals, Inc. (“Arena Pharmaceuticals”) (acquired by Pfizer) from March 2020 until the completion of Pfizer’s acquisition in March 2022. Prior to joining Arena Pharmaceuticals, Ms. Stelzer served as the Chief Financial Officer at Halozyme Therapeutics, Inc. (“Halozyme Therapeutics”) (Nasdaq: HALO) from June 2015 to March 2020, and before Halozyme Therapeutics, she held senior management roles at Shire Plc (acquired by Takeda Pharmaceuticals Company Limited (NYSE: TAK)) and Amgen, Inc. Ms. Stelzer has served as a member of the board of directors of PMV Pharmaceuticals, Inc. (Nasdaq: PMVP) since 2020, Spyre Therapeutics, Inc. (Nasdaq: SYRE) since 2023, Surface Oncology, Inc. (Nasdaq: SURF) from 2018 until its acquisition by Coherus in September 2023 and Longboard Pharmaceuticals from 2020 to 2021. Ms. Stelzer received her B.S. in Accounting from Arizona State University and her M.B.A. from University of California, Los Angeles, Anderson School of Management. We believe Ms. Stelzer’s executive and business experience in the biopharmaceutical industry provides her with the appropriate set of skills to serve as a member of our board of directors.

Peter A. Thompson, M.D., has served as a member of our board of directors since February 2022. Dr. Thompson is a member at OrbiMed Advisors LLC, an investment firm, where he has served in various roles of increasing responsibility since 2010, including as Partner from 2013 to 2021. Dr. Thompson currently serves on the boards of directors of ARS Pharmaceuticals Inc. (formerly Silverback Therapeutics, Inc.) (NASDAQ: SPRY), since April 2016, Corvus Pharmaceuticals, Inc. (NASDAQ: CRVS), since November 2014, and Edgewise Therapeutics, Inc. (NASDAQ: EWTX), since May 2017, as well as several private companies. Previously, Dr. Thompson served on the boards of directors of Alpine Immune Sciences, Inc. (NASDAQ: ALPN), Decibel Therapeutics, Inc. (NASDAQ: DBTX), Janux Therapeutics, Inc. (NASDAQ: JANX), PMV Pharmaceuticals, Inc. (NASDAQ: PMVP), Prevail Therapeutics Inc. (NASDAQ: PRVL) and Synthorx, Inc., until its acquisition by Sanofi. Dr. Thompson previously served in executive leadership roles at Trubion Pharmaceuticals, Inc., Chiron Corporation and Becton, Dickinson and Company. Dr. Thompson is also an Affiliate Professor of Neurosurgery at the University of Washington. In addition, Dr. Thompson holds a Sc. B. in Molecular Biology and Mathematics from Brown University and an M.D. from Brown University Medical School. We believe Dr. Thompson’s management and venture capital experience in the biopharmaceutical industry provides him with the appropriate set of skills to serve as a member of our board of directors.

Joanne Louise Viney, Ph.D., has served as a member of our board of directors since January 2025. Dr. Viney is a co-founder and has served as the chief executive officer of Seismic Therapeutic (“Seismic”) since October 2021. Prior to that, Dr. Viney co-founded and served as the chief scientific officer of Pandion Therapeutics, Inc. (NASDAQ: PAND) (“Pandion”) (acquired by Merck & Co Inc. (NYSE: MRK)) from March 2017 to October 2021. Dr. Viney currently serves on the boards of directors of several private companies, including Seismic and LabCentral. Dr. Viney previously served on the boards of directors of Graphite Bio, Inc. (NASDAQ: GRPH) (now Lenz Therapeutics, Inc. (NASDAQ: LENZ)) from March 2021 to March 2024, Harpoon Therapeutics, Inc. (NASDAQ: HARP) from July 2020 to June 2023, Finch Therapeutics Group, Inc. (NASDAQ: FNCH) from August 2019 to March 2023 and several private companies. Dr. Viney received her B.Sc. with Honors in biophysical science from the University of East London and her Ph.D. from the University of London, St. Bartholomew’s Hospital Medical School. We believe Dr. Viney’s substantial leadership experience and scientific background in the biotechnology industry provides her with the appropriate set of skills to serve as a member of our board of directors.

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Family Relationships

There are no family relationships among any of our executive officers or directors.

Composition of our Board of Directors

Our business and affairs are managed under the direction of our board of directors, which currently consists of nine members. The primary responsibilities of our board of directors are to provide oversight, strategic guidance, counseling and direction to our management. Our board of directors meets on a regular basis and additionally as required.

Certain members of our board of directors were elected under the provisions of our fourth amended and restated certificate of incorporation and agreements with our stockholders. These board composition provisions will terminate upon the completion of this offering. Upon the termination of these provisions, there will be no further contractual obligations regarding the election of our directors. Our nominating and corporate governance committee and our board of directors may therefore consider a broad range of factors relating to the qualifications and background of nominees. Our nominating and corporate governance committee's and our board of directors' priority in selecting board members is identification of persons who will further the interests of our stockholders through their established record of professional accomplishment, the ability to contribute positively to the collaborative culture among board members, knowledge of our business, understanding of the competitive landscape, professional and personal experiences and expertise relevant to our growth strategy. Our directors hold office until their successors have been elected and qualified or until the earlier of their resignation or removal. Our fifth amended and restated certificate of incorporation, which will become effective immediately prior to the completion of this offering, and our amended and restated bylaws, which became effective upon the effectiveness of the registration statement of which this prospectus forms a part, also provide that our directors may be removed only for cause by the affirmative vote of the holders of at least two-thirds of the votes that all our stockholders would be entitled to cast in an annual election of directors, and that any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office.

Staggered Board

Our fifth amended and restated certificate of incorporation, which will become effective immediately prior to the completion of this offering, and our amended and restated bylaws, which became effective upon the effectiveness of the registration statement of which this prospectus forms a part, permit our board of directors to establish the authorized number of directors from time to time by resolution. Each director serves until the expiration of the term for which such director was elected or appointed, or until such director's earlier death, resignation or removal. In accordance with our fifth amended and restated certificate of incorporation, our board of directors will be divided into three classes with staggered three-year terms. At each annual meeting of stockholders, the successors to directors whose terms then expire will be elected to serve from the time of election and qualification until the third annual meeting following election. Our directors will be divided among the three classes as follows:

- the Class I directors will be Joshua Resnick, M.D., M.B.A., Bruce Booth, D.Phil., and Lucian Iancovici, M.D., and their terms will expire at our first annual meeting of stockholders following this offering, to be held in 2025;
- the Class II directors will be H. Edward Fleming, Jr., M.D., Peter A. Thompson, M.D., Joanne Louise Viney, Ph.D., and Marcella Kuhlman Ruddy, M.D., and their terms will expire at our second annual meeting of stockholders following this offering, to be held in 2026; and
- the Class III directors will be Paul Clancy, M.B.A., Michael Cloonan, M.B.A., and Laurie Stelzer, M.B.A. and their terms will expire at our third annual meeting of stockholders following this offering, to be held in 2027.

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We expect that any additional directorships resulting from an increase in the number of directors will be distributed among the three classes so that, as nearly as possible, each class will consist of one third of the directors. The division of our board of directors into three classes with staggered three-year terms may delay or prevent a change of our management or a change in control.

This classification of our board of directors may have the effect of delaying or preventing changes in control of our company.

Director Independence

Under the listing standards, requirements and rules of The Nasdaq Stock Market LLC (the "Nasdaq Listing Rules"), independent directors must comprise a majority of our board of directors as a listed company within one year of the listing date.

Our board of directors has undertaken a review of the independence of each director. Based on information provided by each director concerning his or her background, employment and affiliations, including family relationships, our board of directors has determined that none of the members of our board of directors, except Mr. Cloonan, have relationships that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director and that each of the directors, except Mr. Cloonan, is "independent" as that term is defined under the Nasdaq Listing Rules. Our board of directors has determined that Mr. Cloonan, by virtue of his position as our current Chief Executive Officer, is not independent under applicable rules and regulations of the SEC and the Nasdaq Listing Rules. In making these determinations, our board of directors considered the current and prior relationships that each non-employee director has with our company and all other facts and circumstances our board of directors deemed relevant in determining their independence, including the beneficial ownership of our shares by each non-employee director and the transactions described in "*Certain Relationships and Related Person Transactions*."

Board Diversity

Our nominating and corporate governance committee and our board of directors may consider a broad range of factors relating to the qualifications and background of director nominees, such as a candidate's character, judgment, skills, education, expertise and absence of conflicts of interest, as well as diversity. The nominating and corporate governance committee and the full board of directors are committed to creating a board of directors with diversity, including diversity of expertise, experience, background and gender, and are committed to identifying, recruiting and advancing candidates offering such diversity in future searches.

Board Leadership Structure and Board's Role in Risk Oversight

Mr. Clancy is the current chair of our board of directors and Mr. Cloonan is our current Chief Executive Officer, hence the roles of chair of our board of directors and chief executive officer are separated. We believe that separating these positions allows our Chief Executive Officer to focus on our day-to-day business, while allowing the chair of our board to lead the board of directors in its fundamental role of providing advice to and independent oversight of management. Our board of directors recognizes the time, effort and energy that the Chief Executive Officer is required to devote to his position in the current business environment, as well as the commitment required to serve as chair of our board of directors, particularly as the board of directors' oversight responsibilities continue to grow. While our bylaws and corporate governance guidelines do not require that our board chair and Chief Executive Officer positions be separate, our board of directors believes that having separate positions is the appropriate leadership structure for us at this time and demonstrates our commitment to good corporate governance.

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Risk is inherent with every business, and how well a business manages risk can ultimately determine its success. We face a number of risks, including risks relating to our financial condition, development and commercialization activities, operations, strategic direction and intellectual property as more fully discussed in "*Risk Factors*" appearing elsewhere in this prospectus. Management is responsible for the day-to-day management of risks we face, while our board of directors, as a whole and through its committees, has responsibility for the oversight of risk management. In its risk oversight role, our board of directors has the responsibility to satisfy itself that the risk management processes designed and implemented by management are adequate and functioning as designed.

The role of the board of directors in overseeing the management of our risks is conducted primarily through committees of the board of directors, as disclosed in the descriptions of each of the committees below and in the charters of each of the committees. The full board of directors (or the appropriate board committee in the case of risks that are under the purview of a particular committee) discusses with management our major risk exposures, their potential impact on us, and the steps we take to manage them. When a board committee is responsible for evaluating and overseeing the management of a particular risk or risks, the chairperson of the relevant committee reports on the discussion to the full board of directors during the committee reports portion of the next board meeting. This enables the board of directors and its committees to coordinate the risk oversight role, particularly with respect to risk interrelationships.

Committees of Our Board of Directors

Our board of directors has established an audit committee, a compensation committee and a nominating and corporate governance committee. The composition and responsibilities of each of the committees of our board of directors are described below. Members serve on these committees until their resignation or until otherwise determined by our board of directors. Each committee intends to adopt a written charter that satisfies the application rules and regulation of the SEC and the Nasdaq Listing Rules, which we will post to our website at *www.sionnatx.com* upon the completion of this offering. Our board of directors may establish other committees as it deems necessary or appropriate from time to time.

Audit Committee

Our audit committee currently consists of Laurie Stelzer, M.B.A., the chair of our audit committee, Paul Clancy, M.B.A. and H. Edward Fleming, Jr., M.D. Our board of directors has determined that each member of the audit committee is independent under Nasdaq Listing Rules and Rule 10A-3(b)(1) of the Exchange Act and can read and understand fundamental financial statements in accordance with applicable requirements. Our board of directors has also determined that each of Mr. Clancy and Ms. Stelzer is an "audit committee financial expert" within the meaning of SEC regulations. In arriving at these determinations, our board of directors has examined each audit committee member's scope of experience and the nature of their employment in the corporate finance sector.

The primary purpose of the audit committee is to discharge the responsibilities of our board of directors with respect to our corporate accounting and financial reporting processes, systems of internal control and financial statement audits, and to oversee our independent registered public accounting firm. Specific responsibilities of our audit committee include:

- helping our board of directors oversee our corporate accounting and financial reporting processes;
- managing the selection, engagement, qualifications, independence and performance of a qualified firm to serve as the independent registered public accounting firm to audit our consolidated financial statements;

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- discussing the scope and results of the audit with the independent registered public accounting firm, and reviewing, with management and the independent accountants, our interim and year-end operating results;
- developing procedures for employees to submit concerns anonymously about questionable accounting or audit matters;
- approving insurance coverage for our officers and directors;
- preparing the audit committee report to be included in our annual proxy statement, reviewing with management our consolidated financial statements to be included in our quarterly reports to be filed with the SEC and reviewing with management the "*Risk Factors*" and "*Management's Discussion and Analysis of Financial Condition and Results of Operations*" disclosures in our periodic reports filed with the SEC;
- oversee our risk management policies, procedures and practices, including those related to cybersecurity; and
- approving, or, as permitted, pre-approving, audit and permissible non-audit services to be performed by the independent registered public accounting firm.

Our audit committee will operate under a written charter, which will be effective upon the completion of this offering, that satisfies the applicable Nasdaq Listing Rules.

Compensation Committee

Our compensation committee currently consists of Joanne Louise Viney, Ph.D., the chair of our compensation committee, Lucian Iancovici, M.D., Bruce Booth, D.Phil. and Laurie Stelzer, M.B.A. Our board of directors has determined that each member of the compensation committee is independent under the Nasdaq Listing Rules and is a "non-employee director" as defined in Rule 16b-3 promulgated under the Exchange Act.

The primary purpose of our compensation committee is to discharge the responsibilities of our board of directors in overseeing our compensation policies, plans and programs and to review and determine the compensation to be paid to our executive officers, directors and other senior management, as appropriate. Specific responsibilities of our compensation committee include:

- annually reviewing and recommending to our board of directors the corporate goals and objectives relevant to the compensation of our Chief Executive Officer;
- evaluating the performance of our Chief Executive Officer in light of such corporate goals and objectives and, based on such evaluation, recommending to our board of directors the cash compensation of our Chief Executive Officer;
- reviewing and approving the compensation arrangements with our other executive officers and certain other senior management;
- reviewing and recommending to our board of directors the compensation paid to our directors;
- administering our equity incentive plans and other benefit programs;
- overseeing and administering our compensation and similar plans; and
- reviewing and establishing general policies relating to compensation and benefits of our employees, including our overall compensation philosophy.

Our compensation committee will operate under a written charter, which will be effective upon the completion of this offering, that satisfies the applicable Nasdaq Listing Rules.

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Nominating and Corporate Governance Committee

Our nominating and corporate governance committee currently consists of Paul Clancy, M.B.A., chair of our nominating and corporate governance committee, Marcella Kuhlman Ruddy, M.D. and Peter A. Thompson, M.D. Our board of directors has determined that each member of the nominating and corporate governance committee is independent under the Nasdaq Listing Rules, a non-employee director, and free from any relationship that would interfere with the exercise of his or her independent judgment.

The primary purpose of the nominating and corporate governance committee is to discharge the responsibilities of our board of directors with respect to our corporate governance functions and to identify, communicate with, evaluate and recommend candidates for our board of directors. Specific responsibilities of our nominating and corporate governance committee include:

- identifying and evaluating candidates, including the nomination of incumbent directors for reelection and nominees recommended by stockholders, to serve on our board of directors;
- considering and making recommendations to our board of directors regarding the composition and chairmanship of the committees of our board of directors;
- instituting plans or programs for the continuing education of our board of directors and orientation of new directors;
- developing and making recommendations to our board of directors regarding corporate governance guidelines and matters; and
- overseeing periodic evaluations of the board of directors' performance, including committees of the board of directors and management.

Our nominating and corporate governance committee will operate under a written charter, which will be effective upon the completion of this offering, that satisfies the applicable Nasdaq Listing Rules.

Code of Business Conduct and Ethics

In connection with this offering, we adopted an amended and restated code of business conduct and ethics that applies to all our employees, officers and directors, to be effective upon the effectiveness of the registration statement of which this prospectus forms a part. This includes our principal executive officer, principal financial officer and principal accounting officer or controller, or persons performing similar functions. The full text of our code of business conduct and ethics will be posted on our website at *www.sionnatx.com*. We intend to disclose on our website any future amendments of our code of business conduct and ethics or waivers that exempt any principal executive officer, principal financial officer, principal accounting officer or controller, persons performing similar functions or our directors from provisions in the code of business conduct and ethics. Information contained on, or accessible through, our website is not a part of this prospectus, and the inclusion of our website address in this prospectus is only an inactive textual reference.

Compensation Committee Interlocks and Insider Participation

None of the members of the compensation committee is currently, or has been at any time, one of our officers or employees. None of our officers currently serves, or has served during the last calendar year, as a member of the board of directors or compensation committee of any entity that has one or more executive officers serving as a member of our board of directors or compensation committee.

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Compensation Recovery

In connection with this offering, we adopted a compensation recovery policy that applies to our officers, which became effective as of the day immediately prior to the effectiveness of the registration statement of which this prospectus forms a part. Under the Sarbanes-Oxley Act, in the event of misconduct that results in a financial restatement that would have reduced a previously paid incentive amount, we can recoup those improper payments from our Chief Executive Officer and Chief Financial Officer. The SEC also recently adopted rules which direct national stock exchanges to require listed companies to implement policies intended to recoup bonuses paid to executives if the company is found to have misstated its financial results.

Limitations on Liability and Indemnification

As permitted by Delaware law, provisions in our fifth amended and restated certificate of incorporation, which will become effective immediately prior to the completion of this offering, and amended and restated bylaws, which became effective upon the effectiveness of this registration statement, limit or eliminate the personal liability of officers and directors for a breach of their fiduciary duty of care as a director. The duty of care generally requires that, when acting on behalf of the corporation, an officer or director exercise an informed business judgment based on all material information reasonably available to him or her. Consequently, an officer or director will not be personally liable to us or our stockholders for monetary damages or breach of fiduciary duty as an officer or director, except for liability for:

- any breach of the officer or director's duty of loyalty to us or our stockholders;
- any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- for our directors, unlawful payments of dividends or unlawful stock repurchases, or redemptions as provided in Section 174 of the Delaware General Corporation Law (the "DGCL");
- for our officers, any derivative action by or in the right of the corporation; or
- any transaction from which the officer or director derived an improper personal benefit.

These limitations of liability do not limit or eliminate our rights or any stockholder's rights to seek non-monetary relief, such as injunctive relief or rescission. These provisions will not alter an officer or director's liability under other laws, such as the federal securities laws or other state or federal laws. Our fifth amended and restated certificate of incorporation that will become effective immediately prior to the completion of this offering also authorizes us to indemnify our officers, directors and other agents to the fullest extent permitted under Delaware law.

As permitted by Delaware law, our amended and restated bylaws, which became effective upon the effectiveness of this registration statement will provide that:

- we will indemnify our directors, officers, employees and other agents to the fullest extent permitted by law;
- we must advance expenses to our directors and officers, and may advance expenses to our employees and other agents, in connection with a legal proceeding to the fullest extent permitted by law; and
- the rights provided in our amended and restated bylaws are not exclusive.

If Delaware law is amended to authorize corporate action further eliminating or limiting the personal liability of a director or officer, then the liability of our directors or officers will be so eliminated or limited to the fullest extent permitted by Delaware law, as so amended. Our amended and restated

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bylaws also permit us to secure insurance on behalf of any officer, director, employee or other agent for any liability arising out of his or her actions in connection with their services to us, regardless of whether our bylaws permit such indemnification. We have obtained such insurance.

In addition to the indemnification that will be provided for in our fifth amended and restated certificate of incorporation and amended and restated bylaws, we plan to enter into separate indemnification agreements with each of our directors and executive officers, which may be broader than the specific indemnification provisions contained in the DGCL. These indemnification agreements may require us, among other things, to indemnify our directors and executive officers for some expenses, including attorneys' fees, expenses, judgments, fines and settlement amounts incurred by a director or executive officer in any action or proceeding arising out of his service as one of our directors or executive officers or any other company or enterprise to which the person provides services at our request. We believe that these provisions and agreements are necessary to attract and retain qualified individuals to serve as directors and executive officers.

This description of the indemnification provisions of our fifth amended and restated certificate of incorporation, our amended and restated bylaws and our indemnification agreements is qualified in its entirety by reference to these documents, each of which is attached as an exhibit to the registration statement of which this prospectus forms a part.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to our directors, officers and controlling persons pursuant to the foregoing provisions, or otherwise, we have been advised that, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act, and is, therefore, unenforceable.

There is no pending litigation or proceeding naming any of our directors or officers as to which indemnification is being sought, nor are we aware of any pending or threatened litigation that may result in claims for indemnification by any director or officer.

Rule 10b5-1 Sales Plans

Subject to compliance with the terms of our Rule 10b5-1 trading plan policy, our directors and executive officers may adopt written plans, known as Rule 10b5-1 plans, in which they will contract with a broker to buy or sell shares of our common stock on a periodic basis. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the director or officer when entering into the plan, without further direction from them. The director or officer may amend a Rule 10b5-1 plan in some circumstances and may terminate a plan at any time. Our directors and executive officers also may buy or sell additional shares outside of a Rule 10b5-1 plan when they are not in possession of material nonpublic information subject to compliance with the terms of our insider trading policy. Prior to 180 days after the date of this offering, subject to early termination, the sale of any shares under such plans would be prohibited by the lock-up agreement that the director or officer has entered into with the underwriters.

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EXECUTIVE COMPENSATION

The following discussion contains forward-looking statements that are based on our current plans, considerations, expectations and determinations regarding future compensation programs. The actual amount and form of compensation and the compensation policies and practices that we adopt in the future may differ materially from currently planned programs as summarized in this discussion.

As an emerging growth company, we have opted to comply with the executive compensation disclosure rules applicable to "smaller reporting companies," as such term is defined in the rules promulgated under the Exchange Act. The compensation provided to our named executive officers ("NEOs") for the fiscal years ended December 31, 2024 and December 31, 2023 is detailed in the 2024 Summary Compensation Table and accompanying footnotes and narrative that follow. Our named executive officers for the fiscal year ended December 31, 2024, which consist of our Chief Executive Officer and the next two most highly compensated executive officers (other than our Chief Executive Officer) who were serving as our executive officers on December 31, 2024, are:

- Michael Cloonan, M.B.A., our President and Chief Executive Officer;
- Elena Ridloff, C.F.A., our Chief Financial Officer and Head of Corporate Development; and
- Charlotte McKee, M.D., our Chief Medical Officer.

Compensation Philosophy

Our executive compensation philosophy is to provide a competitive and market-based total compensation program to attract, motivate, and retain our executive team. Our compensation is based heavily on performance, which aligns with our goal to drive long-term growth and value creation.

2024 Summary Compensation Table

The following table sets forth information regarding compensation awarded to, earned by or paid to our NEOs for services rendered to us in all capacities during the fiscal years ended December 31, 2023 and December 31, 2024.

Name and principal position	Year	Salary ($)	Option Awards ($)(1)	Non-Equity Incentive Plan Compensation ($)(2)	Total ($)
Michael Cloonan, M.B.A.	2024	506,189	2,942,192	278,404	3,726,785
Chief Executive Officer	2023	486,720	—	267,696	754,416
Elena Ridloff, C.F.A.	2024	449,946	652,725	197,976	1,300,647
Chief Financial Officer and Head of Corporate Development	2023	432,640	—	190,362	623,002
Charlotte McKee, M.D.	2024	449,946	635,318	197,976	1,283,240
Chief Medical Officer	2023	432,640	—	190,362	623,002

(1) For fiscal year 2024, the amounts reported represent the aggregate grant date fair value of stock option awards granted to our NEOs during such fiscal year, computed in accordance with Financial Accounting Standards Board Accounting Standards Codification Topic 718 ("FASB ASC Topic 718"). A discussion of the assumptions used in determining grant date fair value may be found in Note 12 to our financial statements, included elsewhere in this prospectus. Such grant date fair values do not take into account any estimated forfeitures related to service-based vesting. The amounts reported in this column reflect the accounting cost for the stock option award and do not correspond to the actual economic value that may be received by our NEOs upon the vesting of the awards or any sale of the underlying securities.

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(2) Amounts reflect bonuses earned based on our achievement of specified performance metrics for the applicable fiscal year pursuant to our non-equity incentive plan.

Narrative Disclosure to Summary Compensation Table

2024 Salaries

Our NEOs each receive a base salary to compensate them for services rendered to us. The base salary payable to each NEO is intended to provide a fixed component of compensation reflecting the executive's skill set, experience, role and responsibilities. Base salaries are reviewed annually, typically in connection with our annual performance review process, and approved by our board of directors or our compensation committee, and may be adjusted from time to time to realign salaries with market levels after taking into account individual responsibilities, performance and experience.

Our board of directors, or our compensation committee, as applicable, generally reviews executive officer salaries in December of each year and any adjustment is typically implemented, and effective, on January 1 of the following year. For the fiscal year ended December 31, 2024, the annual base salaries for Mr. Cloonan, Ms. Ridloff and Dr. McKee were $506,189, $449,946 and $449,946, respectively.

In the event that the Company successfully consummates this offering on or prior to February 15, 2025, then the annual base salaries for Mr. Cloonan, Ms. Ridloff and Dr. McKee will increase to $665,000, $515,000 and $525,000, respectively, effective as of the consummation of such offering.

2024 Annual Bonuses

The Company maintains an annual cash incentive program designed to reward employees based on our achievement of certain corporate objectives. Our NEOs are eligible to receive annual cash incentive awards pursuant to this program, with a target annual bonus opportunity determined as a percentage of their annual base salary. Bonus payments are based upon the assessment of our performance measured against prospectively determined objectives, as determined by our board of directors or our compensation committee, as applicable.

For the fiscal year ended December 31, 2024, each NEO was eligible to earn an annual cash bonus based on the achievement of certain corporate performance metrics, and each performance metric was weighted based on a range of 5% to 50%, with the weighted percentages totaling 100%. The corporate performance goals for 2024 annual bonuses for our NEOs related to the achievement of certain preclinical and clinical milestones, the building of our organizational capabilities, and the progression of certain strategic initiatives. The fiscal year 2024 annual bonus targets for Mr. Cloonan, Ms. Ridloff and Dr. McKee were 50%, 40% and 40%, respectively, of their annual base salaries.

Our board of directors determined that, for fiscal year 2024, our corporate performance objectives were achieved at 110% of target, and based on such corporate performance achievement, Mr. Cloonan, Ms. Ridloff and Dr. McKee earned an annual bonus for fiscal year 2024 equal to $278,404, $197,976 and $197,976, respectively.

Equity-Based Compensation

Each of our NEOs holds outstanding equity awards under our 2020 Stock Option and Grant Plan (the "2020 Plan"). We believe that equity grants provide our executives with a strong link to our long- term performance, create an ownership culture, help to align the interests of our executives and our

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stockholders, and enhance executive retention. The market for qualified and talented executives in the biopharmaceutical industry is highly competitive, and we compete for talent with many companies that have greater financial resources than we do. Accordingly, we believe equity compensation is a crucial component of any competitive executive compensation package we offer. As such, our board of directors or our compensation committee periodically reviews the equity incentive compensation of our executives and may grant equity incentive awards to them from time to time.

In March 2024, following the sale and issuance of our Series C Preferred Stock, pursuant to the Series C Preferred Stock Purchase Agreement, dated as of March 4, 2024, our board of directors granted Mr. Cloonan, Ms. Ridloff and Dr. McKee an incentive stock option to purchase 656,984, 145,751 and 141,865 shares of our common stock, respectively. Such options vest in 48 equal monthly installments over a period of four years following March 4, 2024, subject to each applicable NEO's continued service with us through the applicable vesting dates; provided that the vesting of such shares will fully accelerate upon a "sale event" (as defined in the 2020 Plan and which does not include this offering) and as provided in the Severance and CIC Agreements and/or the New Severance and CIC Plan (each as defined below). Each NEO's outstanding awards as of December 31, 2024 are set forth in more detail in the "*Outstanding Equity Awards at Fiscal 2024 Year-End*" table below.

Perquisites or Personal Benefits

We did not provide perquisites or personal benefits to our NEOs during the fiscal year ended December 31, 2024.

401(k) Plan

We currently maintain a tax-qualified 401(k) retirement savings plan for our employees, including our NEOs, who satisfy certain eligibility requirements. Our NEOs are eligible to participate in the 401(k) plan on the same terms as other full-time employees. The 401(k) plan provides eligible employees with an opportunity to save for retirement on a tax advantaged basis. Eligible employees are able to defer eligible compensation subject to applicable annual limits of the Internal Revenue Code of 1986, as amended (the "Code"). Our 401(k) plan is intended to be qualified under Section 401(a) of the Code with our 401(k) plan's related trust intended to be tax exempt under Section 501(a) of the Code. As a tax-qualified retirement plan, contributions to our 401(k) plan and earnings and matching amounts on those contributions are not taxable to the employees until distributed from our 401(k) plan. We did not provide matching or discretionary contributions under the 401(k) plan during the fiscal year ended December 31, 2024. We believe that providing a vehicle for tax-deferred retirement savings though our 401(k) plan adds to the overall desirability of our executive compensation package and further incentivizes our employees, including our NEOs, in accordance with our compensation policies. Other than the 401(k) plan, we do not provide any qualified or non-qualified retirement or deferred compensation benefits to our employees, including our NEOs.

Executive Employment Arrangements

We have entered into an offer letter with each of our NEOs in connection with their employment with us, which sets forth the terms and conditions of each NEO's employment. Each of Mr. Cloonan, Ms. Ridloff and Dr. McKee has entered into a standard employee confidentiality, assignment and nonsolicitation agreement, pursuant to which each NEO has agreed to covenants relating to confidentiality and assignment of inventions, as well as covenants not to solicit certain of our service providers and customers during the NEO's employment and for one year after termination of employment. Dr. McKee has also agreed to a covenant not to compete during her employment and for one year after termination of employment.

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Offer Letters in Place for Our NEOs

Michael Cloonan, M.B.A.

In March 2021, we entered into an offer letter with Mr. Cloonan (the "Cloonan Offer Letter"). Under the Cloonan Offer Letter, Mr. Cloonan is entitled to receive an annual base salary (which has subsequently been increased as described above), an annual bonus opportunity, and an initial equity grant consisting of 547,343 shares of restricted stock, which vested as to 25% on the first anniversary following Mr. Cloonan's start date and vests as to the remaining 75% in 36 equal monthly installments thereafter, subject to Mr. Cloonan's continued service with us through each applicable vesting date; provided that, the vesting of such shares is subject to acceleration as set forth in his Severance and CIC Agreement (as defined below) and/or the New Severance and CIC Plan (as defined below), as applicable. The restricted stock grant is further detailed in the "*Outstanding Equity Awards at Fiscal 2024 Year-End*" section below. Mr. Cloonan is also eligible to participate in our employee benefit plans, subject to the terms of such plans, and is entitled to severance benefits pursuant to his Severance and CIC Agreement (as defined below) and/or the New Severance and CIC Plan (as defined below), as applicable.

In addition to Mr. Cloonan's severance entitlements under his Severance and CIC Agreement and/or the New Severance and CIC Plan, as applicable, each as described below, under the Cloonan Offer Letter, in the event Mr. Cloonan's employment ceases due to his death or disability (as defined in his Severance and CIC Agreement), he will be entitled to any earned but unpaid bonus for the year ending prior to the date of such cessation and the prorated portion of his annual target bonus for the year in which such cessation takes place.

Elena Ridloff, C.F.A.

In August 2021, we entered into an offer letter with Ms. Ridloff (the "Ridloff Offer Letter"). Under the Ridloff Offer Letter, Ms. Ridloff is entitled to receive an annual base salary (which has subsequently been increased as described above), a signing bonus (which was paid in 2021), an annual bonus opportunity, and an initial equity grant consisting of an option to purchase 156,046 shares of our common stock, which vested as to 25% on the first anniversary following Ms. Ridloff's start date and vests as to the remaining 75% in 36 equal monthly installments thereafter, subject to Ms. Ridloff's continued service with us through each applicable vesting date; provided that, the vesting of such shares will fully accelerate upon a "sale event" (as defined in the 2020 Plan and which does not include this offering). The stock option grant is further detailed in the "*Outstanding Equity Awards at Fiscal 2024 Year-End*" section below. Ms. Ridloff is also eligible to participate in our employee benefit plans, subject to the terms of such plans, and is entitled to severance benefits pursuant to her Severance and CIC Agreement (as defined below) and/or the New Severance and CIC Plan (as defined below), as applicable.

In addition to Ms. Ridloff's severance entitlements under her Severance and CIC Agreement and/or the New Severance and CIC Plan, as applicable, each as described below, under the Ridloff Offer Letter, in the event Ms. Ridloff's employment ceases due to her death or disability (as defined in her Severance and CIC Agreement), she will be entitled to any earned but unpaid bonus for the year ending prior to the date of such cessation and the prorated portion of her annual target bonus for the year in which such cessation takes place.

Charlotte McKee, M.D.

In May 2021, we entered into an offer letter with Dr. McKee (the "McKee Offer Letter"). Under the McKee Offer Letter, Dr. McKee is entitled to receive an annual base salary and an annual bonus opportunity (each of which has subsequently been increased as described above), and an initial equity grant consisting of 151,548 shares of restricted stock, which vested as to 25% on the first anniversary

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following Dr. McKee's start date and vests as to the remaining 75% in 36 equal monthly installments thereafter, subject to Dr. McKee's continued service with us through each applicable vesting date; provided that, the vesting of such shares will fully accelerate upon a "sale event" (as defined in the 2020 Plan and which does not include this offering). The restricted stock grant is subject to certain tag along rights upon a sale event. The restricted stock grant is further detailed in the "*Outstanding Equity Awards at Fiscal 2024 Year-End*" section below. Dr. McKee is also eligible to participate in our employee benefit plans, subject to the terms of such plans, and is entitled to severance benefits pursuant to her Severance and CIC Agreement (as defined below) and/or the New Severance and CIC Plan (as defined below), as applicable.

In addition to Dr. McKee's severance entitlements under her Severance and CIC Agreement and/or the New Severance and CIC Plan, as applicable, each as described below, under the McKee Offer Letter, in the event Dr. McKee's employment ceases due to her death or disability (as defined in her Severance and CIC Agreement), she will be entitled to any earned but unpaid bonus for the year ending prior to the date of such cessation and the prorated portion of her annual target bonus for the year in which such cessation takes place.

Severance and Change in Control Arrangements

Each of the NEOs has entered into a Severance and Change in Control Agreement (each, a "Severance and CIC Agreement"). In addition, we have adopted a Severance and Change in Control Plan (the "New Severance and CIC Plan"), subject to the effectiveness of the registration statement of which this prospectus forms a part (the date on which such registration statement becomes effective, the "New Severance and CIC Plan Effective Date").

Severance and Change in Control Agreements

Each of the NEOs has entered into a Severance and CIC Agreement, which provides that upon a "terminating event" (i.e., a termination by us for any reason other than due to "cause," death or "disability," or a resignation by an NEO for "good reason", as such terms are defined in the Severance and CIC Agreements) outside of the "change in control period" (i.e., the period on or within 12 months after the date of a "change in control", as such term is defined in the Severance and CIC Agreements and which does not include this offering), the NEO will be entitled to receive, subject to the execution and delivery of an effective and irrevocable release of claims in favor of us and continued compliance with all applicable restrictive covenants, (i) 12 months of base salary; (ii) earned but unpaid bonus for the year prior to the year of termination; (iii) a prorated annual target bonus for the year of termination; (iv) subject to the NEO's timely COBRA election, the share of the NEO's health premium payments in an amount equal to what we pay for active employees until the earlier of (A) the one-year anniversary of the date of termination and (B) the date that the NEO obtains coverage under another health and dental insurance plan; and (v) with respect to any equity grants held by the NEO, (A) 12 months' acceleration of vesting of any time-based stock option and stock awards; provided, that such options and awards were held by the NEO prior to the New Severance and CIC Plan Effective Date, (B) for any performance-based stock options and stock awards, treatment as set forth in the applicable award agreement and (C) 12 months' extension of any post- termination exercise period for options held by the NEO prior to the New Severance and CIC Plan Effective Date. The amounts payable under (i) through (iii) shall be paid out in substantially equal installments in accordance with our payroll practice over 12 months commencing within 60 days after the date of termination.

Upon a terminating event within the change in control period, the Severance and CIC Agreements provide that the NEO will be entitled to receive, in lieu of above, subject to the execution and delivery of an effective and irrevocable release of claims in favor of us and continued compliance with all applicable restrictive covenants, (i) 100% of the NEO's annual base salary; (ii) earned but

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unpaid bonus for the year prior to the year of termination; (iii) 100% of the NEO's annual target bonus for the year of termination; (iv) subject to the NEO's timely COBRA election, full health premium payments until the earlier of (A) the 18-month anniversary of the date of termination and (B) the date that the NEO obtains coverage under another health and dental insurance plan; and (v) with respect to any equity grants held by the NEO, (A) full acceleration of vesting of any time-based stock option and stock awards; provided, that such options and awards were held by the NEO prior to the New Severance and CIC Plan Effective Date, (B) for any performance-based stock options and stock awards, treatment as set forth in the applicable award agreement, and (C) 12 months' extension of any post-termination exercise period for options held by the NEO prior to the New Severance and CIC Plan Effective Date. The amounts payable under (i) through (iii) shall be paid out in a lump sum within 60 days after the date of termination. As of the New Severance and CIC Plan Effective Date, prongs (i) and (iii) for Mr. Cloonan will be superseded by the New Severance and CIC Plan.

Pursuant to each of the Severance and CIC Agreements, any payments and benefits provided to an NEO in connection with a change in control that would otherwise not be eligible for a federal income tax deduction for us pursuant to Section 280G of the Code and subject the NEO to an excise tax under Section 4999 of the Code shall be reduced, but only if such reduction would result in the NEO retaining a larger portion of such payments and benefits on an after-tax basis than if no reduction was made and the excises taxes had been paid (also known as a 280G modified cutback).

Severance and Change in Control Plan

On December 12, 2024, our board of directors adopted the New Severance and CIC Plan, subject to the effectiveness of the registration statement of which this prospectus forms a part. Employees with the job title of vice president and above at the time of termination (or, if applicable, "change in control" (as defined in the New Severance and CIC Plan and which does not include this offering)) and who have executed a participation agreement (each, an "Eligible Employee"), will be eligible to participate in the New Severance and CIC Plan.

The New Severance and CIC Plan will provide that, at any time outside of the period commencing upon a change in control (as defined in the New Severance and CIC Plan) and ending 12 months after such change in control (the "change in control period"), upon a (i) termination by us for any reason other than due to "cause," death or "disability" (as such terms are defined in the New Severance and CIC Plan) or (ii) for Eligible Employees with a C-level position and who reports directly to our chief executive officer and/or has a job title of Executive Vice President or above (each, an "Executive"), resignation for "good reason" (as defined in the New Severance and CIC Plan), an Eligible Employee or Executive will be entitled to receive, subject to the execution and delivery of an effective and irrevocable release of claims in favor of the Company and continued compliance with all applicable restrictive covenants, (A) (x) 12 months of continued base salary for our chief executive officer and (y) nine months of continued base salary for each of our Executives (which includes the NEOs other than the chief executive officer) and for each of our senior vice presidents, (B) a prorated "target bonus" (i.e., the higher of the target annual performance bonus for the year in which the termination occurs or the target annual performance bonus in effect as of immediately prior to a change in control, as applicable) for each of our Executives (which includes the NEOs) and senior vice presidents and (C) if the Eligible Employee elects to continue health and dental insurance coverage following such termination, an amount equal to the monthly employer contribution, based on the premiums as of the date of termination, that we would have made to provide health insurance for the applicable executive if he or she had remained employed by us for up to (x) 12 months for our chief executive officer and (y) nine months for each of our Executives (which includes the NEOs other than the chief executive officer) and for each of our senior vice presidents. The payments under (A) and (C), as applicable, will be paid in substantially equal installments in accordance with our payroll practice over 12 months for our chief executive officer and nine months for each of our Executives (which includes the NEOs other

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than the chief executive officer) and each of our senior vice presidents, and the payments under (B) are payable upon the first installment of such payments in accordance with our payroll practice.

The New Severance and CIC Plan will also provide that, at any time during the change in control period, upon a (i) termination by us for any reason other than due to cause, death or disability or (ii) for Executives, resignation for good reason, an Eligible Employee will be entitled to receive, in lieu of the payments and benefits above and subject to the execution and delivery of an effective and irrevocable release of claims in favor of the Company and continued compliance with all applicable restrictive covenants, (A) a lump sum cash amount equal to (x) 1.5x the sum of the base salary and the target bonus for our chief executive officer, (y) 1x the sum of the base salary and the target bonus for each of our Executives (which includes the NEOs other than the chief executive officer) and for each of our senior vice presidents and (z) 0.75x the sum of the base salary and the target bonus for our each of our vice presidents, (B) if the Eligible Employee elects to continue health and dental insurance coverage following such termination, an amount equal to the monthly employer contribution, based on the premiums as of the date of termination, that we would have made to provide health insurance for the applicable executive if he or she had remained employed by us for (x) 18 months for our chief executive officer, (y) 12 months for each of our Executives (which includes the NEOs other than the chief executive officer) and for each of our senior vice presidents and (z) nine months for each of our vice presidents, and (C) for all outstanding and unvested equity awards of the Company that are subject to time-based vesting held by the eligible participant, full accelerated vesting of such awards; provided, that any equity awards with performance-based vesting will be treated as specified in the applicable award agreement.

The payments and benefits provided under the New Severance and CIC Plan in connection with a change in control may not be eligible for a federal income tax deduction by us pursuant to Section 280G of the Code. These payments and benefits may also subject an eligible participant, including the NEOs, to an excise tax under Section 4999 of the Code. If the payments or benefits payable in connection with a change in control would be subject to the excise tax imposed under Section 4999 of the Code, then those payments or benefits will be reduced if such reduction would result in a higher net after-tax benefit to the participant.

The New Severance and CIC Plan will fully supersede and replace all previous severance and change in control plans, policies, arrangements, and agreements, except that if an Eligible Employee is party to a fully-executed offer letter, employment agreement, severance or change in control agreement with the Company (each, an “Existing Employment Agreement”), e.g., the Severance and CIC Agreements described above, that, as of the New Severance and CIC Plan Effective Date, provides for more favorable terms or provisions than provided under such New Severance and CIC Plan, then the more favorable definition, term or provision, or relevant combination thereof, will be applicable for the benefit of the eligible employee; provided, however, that there is no duplication of benefits and (i) a more favorable definition of “cause” under an employee non-competition agreement shall not apply for purposes of “cause” as used in New Severance and CIC Plan and (ii) the following provisions under any Existing Employment Agreement will continue to apply to any equity awards granted before the New Severance and CIC Plan Effective Date, but will not apply to any equity awards granted after the New Severance and CIC Plan Effective Date: (A) extending the time for exercising any stock-based awards beyond 3 months following a termination of service (other than due to death or disability), (B) providing for acceleration of vesting of unvested equity award(s) upon a termination of employment prior to a change in control or other sales transaction of the Company; and/or (C) providing for acceleration of vesting of unvested equity award(s) upon a change in control or other sales transaction of the Company.

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Compensation Recovery Policy

In accordance with the requirements of the Dodd-Frank Act, final SEC rules, and applicable Nasdaq listing standards, on December 12, 2024, our board of directors adopted a compensation recovery policy in connection with this offering, which became effective upon the date on which the registration statement of which this prospectus forms a part was declared effective by the SEC. The compensation recovery policy will provide that in the event we are required to prepare a restatement of financial statements due to material noncompliance with any financial reporting requirement under securities laws, we will seek to recover any incentive-based compensation that was based upon the attainment of a financial reporting measure and that was received by any current or former executive officer during the three-year period preceding the date that the restatement was required if such compensation exceeds the amount that the executive officer would have received had the financial results been properly reported.

Outstanding Equity Awards at Fiscal 2024 Year-End

The following table sets forth information concerning outstanding equity awards held by our NEOs as of December 31, 2024.

			Option awards(1)				Stock awards(1)	
Name	Grant Date	Vesting Commencement Date	Number of securities underlying unexercised options (#) exercisable	Number of securities underlying unexercised options (#) unexercisable	Option exercise price ($)	Option expiration date	Number of shares or units of stock that have not vested (#)	Market value of shares or units of stock that have not vested ($)(2)
Michael Cloonan, M.B.A.	3/31/2021	5/10/2021	—	—	—	—	57,015(3)	588,965
	3/2/2022	2/2/2022	235,491	96,967(4)	6.11	3/1/2032	—	—
	3/13/2024	3/4/2024	123,184	533,799(4)	6.11	3/12/2034	—	—
Elena Ridloff, C.F.A.	9/17/2021	9/14/2021	3,250	29,258(5)	0.78	9/16/2031	—	—
	3/2/2022	2/2/2022	77,637	31,968(4)	6.11	3/1/2032	—	—
	3/13/2024	3/4/2024	27,328	118,423(4)	6.11	3/12/2034	—	—
Charlotte McKee, M.D.	7/8/2021	6/1/2021	—	—	—	—	18,943(5)	195,681
	3/2/2022	2/2/2022	75,805	31,214(4)	6.11	3/1/2032	—	—
	3/13/2024	3/4/2024	26,599	115,265(4)	6.11	3/12/2034	—	—

(1) Each equity award is subject to the terms of our 2020 Plan, described below.

(2) This amount is based on the fair market value of a share of our common stock equal to $10.33 as of December 31, 2024, as determined by our board of directors.

(3) The shares underlying this restricted stock award vest over a four-year period as follows: 25% of such shares vested on the first anniversary of the vesting commencement date, and the remaining 75% of the shares vest in 36 equal monthly installments thereafter, in each case, subject to the NEO's continuous "service relationship" (as defined in the 2020 Plan) with us through each applicable vesting date. Notwithstanding the foregoing, this award is subject to acceleration of vesting benefits as set forth in the NEO's Severance and CIC Agreement and/or the New Severance and CIC Plan, as applicable.

(4) The shares underlying this stock option award vest in 48 equal monthly installments over a four-year period following the vesting commencement date, in each case, subject to the NEO's continuous "service relationship" (as defined in the 2020 Plan) with us through each applicable vesting date. Under the applicable award agreement, the shares underlying this option will become fully vested upon a sale event (as defined in the 2020 Plan), which does not include this offering. Notwithstanding the foregoing, this award is also subject to acceleration of vesting benefits as set forth in the NEO's Severance and CIC Agreement and/or the New Severance and CIC Plan, as applicable.

(5) The shares underlying this award vest over a four-year period as follows: 25% of such shares vested on the first anniversary of the vesting commencement date, and the remaining 75% of the shares vest in 36 equal monthly installments thereafter, in each case, subject to the NEO's continuous "service relationship" (as defined in the 2020 Plan) with us through each applicable vesting date. Under the applicable award agreement, the shares underlying this award will become fully vested

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upon a sale event (as defined in the 2020 Plan) of our company, which does not include this offering. Notwithstanding the foregoing, this award is also subject to acceleration of vesting benefits as set forth in the NEO's offer letter, the NEO's Severance and CIC Agreement and/or the New Severance and CIC Plan, as applicable. For Dr. McKee's restricted stock award only, such award is also subject to certain tag along rights upon a sale event.

Employee Benefit and Equity Compensation Plans

2020 Stock Option and Grant Plan

Our 2020 Plan was initially adopted by our board of directors, and approved by our stockholders, on August 20, 2020 and has been subsequently amended thereafter. Our 2020 Plan allows for the grant of incentive stock options to our employees and any of our subsidiary corporations' employees, and for the grant of nonqualified stock options, restricted shares of common stock, unrestricted shares of common stock, and restricted stock units awards to our employees, officers, directors and consultants of ours and our subsidiary corporations. Following this offering, we will not grant any further awards under our 2020 Plan, but all outstanding awards under the 2020 Plan will continue to be governed by their existing terms. In connection with this offering, we intend to adopt a new incentive equity plan under which we will grant equity-based awards following this offering, as described below under "*2025 Stock Option and Incentive Plan*." This summary is not a complete description of all provisions of the 2020 Plan and is qualified in its entirety by reference to the 2020 Plan, which will be filed as an exhibit to the registration statement of which this prospectus forms a part.

Under the 2020 Plan, we reserved for issuance an aggregate of 6,067,288 shares of our common stock. The number of shares of common stock reserved for issuance is subject to adjustment in the event of a reorganization, reclassification, stock dividend, stock split, reverse stock split, recapitalization or other similar change in our capital stock that constitutes an equity restructuring. No more than 6,067,288 shares may be issued pursuant to incentive stock options.

The 2020 Plan is administered by our board of directors or a committee appointed by it. The plan administrator has full power to, among other things, select, from among the individuals eligible for awards, the individuals to whom awards will be granted, to accelerate the time at which a stock award may be exercised or vest, to amend the 2020 Plan and to determine the specific terms and conditions of each award, subject to the provisions of the 2020 Plan. The plan administrator may exercise its discretion to reduce the exercise price of outstanding options under the 2020 Plan or effect repricing through cancellation of such outstanding options and by granting such holders new awards in replacement of the cancelled options.

The 2020 Plan permits the grant of stock options. The exercise price per share of all options must equal at least 100% of the fair market value per share of our common stock on the date of grant. The term of a stock option may not exceed ten years. An incentive stock option granted to a participant who owns more than 10% of the total combined voting power of all classes of our stock on the date of grant, or any subsidiary corporations, may not have a term in excess of five years and must have an exercise price of at least 110% of the fair market value per share of our common stock on the date of grant. The plan administrator will determine the methods of payment of the exercise price of an option, which may include cash, shares or certain other property or other consideration acceptable to the plan administrator. After a participant's termination of service, the participant generally may exercise his or her options, to the extent vested as of such date of termination, for 90 days after termination. If termination is due to death or disability, the option generally will remain exercisable, to the extent vested as of such date of termination, for twelve months after such termination. However, in no event may an option be exercised later than the expiration of its term. If termination is for cause, then an option automatically expires upon the date of the optionee's termination.

The 2020 Plan permits the grant of restricted shares of common stock. Restricted stock awards are grants of shares of our common stock that are subject to various restrictions, including restrictions

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on transferability and forfeitures provisions. Shares of restricted stock will vest, and the restrictions on such shares will lapse, in accordance with terms and conditions established by the plan administrator.

The 2020 Plan permits the grant of unrestricted shares of common stock. Unrestricted stock awards may have been granted to participants in recognition of past services or for other valid consideration and may be issued in lieu of cash compensation due to such participant.

The 2020 Plan permits the grant of restricted stock units. A restricted stock unit is an unfunded and unsecured promise, denominated in shares, to deliver shares or cash measured by the value of shares in the future, subject to the satisfaction of specified vesting requirements. After settlement, the plan administrator issues the underlying shares or the cash equivalent of the number of shares, or a combination of both. The plan administrator determines the terms and conditions of restricted stock units, including the number of units granted, the vesting criteria (which may include accomplishing specified performance criteria or continued service to us) and the form and timing of payment.

Our 2020 Plan generally does not allow for the transfer or assignment of awards, other than, at the discretion of the plan administrator with respect to non-qualified stock options, by gift to an immediate family member, to trusts for the benefit of family members, or to partnerships in which such family members are the only partners, and only the recipient of an award may exercise such an award during his or her lifetime. Our 2020 Plan also provides for drag along rights pursuant to which participants may be obligated to, on the request of the majority of our shareholders, sell, transfer, and deliver, or cause to be sold, transferred, and delivered, to a buyer, his or her shares in the event the majority of our shareholders determine to enter into a “sale event,” as defined in the 2020 Plan and which does not include this offering.

In the event of certain changes in our capitalization, the exercise prices of and the number of shares subject to outstanding options, and the purchase price of and the numbers of shares subject to outstanding awards will be proportionately adjusted.

The 2020 Plan provides that upon the effectiveness of a sale event, an acquirer or successor entity may assume, continue or substitute for the outstanding awards under the 2020 Plan. To the extent that awards granted under the 2020 Plan are not assumed or continued or substituted by the successor entity, all options and all other awards granted under the 2020 Plan shall terminate. In the event of such termination, individuals holding options will be permitted to exercise such options (to the extent exercisable) prior to the sale event. In addition, in connection with the termination of the 2020 Plan upon a sale event, we may make or provide for a cash payment equal to (A) in the case of vested and exercisable options (including by reason of acceleration in connection with the sale event), the difference between (1) the per share cash consideration payable to stockholders (as determined by the plan administrator) in the sale event times the number of shares subject to the options being cancelled and (2) the aggregate exercise price of the options and (B) in the case of restricted stock and restricted stock unit awards, the per share cash consideration payable to stockholders in the sale event multiplied by the number of shares of stock subject to such stock awards (payable at the time of the sale event or upon the later vesting of the awards). In the event of the forfeiture of shares of restricted stock issued under our 2020 Plan, such shares of restricted stock shall be repurchased from the holder at a price per share equal to the original per share purchase price paid by the recipient of such shares. Additionally, the board of directors may resolve, in its sole discretion, to subject any assumed options or payments in respect of options to any escrow, holdback, indemnification, earn-out or similar provisions in the transaction agreements as such provisions apply to holders of our common stock. As of December 31, 2024, options to purchase up to 3,700,335 shares of common stock at a weighted average exercise price of $5.87 per share and 75,958 shares of restricted stock were outstanding under the 2020 Plan.

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2025 Stock Option and Incentive Plan

Our 2025 Stock Option and Incentive Plan (the "2025 Plan") was adopted by our board of directors on December 12, 2024, adopted by our stockholders on January 31, 2025, and became effective upon the date immediately preceding the date on which the registration statement of which this prospectus forms a part was declared effective by the SEC. The 2025 Plan replaces the 2020 Plan, as our board of directors has determined not to make additional awards under the 2020 Plan following the closing of this offering. However, the 2020 Plan will continue to govern outstanding equity awards granted thereunder. The 2025 Plan allows us to make equity-based and cash-based incentive awards to our employees, non-employee directors and consultants. The following summary describes the material terms of the 2025 Plan. This summary is not a complete description of all provisions of the 2025 Plan and is qualified in its entirety by reference to the 2025 Plan, which is filed as an exhibit to the registration statement of which this prospectus forms a part.

We have initially reserved 5,060,000 shares of our common stock for the issuance of awards under the 2025 Plan (the "Initial Limit"). The 2025 Plan provides that the number of shares reserved and available for issuance under the 2025 Plan will automatically be cumulatively increased on January 1, 2026 and each January 1 thereafter through January 1, 2035, by 4% of the sum of (x) the number of shares of our common stock outstanding and (y) the number of shares of our common stock issuable pursuant to the exercise of any outstanding, pre-funded warrants to acquire common stock for a nominal exercise price ((x) and (y) together, the "Outstanding Shares") on the immediately preceding December 31 or such lesser number of shares as determined by our administrator (the "Annual Increase"). The number of shares reserved under the 2025 Plan is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

The shares we issue under the 2025 Plan will be authorized but unissued shares or shares that we reacquire. The shares of common stock underlying any awards under the 2025 Plan and the 2020 Plan that are forfeited, cancelled, held back upon exercise or settlement of an award to satisfy the exercise price or tax withholding, reacquired by us prior to vesting, satisfied without the issuance of stock or are otherwise terminated (other than by exercise) will be added back to the shares of common stock available for issuance under the 2025 Plan.

The maximum number of shares of common stock that may be issued in the form of incentive stock options shall not exceed the Initial Limit, cumulatively increased on January 1, 2026 and on each January 1 thereafter by the lesser of the Annual Increase for such year or 5,060,000 shares of common stock.

The grant date fair value of all awards made under our 2025 Plan and all other cash compensation paid by us to any non-employee director in any calendar year for services as a non-employee director shall not exceed $750,000; provided, however, that such amount shall be $1,000,000 for the calendar year in which the applicable non-employee director is initially elected or appointed to the board of directors.

The 2025 Plan will be administered by our compensation committee. Our administrator has the full power to select, from among the individuals eligible for awards, the individuals to whom awards will be granted and the number of shares subject to such awards, to make any combination of awards to participants, to accelerate at any time the exercisability or vesting of any award and to determine the specific terms and conditions of each award, subject to the provisions of the 2025 Plan. Persons eligible to participate in the 2025 Plan will be those employees, non-employee directors and consultants as selected from time to time by our administrator in its discretion.

The 2025 Plan permits the granting of both options to purchase common stock intended to qualify as incentive stock options under Section 422 of the Code and options that do not so qualify. The option

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exercise price of each option will be determined by our administrator but may not be less than 100% of the closing price of our common stock on the date of grant (or, if no closing price is reported on that date, the closing price on the immediately preceding date on which a closing price was reported) (110% in the case of certain incentive stock options) unless the option (i) is granted pursuant to a transaction described in, and in a manner consistent with Section 424(a) of the Code, (ii) is granted to an individual who is not subject to U.S. income tax or (iii) if the grant is otherwise exempt or compliant with Section 409A of the Code. The term of each option will be fixed by our administrator and may not exceed 10 years from the date of grant (or five years in the case of certain incentive stock options). Our administrator will determine at what time or times each option may be exercised.

Our administrator may award stock appreciation rights under the 2025 Plan subject to such conditions and restrictions as it may determine. Stock appreciation rights entitle the recipient to shares of common stock, or cash, equal to the value of the appreciation in our stock price over the exercise price. The exercise price of each stock appreciation right generally may not be less than 100% of the closing price of our common stock on the date of grant (or, if no closing price is reported on that date, the closing price on the immediately preceding date on which a closing price was reported) unless the share appreciation right (i) is granted pursuant to a transaction described in, and in a manner consistent with Section 424(a) of the Code, (ii) is granted to an individual who is not subject to U.S. income tax or (iii) if the grant is otherwise exempt or compliant with Section 409A of the Code. The term of each stock appreciation right will be fixed by our administrator and may not exceed ten years from the date of grant. Our administrator will determine at what time or times each stock appreciation right may be exercised.

Our administrator may award restricted shares of common stock and restricted stock units to participants subject to such conditions and restrictions as our administrator may determine. These conditions and restrictions may include the achievement of certain performance goals and/or continued employment with us through a specified vesting period. Our administrator may also grant shares of common stock that are free from any restrictions under the 2025 Plan. Unrestricted stock may be granted to participants in recognition of past services or for other valid consideration and may be issued in lieu of cash compensation due to such participant.

Our administrator may grant dividend equivalent rights to participants that entitle the recipient to receive credits for dividends that would be paid if the recipient had held a specified number of shares of common stock.

Our administrator may grant cash-based awards under the 2025 Plan to participants, subject to the achievement of certain performance goals, including continued employment (or other Service Relationship (as defined in the 2025 Plan)).

The 2025 Plan provides that in the case of and subject to the consummation of a “sale event,” as defined in the 2025 Plan, an acquirer or successor entity may assume, continue or substitute outstanding awards under the 2025 Plan. To the extent that awards granted under the 2025 Plan are not assumed or continued or substituted by the successor entity, upon the effective time of the sale event, such awards shall terminate. In such case, except as may be otherwise provided in the relevant award agreement, all awards with time-based vesting conditions or restrictions shall become fully vested and nonforfeitable as of the effective time of the sale event and all awards with conditions and restrictions relating to the attainment of performance goals may become vested and nonforfeitable in connection with a sale event in the administrator’s discretion or to the extent specified in the relevant award agreement. In the event of such termination, (i) individuals holding options and stock appreciation rights will be permitted to exercise such options and stock appreciation rights (to the extent then exercisable (after taking into account any acceleration thereunder)) within a specified period of time prior to the sale event or (ii) we may make or provide for a payment, in cash or in kind, to participants holding vested and exercisable options and stock appreciation rights equal to the difference between the per share consideration payable to stockholders in the sale event and the

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exercise price of the options or stock appreciation rights and we may make or provide for a payment, in cash or in kind, to participants holding other vested awards in an amount equal to the per share consideration payable to stockholders in the sale event multiplied by the number of vested shares under such award.

Our board of directors may amend or discontinue the 2025 Plan and our administrator may amend or cancel outstanding awards for purposes of satisfying changes in law or any other lawful purpose, but no such action may materially and adversely affect rights under an award without the holder's consent. Certain amendments to the 2025 Plan require the approval of our stockholders. The administrator of the 2025 Plan is specifically authorized to exercise its discretion to reduce the exercise price of outstanding stock options and stock appreciation rights or effect the repricing of such awards through cancellation and re-grants or cancellation in exchange for cash or other awards, in each case, without stockholder approval. No awards may be granted under the 2025 Plan after the date that is 10 years from the effective date of the 2025 Plan. No awards under the 2025 Plan have been made prior to the date of this prospectus.

2025 Employee Stock Purchase Plan

Our 2025 Employee Stock Purchase Plan (the "ESPP") was approved by our board of directors on December 12, 2024, adopted by our stockholders on January 31, 2025, and became effective on the date immediately preceding the date on which the registration statement of which this prospectus forms a part was declared effective by the SEC. The ESPP has two components: a component intended to qualify as an "employee stock purchase plan" within the meaning of Section 423 of the Code (the "423 Component"), and a component that is not intended to qualify (the "Non-423 Component"). Except as otherwise provided, the Non-423 Component will be operated and administered in the same manner as the 423 Component, except where prohibited by law or as provided by the administrator. The following summary describes the material terms of the ESPP. This summary is not a complete description of all provisions of the ESPP and is qualified in its entirety by reference to the ESPP, which will be filed as an exhibit to the registration statement of which this prospectus forms a part.

The ESPP initially reserves and authorizes the issuance of up to a total of 390,127 shares of our common stock to participating employees. The ESPP provides that the number of shares reserved and available for issuance will automatically be cumulatively increased on January 1, 2026 and each January 1 thereafter through January 1, 2035, by the least of (i) 780,254 shares of common stock, (ii) 1% of the Outstanding Shares on the immediately preceding December 31 or (iii) such number of shares of common stock as determined by the administrator of the ESPP. The number of shares reserved under the ESPP is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

All employees who are customarily employed by us or one of our designated subsidiaries for more than 20 hours per week and who have been employed for at least 30 days in advance of an offering are eligible to participate in the ESPP; provided, however, that employees who are customarily employed for 20 hours or less per week may be eligible to participate in the ESPP if required by applicable law or regulations. However, any employee who owns 5% or more of the total combined voting power or value of all classes of our stock will not be eligible to purchase shares of common stock under the ESPP.

We may make one or more offerings each year to our employees to purchase shares under the ESPP. Offerings will begin and end on the dates determined by the administrator, referred to as offering periods; provided that, no offerings shall exceed 27 months in duration. Each eligible employee may elect to participate in any offering by submitting an enrollment form to the Company in a manner determined by the administrator by such deadline as established by the administrator. Unless the administrator chooses otherwise prior to an offering date, and to the extent an offering has more than

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one purchase period and to the extent permitted by applicable law, if the fair market value of our common stock on any exercise date in an offering is lower than the fair market value of our common stock on the first day of the applicable offering, then all participants in such offering automatically will be withdrawn from such offering immediately after the exercise of their option on such exercise date and automatically re-enrolled in the immediately following offering as of the first day thereof and the preceding offering will terminate.

Each employee who is a participant in the ESPP may purchase shares of our common stock by authorizing payroll deductions or contributions of up to 15% of his or her eligible compensation during an offering period or such other maximum as may be specified by the administrator in advance of an offering. Unless the participating employee has previously withdrawn from the offering, his or her accumulated payroll deductions or contributions will be used to purchase shares of our common stock on the last business day of the purchase period at a price equal to 85% of the closing price of our common stock on the first day of the offering or the last day of the purchase period (or, if no closing price is reported on that date, the closing price on the immediately preceding date on which a closing price was reported), whichever is lower, provided that no more than a number of shares of common stock determined by dividing $25,000 by the closing price of our common stock on the first day of such offering (or such other lesser maximum number of shares as may be established by the administrator) may be purchased by any one employee during any purchase period. Under applicable tax rules, an employee may purchase no more than $25,000 worth of shares of our common stock, valued at the start of the offering period, under the ESPP for each calendar year during which any option granted to the employee is outstanding at any time.

In the case of and subject to the consummation of a “sale event,” as defined in the ESPP, the administrator of the ESPP, in its discretion, and on such terms and conditions as it deems appropriate, is authorized to take any one or more of the following actions under the ESPP or with respect to any right under the ESPP or to facilitate such transactions or events: (i) provide for either (a) termination of any outstanding option in exchange for an amount of cash, if any, equal to the amount that would have been obtained upon the exercise of such option had such option been currently exercisable or (b) the replacement of such outstanding option with other options or property selected by the administrator of the ESPP in its sole discretion; (ii) provide that the outstanding options under the ESPP shall be assumed by the successor or survivor corporation, or a parent or subsidiary thereof, or shall be substituted for similar options covering the stock of the successor or survivor corporation, or a parent or subsidiary thereof, with appropriate adjustments as to the number and kind of shares and prices; (iii) make adjustments in the number and type of shares of common stock (or other securities or property) subject to outstanding options under the ESPP and/or in terms and conditions of outstanding options and options that may be granted in the future; (iv) provide that the offering with respect to which an option relates will be shortened by setting a new exercise date on which such offering period will end; and (v) provide that all outstanding options shall terminate without being exercised and all amounts in the accounts of participants shall be promptly refunded.

The accumulated payroll deductions or contributions of any employee who is not a participant on the last day of an offering period will be refunded. An employee’s rights under the ESPP terminate upon voluntary withdrawal from the plan or when the employee ceases employment with us for any reason.

The ESPP may be terminated or amended by our board of directors at any time. An amendment that increases the number of shares of our common stock authorized under the ESPP and certain other amendments require the approval of our stockholders.

Senior Executive Cash Incentive Bonus Plan

On December 12, 2024, our board of directors adopted the Senior Executive Cash Incentive Bonus Plan (the “Bonus Plan”), which became effective on the date immediately preceding the date on which the registration statement of which this prospectus forms a part was declared effective by the SEC. The Bonus Plan provides for cash bonus payments based upon company and individual

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performance targets established by our compensation committee. The payment targets will be related to financial and operational measures or objectives with respect to our company, or the corporate performance goals, as well as individual performance objectives. The following summary describes the material terms of the Bonus Plan. This summary is not a complete description of all provisions of the Bonus Plan and is qualified in its entirety by reference to the Bonus Plan, which will be filed as an exhibit to the registration statement of which this prospectus forms a part.

Our compensation committee may select corporate performance goals from among the following: research, preclinical, developmental, publication, clinical or regulatory milestones; cash flow (including, but not limited to, operating cash flow and free cash flow); revenue; earnings before interest, taxes, depreciation and amortization; net income (loss) (either before or after interest, taxes, depreciation and/or amortization); changes in the market price of our common stock; economic value- added; acquisitions, licenses, collaborations or strategic transactions; financing or other capital raising transactions; operating income (loss); return on capital, assets, equity or investment; stockholder returns; return on sales; total shareholder return; gross or net profit levels; productivity; expense efficiency; margins; operating efficiency; working capital; earnings (loss) per share of our common stock; bookings, new bookings or renewals; sales or market shares; number of prescriptions or prescribing physicians; coverage decisions; leadership development, employee retention and recruiting and other human resources matters; operating income and/or net annual recurring revenue, any of which may be (A) measured in absolute terms, as compared to any incremental increase, (B) measured in terms of growth, (C) compared to another company or companies or the results of a peer group, (D) measured against the market as a whole, compared to applicable market indices and/or (E) measured on a pre-tax or post-tax basis.

Each executive officer who is selected to participate in the Bonus Plan will have a target bonus opportunity set for each performance period. The bonus formulas will be adopted in each performance period by the board of directors or the compensation committee, as applicable, and communicated to each executive. The corporate performance goals will be measured at the end of each performance period at such time as the board of directors or the compensation committee determines. If the corporate performance goals and individual performance objectives are met, payments will be made as soon as practicable following the end of each performance period, but not later than 2.5 months after the end of the calendar year in which such performance period ends. Subject to the rights contained in any agreement between the executive officer and us, an executive officer shall be required to be employed by us on the bonus payment date to be eligible to receive a bonus payment under the Bonus Plan. The Bonus Plan also permits the board of directors or the compensation committee to approve additional bonuses to executive officers in its sole discretion.

Equity Grants to Employees (Including NEOs)

In connection with this offering, our board of directors approved grants to certain employees of the Company (including our NEOs) of options to purchase an aggregate of 1,758,504 shares of common stock (the “Employee IPO Grants”) under the 2025 Plan, with Mr. Cloonan, Ms. Ridloff and Dr. McKee being granted options to purchase 450,252, 157,820 and 148,536 shares of our common stock, respectively. The Employee IPO Grants were contingent and subject to the effectiveness of the registration statement of which this prospectus forms a part, which must occur no later than February 15, 2025, after which date these Employee IPO Grants shall be forfeited. The Employee IPO Grants each have an exercise price per share equal to $18.00, the per share “price to the public”, expire ten years from the date of grant and vest in 48 equal monthly installments following their applicable vesting start date, in each case subject to the applicable employee’s continued service relationship through each such vesting date. Vesting of the Employee IPO Grants for our NEOs commenced on the date that the registration statement of which this prospectus forms a part became effective. Certain of our non-employee directors also received equity grants in connection with this offering, as described in more detail below under “*Non-Employee Director Compensation Policy.*”

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DIRECTOR COMPENSATION

The following table presents the compensation awarded to, earned by, or paid to each person who served as a non-employee member of our board of directors during the fiscal year ended December 31, 2024 for their service on our board of directors during the year ended December 31, 2024. Non-employee directors Bruce Booth, Lucian Iancovici, H. Edward Fleming, Jr., Joshua Resnick, and Peter A. Thompson did not receive cash or equity compensation from us for their services as directors during 2024 due to their affiliation with Atlas Venture, TPG Growth, Enavate Sciences, RA Capital Management, L.P. and OrbiMed Advisors LLC, respectively. Our former non-employee director, Adam Rosenberg, also did not receive cash or equity compensation from us for his services as a director due to his former affiliation with RA Capital Management, L.P. and his former position as our chief executive officer prior to 2023. Other than as set forth in the table and described more fully below, we did not pay any compensation, make any equity awards or non-equity awards to, or pay any other compensation to any of the non-employee members of our board of directors in 2024. During the fiscal year ended December 31, 2024, Mr. Cloonan, our Chief Executive Officer, served as a member of our board of directors and received no additional compensation for his services as a member of our board of directors. The compensation for the fiscal year ended December 31, 2024 received by Mr. Cloonan, as an NEO, is presented in "*Executive Compensation—2024 Summary Compensation Table*" above.

2024 Director Compensation Table

Name	Fees Earned or Paid in Cash ($)(1)	Option Awards ($)(2)	Total ($)
Paul Clancy, M.B.A.(3)	50,000	294,287	344,287
Adam Rosenberg(4)	—	—	—
Bruce Booth, D.Phil.(5)	—	—	—
Lucian Iancovici, M.D.(5)	—	—	—
H. Edward Fleming, Jr., M.D.(5)	—	—	—
Joshua Resnick, M.D.(5)	—	—	—
Laurie Stelzer(6)	5,217	333,784	339,001
Peter A. Thompson, M.D.(5) . . .	—	—	—

(1) The amount reported represents the fees each director received for their services to our board of directors during the fiscal year ended December 31, 2024.

(2) The amounts reported represent the aggregate grant date fair value of stock option awards granted to Mr. Clancy and Ms. Stelzer during fiscal year 2024, computed in accordance with FASB ASC Topic 718. A discussion of the assumptions used in determining grant date fair value may be found in Note 12 to our financial statements, included elsewhere in this prospectus. Such grant date fair values do not take into account any estimated forfeitures related to service-based vesting. The amounts reported in this column reflect the accounting cost for the stock option award and do not correspond to the actual economic value that may be received by Mr. Clancy and Ms. Stelzer upon the vesting of the awards or any sale of the underlying securities.

(3) As of December 31, 2024, Mr. Clancy held outstanding options to purchase an aggregate of 185,485 shares of our common stock, and did not hold any other unvested stock awards.

(4) As of December 31, 2024, Mr. Rosenberg did not hold any outstanding options to purchase shares of our common stock, and did not hold any other unvested stock awards. Mr. Rosenberg resigned from the board of directors as of June 13, 2024.

(5) As of December 31, 2024, Drs. Booth, Iancovici, Fleming, Resnick and Thompson did not hold any outstanding options or any unvested stock awards.

(6) As of December 31, 2024, Ms. Stelzer held outstanding options to purchase an aggregate of 44,480 shares of our common stock, and did not hold any other unvested stock awards. Ms. Stelzer was appointed to the board of directors, effective November 12, 2024.

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Effective January 6, 2025, Marcella Ruddy and Joanne Viney both joined our board of directors.

Director Engagement Letters

We have entered into certain engagement letters with Paul Clancy, Laurie Stelzer, Marcella Ruddy and Joanne Viney. These engagement letters will be terminated in connection with this offering and director compensation shall be governed by the non-employee director compensation policy, as further detailed below.

Paul Clancy, M.B.A.

In March 2022, we entered into a board of directors agreement with Paul Clancy (the "Clancy Agreement") pursuant to which Mr. Clancy serves as the independent chairman of our board of directors, effective as of June 6, 2022. Under the Clancy Agreement, Mr. Clancy was entitled to receive an initial equity grant consisting of an option to purchase 119,772 shares of common stock, which vests over a four-year period in 16 equal quarterly installments following June 6, 2022, subject to Mr. Clancy's continued service with us through each applicable vesting date. In addition, under the Clancy Agreement, Mr. Clancy is entitled to an annual cash retainer of $50,000, payable quarterly, as well as reimbursement for reasonable travel expenses incurred by Mr. Clancy for his attendance at meetings of our board of directors.

In March 2024, following the Series C Financing, our board of directors granted Mr. Clancy a non-qualified stock option to purchase 65,713 shares of common stock, which vests in 48 equal monthly installments over a period of four years following March 4, 2024, subject to Mr. Clancy's continued service with us through each applicable vesting date.

Laurie Stelzer

On November 12, 2024, we entered into a board of directors agreement with Laurie Stelzer (the "Stelzer Agreement") pursuant to which Ms. Stelzer serves as a director and chair of the audit committee, effective as of November 14, 2024. In accordance with the Stelzer Agreement in November 2024, Ms. Stelzer was granted an initial equity grant consisting of an option to purchase 44,480 shares of common stock, which vests in equal annual installments over a three-year period following November 14, 2024, subject to Ms. Stelzer's continued service with us through each applicable vesting date. In addition, Ms. Stelzer is entitled to an annual cash retainer of $40,000 for her role as a director, as well as reimbursement for reasonable travel expenses incurred by Ms. Stelzer for her attendance at meetings of our board of directors, and, contingent upon the consummation of this offering, consistent with the terms of the non-employee director compensation policy, $16,000 for her role as the chair of the audit committee, in each case, payable quarterly.

Marcella Ruddy

On December 18, 2024, we entered into a board of directors agreement with Marcella Ruddy (the "Ruddy Agreement") pursuant to which Ms. Ruddy serves as a director and member of the nominating and corporate governance committee, effective as of January 6, 2025. Under the Ruddy Agreement, and subject to Board approval, Ms. Ruddy is entitled to receive an initial equity grant pursuant to the non-employee director compensation policy. In addition, Ms. Ruddy is entitled to an annual cash retainer of $40,000 for her role as a director, as well as reimbursement for reasonable travel expenses incurred by Ms. Ruddy for her attendance at meetings of our board of directors, and, contingent upon the consummation of this offering, consistent with the terms of the non-employee director compensation policy, $4,500 for her role as a member of the nominating and corporate governance committee, in each case, payable quarterly.

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Joanne Viney

On December 18, 2024, we entered into a board of directors agreement with Joanne Viney (the "Viney Agreement") pursuant to which Ms. Viney serves as a director and the chair of the compensation committee, effective as of January 6, 2025. Under the Viney Agreement, and subject to Board approval, Ms. Viney is entitled to receive an initial equity grant pursuant to the non-employee director compensation policy. In addition, Ms. Viney is entitled to an annual cash retainer of $40,000 for her role as a director as well as reimbursement for reasonable travel expenses incurred by Ms. Viney for her attendance at meetings of our board of directors, and, contingent upon the consummation of this offering, consistent with the terms of the non-employee director compensation policy, $12,000 for her role as the chair of the compensation committee, in each case, payable quarterly.

Indemnification Agreements

We have entered into customary indemnification agreements with each of our directors. In addition, in connection with this offering, we intend to enter into new indemnification agreements with each of our directors. For more information regarding these agreements, see the section titled "*Management—Limitations on Liability and Indemnification*."

Non-Employee Director Compensation Policy

In connection with this offering, our board of directors adopted a non-employee director compensation policy, which became effective as of the date on which the registration statement of which this prospectus forms a part was declared effective by the SEC, and which supersedes all existing board of directors agreements. The policy is designed to enable us to attract and retain, on a long-term basis, highly qualified non-employee directors. Under the policy, our non-employee directors will be eligible to receive cash retainers (which will be payable quarterly in arrears and prorated for partial years of service) and equity awards as set forth below:

Annual Retainer for Board Membership	
Members	$40,000
Additional Retainer for Non-executive Chair	$30,000
Additional Retainer for Committee Membership	
Audit Committee:	
Members (other than chair)	$ 8,000
Chair	$16,000
Compensation Committee:	
Members (other than chair)	$ 6,000
Chair	$12,000
Nominating and Corporate Governance Committee:	
Members (other than chair)	$ 4,500
Chair	$ 9,000

In addition, the non-employee director compensation policy provides that, upon initial election or appointment to our board of directors after the effective date of the registration statement of which this prospectus forms a part, each non-employee director will be granted an initial, one-time equity award consisting of a stock option grant to purchase 39,919 shares of our common stock (the "Initial Grant"). The Initial Grant will vest in equal annual installments over three years following the grant date, subject to continued service through the applicable vesting date. Mses. Ruddy and Viney each became a member of our board of directors on January 6, 2025 and each received an Initial Grant upon the

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effective date of this registration statement, with terms in accordance with the non-employee director compensation policy described above. Furthermore, on the date of each annual meeting of stockholders following the completion of this offering, each non-employee director who continues as a non-employee director following such meeting (other than a director receiving an Initial Grant on the date of such meeting) will be granted an annual stock option award to purchase 19,959 shares of our common stock (the "Annual Grant"). The Annual Grant will vest in full upon the earlier of (i) the first anniversary of the date of grant or (ii) the date of the next annual meeting of stockholders, subject to continued service through the applicable vesting date. Following the effective date of the non-employee director compensation policy, if a non-employee director joins our board of directors on a date other than the date of the annual meeting of stockholders, then in lieu of a full Annual Grant, such non-employee director will be granted a pro-rated portion of the Annual Grant based on the number of full months between the director's initial election or appointment and such annual meeting of stockholders, which will vest in accordance with the same terms as the Annual Grants. The Initial Grants and Annual Grants (including any pro rata portions thereof) will each expire ten years from the applicable date of grant and have an exercise price per share equal to the fair market value of our common stock on the date of grant.

The Initial Grants and the Annual Grants (including any pro rata portions thereof) are subject to full accelerated vesting upon a sale event (as defined in the 2025 Plan).

The aggregate amount of compensation, including both equity compensation and cash compensation, paid to any non-employee director for service as a non-employee director in a calendar year period will not exceed $1,000,000 in the first calendar year such individual becomes a non-employee director and $750,000 in any other calendar year (or such other limits as may be set forth in the 2025 Plan or any successor plan).

We will reimburse all reasonable out-of-pocket expenses incurred by non-employee directors for their attendance at meetings of our board of directors or any committee thereof.

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CERTAIN RELATIONSHIPS AND RELATED PERSON TRANSACTIONS

In addition to the compensation arrangements, including employment, termination of employment and change in control arrangements, and indemnification arrangements discussed, when required, in the sections titled "*Management*" and "*Executive Compensation*" and the registration rights described in the section titled "*Description of Capital Stock—Registration Rights*," the following is a description of all transactions since January 1, 2021 and each currently proposed transaction in which:

- we have been or are to be a participant;
- the amounts involved exceeded or will exceed the lesser of $120,000 or 1% of the average of our total assets at the year-end for the last two completed fiscal years; and
- any of our directors, executive officers or holders of more than 5% or more of our outstanding capital stock, or any immediate family member of, or person sharing the household with, any of these individuals or entities or affiliated entities, had or will have a direct or indirect material interest.

Financings

Series B Preferred Stock Financing

On February 2, 2022, we issued and sold an aggregate of 11,370,621 shares of our Series B convertible preferred stock, par value $0.001 per share ("Series B preferred stock"), at a purchase price of $9.762 per share, for an aggregate purchase price of approximately $111.0 million.

Each outstanding share of Series B preferred stock will convert into shares of common stock at a ratio of 1-for-1.4611 immediately prior to the completion of this offering. The following table summarizes the shares of our Series B preferred stock issued to our related parties:

Purchasers(1)	Shares of Series B Preferred Stock	Total Purchase Price
Entities affiliated with Atlas Venture(2)	1,298,332	$ 12,674,317
OrbiMed Private Investments VIII, LP(3)	2,560,951	$ 25,000,004
Entities affiliated with RA Capital(4)	3,079,242	$ 30,059,560
Entities affiliated with TPG Growth(5)	2,275,528	$ 22,213,704

(1) Additional details regarding these stockholders and their equity holdings are included in this prospectus under the section "*Principal Stockholders*."

(2) Consists of 1,298,332 shares of Series B preferred stock held by Atlas Venture Fund XI, L.P. Atlas Venture Fund XI, L.P. is an affiliate of Atlas Venture. Entities affiliated with Atlas Ventures beneficially own more than 5% of our outstanding capital stock. Bruce Booth, D.Phil., a member of our board of directors, is a general partner at Atlas Venture.

(3) OrbiMed Private Investments VIII, LP ("OrbiMed") beneficially owns more than 5% of our outstanding capital stock. Peter A. Thompson, M.D., a member of our board of directors, is a member at OrbiMed.

(4) Consists of (i) 2,155,469 shares of Series B preferred stock held by RA Capital Nexus Fund, L.P. and (ii) 923,773 shares of Series B preferred stock held by RA Capital Healthcare Fund, L.P. Entities affiliated with RA Capital beneficially own more than 5% of our outstanding capital stock. Joshua Resnick, M.D., M.B.A., a member of our board of directors, is a senior managing director at RA Capital.

(5) Consists of 2,275,528 shares of Series B preferred stock held by The Rise Fund Sling, L.P. Entities affiliated with TPG Growth beneficially owns more than 5% of our outstanding capital stock. Lucian Iancovici, M.D., a member of our board of directors, is a managing director at TPG Growth.

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Series C Preferred Stock Financing

On March 4, 2024, in an initial closing and a subsequent closing, we issued and sold 17,717,162 and 911,808 shares of our Series C convertible preferred stock, par value $0.001 per share ("Series C preferred stock"), respectively, representing an aggregate of 18,628,970 shares of Series C preferred stock, at a purchase price of $9.762 per share, for an aggregate purchase price of approximately $181.9 million.

Each outstanding share of Series C preferred stock will convert into shares of common stock at a ratio of 1-for-1.4611 immediately prior to the completion of this offering. The following table summarizes the shares of our Series C preferred stock issued to our related parties:

Purchasers(1)	Shares of Series C Preferred Stock	Total Purchase Price
Entities affiliated with Atlas Venture(2)	1,153,967	$ 11,265,026
OrbiMed Private Investments VIII, LP(3)	2,048,760	$ 19,999,995
Entities affiliated with RA Capital(4)	5,456,093	$ 53,262,380
Sionna Aggregator, LP(5)	2,817,045	$ 27,499,993
Entities affiliated with TPG Growth(6)	1,765,541	$ 17,235,211
Entities affiliated with Viking Global Opportunities(7)	2,560,950	$ 24,999,994

(1) Additional details regarding these stockholders and their equity holdings are included in this prospectus under the section "*Principal Stockholders*."

(2) Consists of (i) 61,463 shares of Series C preferred stock held by Atlas Venture Fund XI, L.P. and (ii) 1,092,504 shares of Series C preferred stock held by Atlas Venture Opportunity Fund II, L.P. Entities affiliated with Atlas Ventures beneficially own more than 5% of our outstanding capital stock. Bruce Booth, D.Phil., a member of our board of directors, is a general partner at Atlas Ventures.

(3) OrbiMed beneficially owns more than 5% of our outstanding capital stock. Peter A. Thompson, M.D., a member of our board of directors, is a member at OrbiMed.

(4) Consists of (i) 4,364,874 shares of Series C preferred stock held by RA Capital Healthcare Fund, L.P. and (ii) 1,091,219 shares of Series C preferred stock held by RA Capital Nexus Fund, L.P. Entities affiliated with RA Capital beneficially own more than 5% of our outstanding capital stock. Joshua Resnick, M.D., M.B.A., a member of our board of directors, is a senior managing director at RA Capital.

(5) Sionna Aggregator, LP beneficially owns more than 5% of our outstanding capital stock. Sionna Aggregator, LP is an affiliate of Enavate Sciences. H. Edward Fleming, Jr., M.D., a member of our board of directors, is an executive vice president of commercialization at Enavate Sciences.

(6) Consists of 1,765,541 shares of Series C preferred stock held by The Rise Fund Sling II, L.P. Entities affiliated with TPG Growth beneficially owns more than 5% of our outstanding capital stock. Lucian Iancovici, M.D., a member of our board of directors, is a managing director at TPG Growth.

(7) Consists of (i) 1,715,837 shares of Series C preferred stock held by Viking Global Opportunities Illiquid Investments Sub-Master LP and (ii) 845,113 shares of Series C preferred stock held by Viking Global Opportunities Drawdown (Aggregator) LP. Entities affiliated with Viking Global Opportunities beneficially own more than 5% of our outstanding capital stock.

Employment Arrangements

We have entered into employment agreements with certain of our named executive officers, and granted stock options to our named executive officers and certain of our directors, as more fully described in "*Executive Compensation*" and "*Director Compensation*."

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Investors' Rights Agreement

In March 2024, we entered into a third amended and restated investors' rights agreement (the "investors' rights agreement") with certain holders of more than 5% of our outstanding capital stock.

This investors' rights agreement provides certain holders of our preferred stock with a participation right to purchase their pro rata share of new securities that we may propose to sell and issue, subject to certain exceptions. Such participation right will terminate upon the completion of this offering. The investors' rights agreement further provides certain holders of our preferred stock with certain rights, including certain registration rights with respect to the registrable securities held by them. See the section titled "*Description of Capital Stock—Registration Rights*" for additional information.

Voting Agreement

In March 2024, we entered into a second amended and restated voting agreement ("voting agreement") with certain holders of more than 5% of our outstanding capital stock.

Our voting agreement provides for drag-along rights in respect of sales by certain holders of our capital stock. The voting agreement also contains provisions with respect to the elections of our board of directors and its composition. The rights under the voting agreement will terminate upon the completion of this offering.

Right of First Refusal and Co-Sale Agreement

In March 2024, we entered into a second amended and restated right of first refusal and co-sale agreement ("right of first refusal and co-sale agreement") with certain holders of more than 5% of our outstanding capital stock.

Our right of first refusal and co-sale agreement provides for rights of first refusal and co-sale rights in respect of sales by certain holders of our capital stock. The rights under the right of first refusal and co-sale agreement will terminate upon the completion of this offering.

Indemnification Agreements

Our fifth amended and restated certificate of incorporation will contain provisions limiting the liability of directors and officers, and our amended and restated bylaws provide that we will indemnify each of our directors and officers to the fullest extent permitted under Delaware law. Our fifth amended and restated certificate of incorporation and amended and restated bylaws also provide our board of directors with discretion to indemnify our employees and other agents when determined appropriate by the board. In addition, we have entered into or intend to enter into an indemnification agreement with each of our directors and executive officers, which will require us to indemnify them. For more information regarding these agreements, see the section titled "*Management—Limitations on Liability and Indemnification*."

Policies and Procedures for Transactions with Related Persons

In connection with this offering, we adopted, effective upon the effectiveness of the registration statement of which this prospectus forms a part, a written policy that our executive officers, directors, nominees for election as a director, beneficial owners of more than 5% of any series of our common stock and any members of the immediate family of any of the foregoing persons are not permitted to enter into a related person transaction with us without the approval or ratification of our board of directors or our audit committee. Any request for us to enter into a transaction with an executive officer,

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director, nominee for election as a director, beneficial owner of more than 5% of any series of our common stock or any member of the immediate family of any of the foregoing persons, in which the amount involved exceeds $120,000 (or, if less, 1% of the average of our total assets in a fiscal year) and such person would have a direct or indirect interest, must be presented to our board of directors or our audit committee for review, consideration and approval. In approving or rejecting any such proposal, our board of directors or our audit committee is to consider the material facts of the transaction, including whether the transaction is on terms no less favorable than terms generally available to an unaffiliated third party under the same or similar circumstances and the extent of the related person's interest in the transaction.

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PRINCIPAL STOCKHOLDERS

The following table sets forth information regarding beneficial ownership of our capital stock as of December 31, 2024 by:

- each person, or group of affiliated persons, known by us to beneficially own more than 5% of our common stock;
- each of our directors;
- each of our named executive officers; and
- all of our current executive officers and directors as a group.

We have determined beneficial ownership in accordance with the rules of the SEC. Under these rules, beneficial ownership includes any shares of common stock as to which the individual or entity has sole or shared voting power or investment power. Unless otherwise indicated below, to our knowledge the persons and entities named in the table have sole voting and sole investment power with respect to all shares that they beneficially owned, subject to community property laws where applicable. We have deemed shares of common stock subject to options that are currently exercisable or exercisable within 60 days of December 31, 2024, to be outstanding and to be beneficially owned by the person holding the option for the purpose of computing the percentage ownership of that person but have not treated them as outstanding for the purpose of computing the percentage ownership of any other person.

Applicable percentage ownership before the offering is based on an aggregate of 31,947,927 shares of common stock (which includes 75,958 shares of restricted common stock) deemed to be outstanding as of December 31, 2024, after giving effect to the automatic conversion of all outstanding shares of preferred stock into 27,149,206 shares of common stock immediately prior to the completion of this offering, and based on the initial public offering price of $18.00 per share.

Applicable percentage ownership after the offering is based on 42,536,160 shares of common stock assumed to be outstanding immediately after the completion of this offering (including the sale of shares of common stock in this offering and assuming no exercise of the underwriters' option to purchase additional shares), and does not include any shares which may be purchased by any person or entity named in the table in this offering.

Unless otherwise indicated, the address for each beneficial owner listed in the table below is c/o Sionna Therapeutics, Inc., 21 Hickory Drive, Suite 500, Waltham, MA 02451.

	Before Offering		After Offering	
Name of Beneficial Owner	Number of Shares Beneficially Owned	Percentage of Shares Beneficially Owned	Number of Shares Beneficially Owned	Percentage of Shares Beneficially Owned
5% or Greater Shareholders:				
Entities affiliated with Atlas Venture(1)	3,634,020	11.4%	3,634,020	8.5%
OrbiMed Private Investments VIII, LP(2)	3,154,959	9.9%	3,154,959	7.4%
Entities affiliated with RA Capital(3)	9,320,322	29.2%	9,320,322	21.9%
Sionna Aggregator, LP(4)	1,928,030	6.0%	1,928,030	4.5%
Entities affiliated with TPG Growth(5)	5,559,962	17.4%	5,559,962	13.1%
Entities affiliated with Viking Global Opportunities(6)	1,752,754	5.5%	1,752,754	4.1%
Named Executive Officers and Directors:				
Michael Cloonan, M.B.A., *Chief Executive Officer, President and Director*(7)	947,245	2.9%	947,245	2.2%
Charlotte McKee, M.D., *Chief Medical Officer*(8)	141,189	*	141,189	*

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Name of Beneficial Owner	Before Offering: Number of Shares Beneficially Owned	Before Offering: Percentage of Shares Beneficially Owned	After Offering: Number of Shares Beneficially Owned	After Offering: Percentage of Shares Beneficially Owned
Elena Ridloff, C.F.A., *Chief Financial Officer and Head of Corporate Development*(9)	248,892	*	248,892	*
Bruce Booth, D.Phil.	—	*	—	*
Paul Clancy, M.B.A.(10)	89,916	*	89,916	*
H. Edward Fleming, Jr., M.D.	—	*	—	*
Lucian Iancovici, M.D.	—	*	—	*
Joshua Resnick, M.D., M.B.A.	—	*	—	*
Marcella Kuhlman Ruddy, M.D.	—	*	—	*
Laurie Stelzer, M.B.A.	—	*	—	*
Peter A. Thompson, M.D.	—	*	—	*
Joanne Louise Viney, Ph.D.	—	*	—	*
All executive officers and directors as a group (12 persons)(11)	1,427,242	4.4%	1,427,242	3.3%

* Represents beneficial ownership of less than 1%

(1) Consists of (i) 719,355 shares of common stock issuable upon the conversion of Series Seed preferred stock held by Atlas Venture Fund XI, L.P. (“AVF XI”), (ii) 1,236,271 shares of common stock issuable upon the conversion of Series A preferred stock held by AVF XI, (iii) 888,599 shares of common stock issuable upon the conversion of Series B preferred stock held by AVF XI, (iv) 42,068 shares of common stock issuable upon the conversion of Series C preferred stock held by AVF XI, and (v) 747,727 shares of common stock issuable upon the conversion of Series C preferred stock held by Atlas Venture Opportunity Fund II, L.P. (“AVOF II”). Atlas Venture Associates XI, L.P. is the general partner of AVF XI and Atlas Venture Associates XI, LLC is the general partner of Atlas Venture Associates XI, L.P. The members of Atlas Venture Associates XI, LLC collectively make investment decisions on behalf of Atlas Venture Fund XI, LLC. Bruce Booth is a member of Atlas Venture Associates XI, LLC and a member of our board of directors. Each of AVF XI, Atlas Venture Associates XI, L.P. and Atlas Venture Associates XI, LLC may be deemed to beneficially own the shares held by AVF XI. Dr. Booth expressly disclaims beneficial ownership of the shares owned by AVF XI, except to the extent of his pecuniary interest therein, if any. Atlas Venture Associates Opportunity II, L.P. is the general partner of AVOF II, and Atlas Venture Associates Opportunity II, LLC is the general partner of Atlas Venture Associates Opportunity II, L.P. The members of Atlas Venture Associates Opportunity II, LLC collectively make investment decisions on behalf of Atlas Venture Associates Opportunity II, LLC. Dr. Booth is a member of Atlas Venture Associates Opportunity II, LLC. Each of AVOF II, Atlas Venture Associates Opportunity II, L.P., and Atlas Venture Associates Opportunity II, LLC may be deemed to beneficially own the shares held by AVOF II. Dr. Booth expressly disclaims beneficial ownership of the shares owned by AVOF II, except to the extent of his pecuniary interest therein, if any. The address for each of these entities and individuals is 300 Technology Square, 8th Floor, Cambridge, MA 02139.

(2) Consists of (i) 1,752,755 shares of common stock issuable upon the conversion of Series B preferred stock held by OrbiMed Private Investments VIII, LP (“OPI VIII”) and (ii) 1,402,204 shares of common stock issuable upon the conversion of Series C preferred stock held by OPI VIII. OrbiMed Capital GP VIII LLC (“GP VIII”) is the general partner of OPI VIII. OrbiMed Advisors LLC (“OrbiMed Advisors”) is the managing member of GP VIII. By virtue of such relationships, GP VIII and OrbiMed Advisors may be deemed to have voting power and investment power over the securities held by OPI VIII and as a result, may be deemed to have beneficial ownership over such securities. OrbiMed Advisors exercises voting and investment power through a management committee comprised of Carl L. Gordon, Sven H. Borho, and W. Carter Neild, each of whom disclaims beneficial ownership of the shares held by OPI VIII. The address for each of these entities and individuals is 601 Lexington Avenue, 54th Floor, New York, NY 10022.

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(3) Consists of (i) 878,789 shares of common stock held by RA Capital Healthcare Fund, L.P. ("RACHF"), (ii) 560,642 shares of common stock issuable upon the conversion of Series Seed preferred stock held by RACHF, (iii) 841,716 shares of common stock issuable upon the conversion of Series A preferred stock held by RACHF, (iv) 632,244 shares of common stock issuable upon the conversion of Series B preferred stock held by RACHF, (v) 2,987,388 shares of common stock issuable upon the conversion of Series C preferred stock held by RACHF, (vi) 342,207 shares of common stock held by RA Capital Nexus Fund, L.P. ("Nexus"), (vii) 218,375 shares of common stock issuable upon conversion of Series Seed preferred stock held by Nexus, (viii) 309,069 shares of common stock issuable upon the conversion of Series A preferred stock held by Nexus, (ix) 1,475,237 shares of common stock issuable upon the conversion of Series B preferred stock held by RA Capital Nexus Fund III, L.P. ("Nexus III"), (x) 746,847 shares of common stock issuable upon the conversion of Series C preferred stock held by Nexus III, (xi) 147,833 shares of common stock held by Blackwell Partners LLC—Series A ("Blackwell"), (xii) 94,486 shares of common stock issuable upon the conversion of Series Seed preferred stock held by Blackwell and (xiii) 85,489 shares of common stock issuable upon the conversion of Series A preferred stock held by Blackwell. RA Capital Management, LP ("RACM") is the investment adviser to RACHF, Nexus, Nexus III and Blackwell. RA Capital Management GP, LLC ("RACM GP") is the general partner of RACM. Peter Kolchinsky and Rajeev Shah are the managing members of RACM GP and may be deemed to have voting and investment power over the shares held by RACHF, Nexus, Nexus III and Blackwell. RACM, RACM GP, Peter Kolchinsky and Rajeev Shah disclaim beneficial ownership of the shares owned by RACHF, Nexus, Nexus III and Blackwell, except to the extent of their pecuniary interest therein, if any. The address for each of these entities and individuals is 200 Berkeley Street, 18th Floor, Boston, MA 02116.

(4) Consists of 1,928,030 shares of common stock issuable upon the conversion of Series C preferred stock held by Sionna Aggregator, LP. ("Sionna Aggregator"), a limited partnership affiliated with Enavate Sciences. Enavate Sciences GP, LLC ("Enavate GP") is the general partner of Sionna Aggregator. Voting, investment and dispositive power with respect to the shares held by Sionna Aggregator are made collectively by the managers of Enavate GP: Jim Momtazee, Laura Furmanski, Neel Varshney and James P. Boylan, each of whom expressly disclaims beneficial ownership of the shares. H. Edward Fleming, Jr., M.D. is an executive vice president at Enavate Sciences and a member of our board of directors and expressly disclaims beneficial ownership of the shares held by Sionna Aggregator. The address for each of these entities and individuals is 106 W 56th Street, 8th Floor, New York, NY 10019.

(5) Consists of (i) 684,415 shares of common stock held by The Rise Fund Sling, L.P., a Delaware limited partnership ("RFS"), (ii) 873,503 shares of common stock issuable upon the conversion of Series Seed preferred stock held by RFS, (iii) 1,236,272 shares of common stock issuable upon the conversion of Series A preferred stock held by RFS, (iv) 1,557,408 shares of common stock issuable upon the conversion of Series B preferred stock held by RFS, and (v) 1,208,364 shares of common stock issuable upon the conversion of Series C preferred stock held by The Rise Fund Sling II, L.P., a Delaware limited partnership ("RFS II"). The general partner of each of RFS and RFS II is The Rise Fund SPV GP, LLC, a Delaware limited liability company, whose managing member is The Rise Fund GenPar, L.P., a Delaware limited partnership, whose general partner is The Rise Fund GenPar Advisors, LLC, a Delaware limited liability company, whose sole member is TPG Operating Group I, L.P., a Delaware limited partnership, whose general partner is TPG Holdings I-A, LLC, a Delaware limited liability company, whose sole member is TPG Operating Group II, L.P., a Delaware limited partnership, whose general partner is TPG Holdings II-A, LLC, a Delaware limited liability company, whose sole member is TPG GPCo, LLC, a Delaware limited liability company, whose sole member is TPG Inc., a Delaware corporation, whose shares of Class B common stock (which represent a majority of the combined voting power of the common stock) are held collectively by (i) TPG Group Holdings (SBS), L.P., a Delaware limited partnership, whose general partner is TPG Group Holdings (SBS) Advisors, LLC, a Delaware limited liability

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company, (ii) Alabama Investments (Parallel), LP, a Delaware limited partnership, whose general partner is Alabama Investments (Parallel) GP, LLC, a Delaware limited liability company ("Alabama Investments"), (iii) Alabama Investments (Parallel) Founder A, LP, a Delaware limited partnership, whose general partner is Alabama Investments, and (iv) Alabama Investments (Parallel) Founder G, LP, a Delaware limited partnership, whose general partner is Alabama Investments. The managing member of each of TPG Group Holdings (SBS) Advisors, LLC and Alabama Investments is TPG GP A, LLC, a Delaware limited liability company, which is controlled by entities owned by David Bonderman, James G. Coulter and Jon Winkelried. Messrs. Bonderman, Coulter and Winkelried disclaim beneficial ownership of the securities held by RFS and RFS II except to the extent of their pecuniary interest therein. Lucian Iancovici, M.D. is a managing director with TPG Growth and expressly disclaims beneficial ownership of the shares held by RFS and RFS II. The address of each of these entities and individuals is 301 Commerce Street, Suite 3300, Fort Worth, TX 76102.

(6) Consists of (i) 578,408 shares of common stock issuable upon the conversion of Series C preferred stock held by Viking Global Opportunities Drawdown (Aggregator) LP (the "Drawdown Fund") which has the authority to dispose of and vote such shares which power may be exercised by its general partner, Viking Global Opportunities Drawdown Portfolio GP LLC (the "Drawdown GP") and (ii) 1,174,346 shares of common stock issuable upon the conversion of Series C preferred stock held by Viking Global Opportunities Illiquid Investments Sub-Master LP (the "Opportunities Fund"), which has the authority to dispose of and vote such shares which power may be exercised by its general partner, Viking Global Opportunities Portfolio GP LLC (the "Opportunities GP"). Viking Global Investors LP ("VGI"), which provides managerial services to the Drawdown Fund and the Opportunities Fund, has the authority to dispose of and vote the shares held by the Opportunities Fund and the Drawdown Fund. O. Andreas Halvorsen, David C. Ott and Rose Shabet, as Executive Committee members of Viking Global Partners LLC (the general partner of VGI) and Viking Global Opportunities Parent GP LLC, the sole member of each of (a) Viking Global Opportunities Drawdown GP LLC (which is the sole member of the Drawdown GP) and (b) Viking Global Opportunities GP LLC (which is the sole member of the Opportunities GP), have shared authority to direct the voting and disposition of investments beneficially owned by VGI, the Opportunities GP and the Drawdown GP, respectively. The address of each of these entities and individuals is c/o Viking Global Investors LP, 600 Washington Blvd. Floor 11, Stamford, CT 06901.

(7) Consists of (i) 547,343 shares of restricted common stock and (ii) 399,902 shares of common stock subject to options exercisable within 60 days of December 31, 2024.

(8) Consists of (i) 28,415 shares of restricted common stock and (ii) 112,774 shares of common stock subject to options exercisable within 60 days of December 31, 2024.

(9) Consists of (i) 123,537 shares of restricted common stock and (ii) 125,355 shares of common stock subject to options exercisable within 60 days of December 31, 2024.

(10) Consists of 89,916 shares of common stock subject to options exercisable within 60 days of December 31, 2024.

(11) Consists of (i) 699,295 shares of restricted common stock and (ii) 727,947 shares of common stock subject to options exercisable within 60 days of December 31, 2024, held by executive officers and directors, as described in footnotes 7 through 10 above.

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DESCRIPTION OF CAPITAL STOCK

General

The following description of our capital stock and certain provisions of our fifth amended and restated certificate of incorporation and amended and restated bylaws are summaries and are qualified by reference to the fifth amended and restated certificate of incorporation, which will become effective immediately prior to the completion of this offering, and the amended and restated bylaws, which became effective upon the effectiveness of the registration statement of which this prospectus forms a part. Copies of these documents have been filed with the SEC as exhibits to our registration statement, of which this prospectus forms a part. The descriptions of the common stock and preferred stock reflect changes to our capital structure that will be in effect on the completion of this offering.

Upon filing of our fifth amended and restated certificate of incorporation and the completion of this offering, our authorized capital stock will consist of 500,000,000 shares of common stock, par value $0.001 per share, and 10,000,000 shares of preferred stock, par value $0.001 per share, all of which shares of preferred stock will be undesignated.

As of December 31, 2024, there were 31,947,927 shares of common stock outstanding and held of record by 39 stockholders. This amount assumes the conversion of all outstanding shares of our preferred stock into common stock, which will occur immediately prior to the completion of this offering.

Common Stock

Holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of the stockholders. The holders of our common stock do not have any cumulative voting rights. Holders of our common stock are entitled to receive ratably any dividends declared by our board of directors out of funds legally available for that purpose, subject to any preferential dividend rights of any outstanding preferred stock. Our common stock has no preemptive rights, conversion rights or other subscription rights or redemption or sinking fund provisions.

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in all assets remaining after payment of all debts and other liabilities and any liquidation preference of any outstanding preferred stock. The shares to be issued by us in this offering will be, when issued and paid for, validly issued, fully paid and non-assessable.

Preferred Stock

Immediately prior to the completion of this offering, all outstanding shares of our preferred stock will be converted into shares of our common stock. Upon the consummation of this offering, our board of directors will have the authority, without further action by our stockholders, to issue up to 10,000,000 shares of preferred stock in one or more series and to fix the rights, preferences, privileges and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, terms of redemption, liquidation preferences, sinking fund terms and the number of shares constituting, or the designation of, such series, any or all of which may be greater than the rights of common stock. The issuance of our preferred stock could adversely affect the voting power of holders of common stock and the likelihood that such holders will receive dividend payments and payments upon our liquidation. In addition, the issuance of preferred stock could have the effect of delaying, deferring or preventing a change in control of our company or other corporate action. Immediately after consummation of this offering, no shares of preferred stock will be outstanding, and we have no present plan to issue any shares of preferred stock.

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Stock Options

As of December 31, 2024, 3,700,335 shares of common stock were issuable upon the exercise of outstanding stock options under the 2020 Plan, at a weighted-average exercise price of $5.87 per share and 5,060,000 shares of our common stock reserved for future issuance under the 2025 Plan, which became effective once the registration statement of which this prospectus forms a part was declared effective, as well as any future automatic annual increases in the number of shares of common stock reserved for issuance under the 2025 Plan and any shares underlying outstanding stock awards granted under the 2020 Plan, that expire or are repurchased, forfeited, cancelled or withheld. For additional information regarding terms of our equity incentive plans, see the section titled "*Executive Compensation—Employee Benefit and Equity Compensation Plans*."

Registration Rights

Upon the completion of this offering and subject to the lock-up agreements entered into in connection with this offering and federal securities laws, certain holders of shares of our common stock, including those shares of our common stock that will be issued upon the conversion of our preferred stock in connection with this offering, will initially be entitled to certain rights with respect to registration of such shares under the Securities Act. These shares are referred to as registrable securities. The holders of these registrable securities possess registration rights pursuant to the terms of our third amended and restated investors' rights agreement and are described in additional detail below. The registration of shares of our common stock pursuant to the exercise of the registration rights described below would enable the holders to trade these shares without restriction under the Securities Act when the applicable registration statement is declared effective. We will pay all registration expenses, other than underwriting discounts, selling commissions and stock transfer taxes, of the shares registered pursuant to the demand, piggyback and Form S-3 registrations described below.

Generally, in an underwritten offering, the managing underwriter, if any, has the right, subject to specified conditions and limitations, to limit the number of shares the holders may include. The demand, piggyback and Form S-3 registration rights described below will expire no later than four years after the completion of this offering.

Form S-1 Registration Rights

Upon the completion of this offering, certain holders of our common stock, including those issuable upon the conversion of shares of our preferred stock upon completion of this offering, will be entitled to certain demand registration rights. At any time beginning 180 days after the completion of this offering, the holders of a majority of registrable securities then outstanding may request that we register all or a portion of their shares on Form S-1 with respect to at least 40% of the registrable securities then outstanding. With certain exceptions, we are not required to effect the filing of a registration statement during the period starting with the date of the filing of, and ending on a date 60 days following the effective date of the registration statement for this offering.

Piggyback Registration Rights

In connection with this offering, certain holders of our common stock, including those issuable upon the conversion of shares of our preferred stock upon completion of this offering, were entitled to, and the necessary percentage of holders waived, their rights to notice of this offering and to include their shares of registrable securities in this offering. After this offering, in the event that we propose to register any of our securities under the Securities Act, either for our own account or for the account of other security holders, the holders of these shares will be entitled to certain piggyback registration rights allowing the holder to include their shares in such registration, subject to certain marketing and other limitations.

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Form S-3 Registration Rights

Upon the completion of this offering, certain holders of our common stock, including those issuable upon the conversion of shares of our preferred stock upon completion of this offering, will be entitled to certain Form S-3 registration rights. Holders of at least 10% of registrable securities then outstanding can make a request that we register their shares on Form S-3 if we are qualified to file a registration statement on Form S-3 and if the reasonably anticipated aggregate net proceeds of the shares offered would equal or exceed $5 million. We will not be required to effect more than two registrations on Form S-3 within any twelve-month period. The right to have such shares registered on Form S-3 is further subject to other specified conditions and limitations.

Expiration of Registration Rights

The demand registration rights and short-form registration rights granted under the investor rights agreement will terminate on the fifth anniversary of the completion of this offering.

Anti-takeover Effects of Our Certificate of Incorporation and Bylaws and Delaware Law

Our fifth amended and restated certificate of incorporation and amended and restated bylaws include a number of provisions that may have the effect of delaying, deferring or preventing another party from acquiring control of us and encouraging persons considering unsolicited tender offers or other unilateral takeover proposals to negotiate with our board of directors rather than pursue non-negotiated takeover attempts. These provisions include the items described below.

Board Composition and Filling Vacancies

Our fifth amended and restated certificate of incorporation will provide for the division of our board of directors into three classes serving staggered three-year terms, with one class being elected each year. Our fifth amended and restated certificate of incorporation also will provide that directors may be removed only for cause and then only by the affirmative vote of the holders of two-thirds or more of the shares then entitled to vote at an election of directors. Furthermore, any vacancy on our board of directors, however occurring, including a vacancy resulting from an increase in the size of our board, may only be filled by the affirmative vote of a majority of our directors then in office even if less than a quorum. The classification of directors, together with the limitations on removal of directors and treatment of vacancies, has the effect of making it more difficult for stockholders to change the composition of our board of directors.

No Written Consent of Stockholders

Our fifth amended and restated certificate of incorporation will provide that all stockholder actions are required to be taken by a vote of the stockholders at an annual or special meeting, and that stockholders may not take any action by written consent in lieu of a meeting. This limit may lengthen the amount of time required to take stockholder actions and would prevent the amendment of our amended and restated bylaws or removal of directors by our stockholders without holding a meeting of stockholders.

Meetings of Stockholders

Our fifth amended and restated certificate of incorporation and amended and restated bylaws provide that only a majority of the members of our board of directors then in office may call special meetings of stockholders and only those matters set forth in the notice of the special meeting may be considered or acted upon at a special meeting of stockholders. Our amended and restated bylaws limit the business that may be conducted at an annual meeting of stockholders to those matters properly brought before the meeting.

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Advance Notice Requirements

Our amended and restated bylaws will establish advance notice procedures with regard to stockholder proposals relating to the nomination of candidates for election as directors or new business to be brought before meetings of our stockholders. These procedures provide that notice of stockholder proposals must be timely given in writing to our corporate secretary prior to the meeting at which the action is to be taken. Generally, to be timely, notice must be received at our principal executive offices not less than 90 days nor more than 120 days prior to the first anniversary date of the annual meeting for the preceding year. Our amended and restated bylaws specify the requirements as to form and content of all stockholders' notices. These requirements may preclude stockholders from bringing matters before the stockholders at an annual or special meeting.

Amendment to Certificate of Incorporation and Bylaws

Any amendment of our fifth amended and restated certificate of incorporation must first be approved by a majority of our board of directors, and if required by law or our fifth amended and restated certificate of incorporation, must thereafter be approved by a majority of the outstanding shares entitled to vote on the amendment and a majority of the outstanding shares of each class entitled to vote thereon as a class, except that the amendment of the provisions relating to stockholder action, board composition and limitation of liability must be approved by not less than two-thirds of the outstanding shares entitled to vote on the amendment, and not less than two-thirds of the outstanding shares of each class entitled to vote thereon as a class. Our amended and restated bylaws may be amended by the affirmative vote of a majority of the directors then in office, subject to any limitations set forth in the amended and restated bylaws; and may also be amended by the affirmative vote of a majority of the outstanding shares entitled to vote on the amendment, voting together as a single class, except that the amendment of the provisions relating to notice of stockholder business and nominations and special meetings must be approved by not less than two-thirds of the outstanding shares entitled to vote on the amendment, and not less than two-thirds of the outstanding shares of each class entitled to vote thereon as a class, or, if our board of directors recommends that the stockholders approve the amendment, by the affirmative vote of the majority of the outstanding shares entitled to vote on the amendment, in each case voting together as a single class.

Undesignated Preferred Stock

Our fifth amended and restated certificate of incorporation will provide for 10,000,000 authorized shares of preferred stock. The existence of authorized but unissued shares of preferred stock may enable our board of directors to discourage an attempt to obtain control of us by means of a merger, tender offer, proxy contest or otherwise. For example, if in the due exercise of its fiduciary obligations, our board of directors were to determine that a takeover proposal is not in the best interests of our stockholders, our board of directors could cause shares of preferred stock to be issued without stockholder approval in one or more private offerings or other transactions that might dilute the voting or other rights of the proposed acquirer or insurgent stockholder or stockholder group. In this regard, our fifth amended and restated certificate of incorporation will grant our board of directors broad power to establish the rights and preferences of authorized and unissued shares of preferred stock. The issuance of shares of preferred stock could decrease the amount of earnings and assets available for distribution to holders of shares of common stock. The issuance may also adversely affect the rights and powers, including voting rights, of these holders and may have the effect of delaying, deterring or preventing a change in control of us.

Section 203 of the Delaware General Corporation Law

Upon completion of this offering, we will be subject to the provisions of Section 203 of the Delaware General Corporation Law (the "DGCL"). In general, Section 203 prohibits a publicly held

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Delaware corporation from engaging in a "business combination" with an "interested stockholder" for a three-year period following the time that this stockholder becomes an interested stockholder, unless the business combination is approved in a prescribed manner. Under Section 203, a business combination between a corporation and an interested stockholder is prohibited unless it satisfies one of the following conditions:

- before the stockholder became interested, our board of directors approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;
- upon consummation of the transaction which resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding, shares owned by persons who are directors and also officers, and employee stock plans, in some instances, but not the outstanding voting stock owned by the interested stockholder; or
- at or after the time the stockholder became interested, the business combination was approved by our board of directors and authorized at an annual or special meeting of the stockholders by the affirmative vote of at least two-thirds of the outstanding voting stock which is not owned by the interested stockholder.

Section 203 defines a business combination to include:

- any merger or consolidation involving the corporation and the interested stockholder;
- any sale, transfer, lease, pledge or other disposition involving the interested stockholder of 10% or more of the assets of the corporation;
- subject to exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;
- subject to exceptions, any transaction involving the corporation that has the effect of increasing the proportionate share of the stock of any class or series of the corporation beneficially owned by the interested stockholder; and
- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits provided by or through the corporation.

In general, Section 203 defines an interested stockholder as any entity or person beneficially owning 15% or more of the outstanding voting stock of the corporation and any entity or person affiliated with or controlling or controlled by the entity or person.

Choice of Forum

Our amended and restated bylaws provide that the Court of Chancery of the State of Delaware is the sole and exclusive forum for the following claims or causes of action under the Delaware statutory or common law: (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim of, or a claim based on, a breach of a fiduciary duty owed by any of our current or former directors, officers or other employees or stockholders to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the DGCL or our fifth amended and restated certificate of incorporation or amended and restated bylaws (including the interpretation, validity or enforceability thereof) or as to which the DGCL confers jurisdiction on the Court of Chancery of the State of Delaware or (iv) any action asserting a claim governed by the internal affairs doctrine.

However, Section 27 of the Exchange Act creates exclusive federal jurisdiction over all claims brought to enforce any duty or liability created by the Exchange Act or the rules and regulations

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thereunder. Consequently, this choice of forum provision would not apply to claims or causes of action brought to enforce a duty or liability created by the Exchange Act or any other claim for which the federal courts have exclusive jurisdiction or the Securities Act. Moreover, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all claims brought to enforce any duty or liability created by the Securities Act or the rules and regulations thereunder.

In addition, our amended and restated bylaws provide that, unless we consent in writing to the selection of an alternative forum, to the fullest extent permitted by law, the federal district courts of the U.S. of America shall be the exclusive forum for the resolution of any complaint asserting a cause or causes of action arising under the Securities Act, including all causes of action asserted against any defendant to such complaint. For the avoidance of doubt, this provision is intended to benefit and may be enforced by us, our officers and directors, the underwriters to any offering giving rise to such complaint, and any other professional entity whose profession gives authority to a statement made by that person or entity and who has prepared or certified any part of the documents underlying the offering.

While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions, and there can be no assurance that such provisions will be enforced by a court in those other jurisdictions. We note that investors cannot waive compliance with the federal securities laws and the rules and regulations thereunder.

Additionally, our amended and restated bylaws provide that any person or entity holding, owning or otherwise acquiring any interest in any of our securities shall be deemed to have notice of and consented to these provisions.

Limitations on Liability and Indemnification

See the section titled "*Management—Limitations on Liability and Indemnification*" appearing elsewhere in this prospectus.

Exchange Listing

Our common stock has been approved for listing on The Nasdaq Global Market under the symbol "SION."

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is Computershare Trust Company, N.A.

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SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for our common stock. Future sales of substantial amounts of our common stock, including shares issued on the exercise of outstanding options, in the public market after this offering, or the possibility of these sales or issuances occurring, could adversely affect the prevailing market price for our common stock or impair our ability to raise equity capital. Although we have been approved to list our common stock on The Nasdaq Global Market, we cannot assure you that there will be an active public market for our common stock.

Following the completion of this offering, based on our shares outstanding as of September 30, 2024, a total of 42,520,700 shares of common stock will be outstanding, assuming no exercise of the underwriters' option to purchase additional shares and no exercise of outstanding options. Of the outstanding shares, all of the shares sold in this offering will be freely tradable.

All remaining shares of common stock held by existing stockholders immediately prior to the completion of this offering will be "restricted securities" as such term is defined in Rule 144. These restricted securities were issued and sold by us, or will be issued and sold by us, in private transactions and are eligible for public sale only if registered under the Securities Act or if they qualify for an exemption from registration under the Securities Act, including the exemptions provided by Rule 144 or Rule 701, summarized below.

Rule 144

In general, under Rule 144 as currently in effect, once we have been subject to public company reporting requirements of Section 13 or Section 15(d) of the Exchange Act for at least 90 days, an eligible stockholder is entitled to sell such shares without complying with the manner of sale, volume limitation or notice provisions of Rule 144, subject to compliance with the public information requirements of Rule 144. To be an eligible stockholder under Rule 144, such stockholder must not be deemed to have been one of our affiliates for purposes of the Securities Act at any time during the 90 days preceding a sale and must have beneficially owned the shares proposed to be sold for at least six months, including the holding period of any prior owner other than our affiliates. If such a person has beneficially owned the shares proposed to be sold for at least one year, including the holding period of any prior owner other than our affiliates, then such person is entitled to sell such shares without complying with any of the requirements of Rule 144, subject to the expiration of the lock-up agreements described below.

In general, under Rule 144, as currently in effect, our affiliates or persons selling shares on behalf of our affiliates are entitled to sell shares on expiration of the lock-up agreements described below. Beginning 90 days after the date of this prospectus, within any three-month period, such stockholders may sell a number of shares that does not exceed the greater of:

- 1% of the number of shares of common stock then outstanding, which will equal approximately 407,560 shares immediately after this offering, assuming no exercise of the underwriters' option to purchase additional shares of common stock from us; or
- the average weekly trading volume of our common stock on Nasdaq during the four calendar weeks preceding the filing of a notice on Form 144 with respect to such sale.

Sales under Rule 144 by our affiliates or persons selling shares on behalf of our affiliates are also subject to certain manner of sale provisions and notice requirements and to the availability of current public information about us.

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Rule 701

Rule 701 under the Securities Act ("Rule 701") generally allows a stockholder who was issued shares under a written compensatory plan or contract and who is not deemed to have been an affiliate of our company during the immediately preceding 90 days, to sell these shares in reliance on Rule 144, but without being required to comply with the public information, holding period, volume limitation or notice provisions of Rule 144. Rule 701 also permits affiliates of our company to sell their Rule 701 shares under Rule 144 without complying with the holding period requirements of Rule 144. All holders of Rule 701 shares, however, are required by that rule to wait until 90 days after the date of this prospectus before selling those shares under Rule 701, subject to the expiration of the lock-up agreements described below.

Form S-8 Registration Statements

We intend to file one or more registration statements on Form S-8 under the Securities Act with the SEC to register the offer and sale of shares of our common stock that are issuable under the 2020 Plan, the 2025 Plan and the ESPP. These registration statements will become effective immediately upon filing. Shares covered by these registration statements will then be eligible for sale in the public markets, subject to vesting restrictions, any applicable lock-up agreements described below, and Rule 144 limitations applicable to affiliates.

Lock-Up Arrangements

We, all of our directors and executive officers, and the holders of substantially all of our capital stock and securities convertible into or exchangeable for our capital stock have entered into lock-up agreements with the underwriters and/or are subject to market standoff agreements or other agreements with us, which prevents them from transferring any of our common stock or any securities convertible into or exercisable or exchangeable for common stock for a period of not less than 180 days from the date of this prospectus without the prior written consent of Goldman Sachs & Co. LLC and TD Securities (USA) LLC, subject to certain exceptions. See the section titled "*Underwriting*" appearing elsewhere in this prospectus for more information.

Registration Rights

Upon completion of this offering, certain holders of our securities will be entitled to various rights with respect to registration of their shares under the Securities Act. Registration of these shares under the Securities Act would result in these shares becoming fully tradable without restriction under the Securities Act immediately upon the effectiveness of the registration. See the section titled "*Description of Capital Stock—Registration Rights*" appearing elsewhere in this prospectus for more information.

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MATERIAL U.S. FEDERAL INCOME AND ESTATE TAX CONSEQUENCES FOR NON-U.S. HOLDERS

The following is a summary of material U.S. federal income and estate tax consequences to non-U.S. holders (as defined below) of the purchase, ownership and disposition of our common stock issued pursuant to this offering. This discussion is based on the Internal Revenue Code of 1986, as amended (referred to as the "Code"), Treasury Regulations promulgated thereunder, judicial decisions, and published rulings and administrative pronouncements of the IRS all as in effect on the date hereof. These authorities are subject to differing interpretations and may change, possibly retroactively, resulting in U.S. federal income or estate tax consequences different from those discussed below. We have not requested a ruling from the IRS with respect to the statements made and the conclusions reached in the following summary, and there can be no assurance that the IRS or a court will agree with such statements and conclusions.

This discussion is limited to non-U.S. holders who purchase our common stock pursuant to this offering and who hold our common stock as a "capital asset" within the meaning of Section 1221 of the Code (generally, property held for investment). This discussion is not a complete analysis of all potential U.S. federal income or estate tax consequences relating thereto, does not address the potential application of the Medicare contribution tax on net investment income, the alternative minimum tax, or the special tax accounting rules under Section 451(b) of the Code, and also does not address any U.S. federal non-income tax consequences, such as estate or gift tax consequences, or any tax consequences arising under any state, local or foreign tax laws. This discussion does not address all of the U.S. federal income or estate tax consequences that may be relevant to a non-U.S. holder in light of such non-U.S. holder's particular circumstances. This discussion also does not consider any specific facts or circumstances that may be relevant to non-U.S. holders subject to special rules under the U.S. federal income or estate tax laws, including:

- U.S. expatriates, former citizens or long-term residents of the U.S.;
- partnerships or other entities or arrangements treated as pass-through or disregarded entities for U.S. federal income tax purposes (and investors therein);
- "controlled foreign corporations";
- "passive foreign investment companies";
- corporations that accumulate earnings to avoid U.S. federal income tax;
- banks, financial institutions, investment funds, insurance companies, brokers, dealers or traders in securities;
- tax-exempt organizations and governmental organizations;
- tax-qualified retirement plans;
- persons who acquire our common stock through the exercise of an option or otherwise as compensation;
- qualified foreign pension funds as defined in Section 897(l)(2) of the Code and entities all of the interests of which are held by qualified foreign pension funds;
- persons that elect to apply Section 1400Z-2 of the Code to gains recognized with respect to shares of our common stock;
- persons that own or have owned, actually or constructively, more than 5% of our common stock;
- persons who have elected to mark securities to market; and

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- persons holding our common stock as part of a hedging or conversion transaction or straddle, or synthetic security or a constructive sale, or other risk reduction strategy or integrated investment.

If an entity or arrangement that is classified as a partnership for U.S. federal income tax purposes holds our common stock, the U.S. federal income tax treatment of a partner in the partnership will generally depend on the status of the partner, the activities of the partnership and certain determinations made at the partner level. Partnerships holding our common stock and the partners in such partnerships are urged to consult their tax advisors about the particular U.S. federal income tax consequences to them of holding and disposing of our common stock.

THIS DISCUSSION IS FOR INFORMATIONAL PURPOSES ONLY AND IS NOT TAX ADVICE. PROSPECTIVE INVESTORS SHOULD CONSULT THEIR TAX ADVISORS REGARDING THE PARTICULAR U.S. FEDERAL INCOME TAX CONSEQUENCES TO THEM OF ACQUIRING, OWNING AND DISPOSING OF OUR COMMON STOCK, AS WELL AS ANY TAX CONSEQUENCES ARISING UNDER ANY STATE, LOCAL OR FOREIGN TAX LAWS AND ANY OTHER U.S. FEDERAL TAX LAWS OR UNDER ANY APPLICABLE INCOME TAX TREATY.

Definition of Non-U.S. Holder

For purposes of this discussion, a non-U.S. holder is any beneficial owner of our common stock that is for U.S. federal income tax purposes:

- a non-resident alien individual;
- a corporation or other organization taxable as a corporation for U.S. federal income taxes that is not created or organized under the laws of the U.S., any state thereof, or the District of Columbia; or
- a foreign trust or estate, the income of which is not subject to U.S. federal income tax on a net income basis.

Distributions on Our Common Stock

As described under "*Dividend Policy*," we do not currently anticipate declaring or paying, for the foreseeable future, any cash distributions on our capital stock. However, if we were to distribute cash or other property on our common stock, such distributions will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles.

Subject to the discussion below regarding effectively connected income, backup withholding and FATCA (as defined below), dividends paid to a non-U.S. holder of our common stock generally will be subject to U.S. federal withholding tax at a rate of 30% of the gross amount of the dividends or such lower rate specified by an applicable income tax treaty. Even if our current or accumulated earnings and profits are less than the amount of the distribution, the applicable withholding agent may elect to treat the entire distribution as a dividend for U.S. federal withholding tax purposes. To receive the benefit of a reduced treaty rate, a non-U.S. holder must furnish us or our withholding agent with a valid IRS Form W-8BEN (in the case of individuals) or IRS Form W-8BEN-E (in the case of entities), or other appropriate form, certifying such holder's qualification for the reduced rate. This certification must be provided to us or our withholding agent before the payment of the dividends and must be updated periodically. If the non-U.S. holder holds our common stock through a financial institution or other agent acting on the non-U.S. holder's behalf, the non-U.S. holder will be required to provide appropriate documentation to the agent, which then will be required to provide certification to us or our withholding agent, either directly or through other intermediaries.

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Non-U.S. holders that do not provide the required certification on a timely basis, but that qualify for a reduced treaty rate, may obtain a refund or credit of any excess amounts withheld by timely filing an appropriate claim with the IRS. Non-U.S. holders should consult their tax advisors regarding any applicable income tax treaties that may provide for different rules.

If a non-U.S. holder holds our common stock in connection with the conduct of a trade or business in the U.S., and dividends paid on our common stock are effectively connected with such holder's U.S. trade or business (and are attributable to such holder's permanent establishment or fixed base in the U.S. if required by an applicable tax treaty), the non-U.S. holder generally will be exempt from U.S. federal withholding tax. To claim the exemption, the non-U.S. holder must generally furnish a valid IRS Form W-8ECI (or applicable successor form) to the applicable withholding agent. However, any such effectively connected dividends paid on our common stock generally will be subject to U.S. federal income tax on a net income basis at the regular U.S. federal income tax rates in the same manner as if such holder were a resident of the U.S. A non-U.S. holder that is a foreign corporation also may be subject to an additional branch profits tax equal to 30% (or such lower rate specified by an applicable income tax treaty) of its effectively connected dividends, as adjusted for certain items.

Amounts not treated as dividends for U.S. federal income tax purposes will constitute a return of capital and will first be applied against and reduce a holder's tax basis in our common stock, but not below zero. Any excess will be treated as gain realized on the sale or other disposition of our common stock and will be treated as described under "*—Gain on Sale or Other Taxable Disposition of Our Common Stock*" below.

Gain on Sale or Other Taxable Disposition of Our Common Stock

Subject to the discussion below regarding backup withholding and FATCA (as defined below), a non-U.S. holder generally will not be subject to U.S. federal income tax on any gain realized on the sale or other taxable disposition of our common stock, unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a trade or business in the U.S. and, if required by an applicable income tax treaty, is attributable to a permanent establishment or fixed base maintained by the non-U.S. holder in the U.S.;
- the non-U.S. holder is a nonresident alien individual who is present in the U.S. for a period or periods aggregating 183 days or more during the taxable year of the disposition, and certain other requirements are met; or
- our common stock constitutes a "United States real property interest" by reason of our status as a U.S. real property holding corporation ("USRPHC") for U.S. federal income tax purposes at any time within the shorter of the five-year period preceding the disposition or the non-U.S. holder's holding period for our common stock, and our common stock is not "regularly traded" on an established securities market during the calendar year in which the sale or other disposition occurs.

Gain described in the first bullet point above generally will be subject to U.S. federal income tax on a net income basis at the regular U.S. federal income tax rates in the same manner as if such holder were a resident of the U.S. A non-U.S. holder that is a foreign corporation also may be subject to an additional branch profits tax equal to 30% (or such lower rate specified by an applicable income tax treaty) of its effectively connected earnings and profits for the taxable year, as adjusted for certain items.

Gain described in the second bullet point above will be subject to U.S. federal income tax at a flat 30% rate (or such lower rate specified by an applicable income tax treaty), but may be offset by certain

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U.S.-source capital losses (even though the individual is not considered a resident of the U.S.), provided that the non-U.S. holder has timely filed U.S. federal income tax returns with respect to such losses.

Determining whether we are a USRPHC depends on the fair market value of our U.S. real property interests relative to the fair market value of our worldwide real property interests and our other trade or business assets. We believe that we are not currently and we do not anticipate becoming a USRPHC for U.S. federal income tax purposes, although there can be no assurance we will not in the future become a USRPHC. Even if we are treated as a USRPHC, gain realized by a non-U.S. holder on a disposition of our common stock will not be subject to U.S. federal income tax so long as (1) the non-U.S. holder owned, directly, indirectly and constructively, no more than 5% of our common stock at all times within the shorter of (a) the five-year period preceding the disposition or (b) the holder's holding period and (2) our common stock is "regularly traded" on an established securities market within the meaning of applicable U.S. Treasury regulations. There can be no assurance that our common stock qualifies as regularly traded on an established securities market for purposes of the rules described above.

Non-U.S. holders should consult their tax advisors regarding any applicable income tax treaties that may provide for different rules.

Federal Estate Tax

Common stock owned or treated as owned by an individual who is not a U.S. citizen or resident (as specifically determined for U.S. federal estate tax purposes) at the time of the individual's death will be included in the individual's gross estate for U.S. federal estate tax purposes, unless an applicable estate tax treaty provides otherwise.

Information Reporting and Backup Withholding

Annual reports are required to be filed with the IRS and provided to each non-U.S. holder indicating the amount of distributions on our common stock paid to such holder and the amount of any tax withheld with respect to those distributions. These information reporting requirements apply even if no withholding was required because the distributions were effectively connected with the holder's conduct of a U.S. trade or business, or withholding was reduced or eliminated by an applicable income tax treaty. This information also may be made available under a specific treaty or agreement with the tax authorities in the country in which the non-U.S. holder resides or is established. Backup withholding, currently at a 24% rate, generally will not apply to payments to a non-U.S. holder of distributions on or the gross proceeds of a disposition of our common stock provided the non-U.S. holder furnishes the required certification for its non-U.S. status, such as by providing a valid IRS Form W-8BEN, IRS Form W-8BEN-E or IRS Form W-8ECI, or otherwise establishes an exemption, and if the payor does not have actual knowledge, or reason to know, that the holder is a U.S. person who is not an exempt recipient.

Backup withholding is not an additional tax. If any amount is withheld under the backup withholding rules, the non-U.S. holder should consult with a U.S. tax advisor regarding the possibility of and procedure for obtaining a refund or a credit against the non-U.S. holder's U.S. federal income tax liability, if any.

Withholding on Foreign Entities

Sections 1471 through 1474 of the Code, which are commonly referred to as FATCA, impose a U.S. federal withholding tax of 30% on certain payments made to a "foreign financial institution" (as

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specially defined under these rules) unless such institution enters into an agreement with the U.S. government to withhold on certain payments and to collect and provide to the U.S. tax authorities substantial information regarding certain U.S. account holders of such institution (which includes certain equity and debt holders of such institution, as well as certain account holders that are foreign entities with U.S. owners) or an exemption applies. FATCA also generally imposes a U.S. federal withholding tax of 30% on certain payments made to a "non-financial foreign entity" (as specially defined under these rules) unless such entity provides the withholding agent a certification that it does not have any "substantial United States owners" or provides information identifying certain direct and indirect U.S. owners of the entity or an exemption applies. An intergovernmental agreement between the U.S. and an applicable foreign country may modify these requirements. Under certain circumstances, a non-U.S. holder might be eligible for refunds or credits of such taxes. FATCA currently applies to dividends paid on our common stock and would have applied also to payments of gross proceeds from the sale or other disposition of our common stock. However, proposed regulations under FATCA provide for the elimination of the federal withholding tax of 30% applicable to gross proceeds of a sale or other disposition of from property of a type that can produce U.S. source dividends or interest. Under these proposed Treasury Regulations (which may be relied upon by taxpayers prior to finalization), FATCA withholding does not apply to gross proceeds from sales or other dispositions of our common stock.

Prospective investors are encouraged to consult with their tax advisors regarding the possible implications of FATCA on their investment in our common stock.

EACH PROSPECTIVE INVESTOR SHOULD CONSULT ITS TAX ADVISOR REGARDING THE TAX CONSEQUENCES OF PURCHASING, HOLDING AND DISPOSING OF OUR COMMON STOCK, INCLUDING THE CONSEQUENCES OF ANY RECENT AND PROPOSED CHANGE IN APPLICABLE LAW, AS WELL AS TAX CONSEQUENCES ARISING UNDER ANY STATE, LOCAL, NON-U.S. OR U.S. FEDERAL NON-INCOME TAX LAWS.

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UNDERWRITING

The company and the underwriters named below have entered into an underwriting agreement with respect to the shares being offered. Subject to certain conditions, each underwriter has severally agreed to purchase the number of shares indicated in the following table. Goldman Sachs & Co. LLC, TD Securities (USA) LLC, Stifel, Nicolaus & Company, Incorporated and Guggenheim Securities, LLC are the representatives of the underwriters.

Underwriters	Number of Shares
Goldman Sachs & Co. LLC	4,235,293
TD Securities (USA) LLC	2,858,823
Stifel, Nicolaus & Company, Incorporated	1,905,882
Guggenheim Securities, LLC	1,588,235
Total	10,588,233

The underwriters are committed to take and pay for all of the shares being offered, if any are taken, other than the shares covered by the option described below unless and until this option is exercised.

The underwriters have an option to buy up to an additional 1,588,234 shares from the company to cover sales by the underwriters of a greater number of shares than the total number set forth in the table above. The underwriters may exercise that option for 30 days from the date of this prospectus. If any shares are purchased pursuant to this option, the underwriters will severally purchase shares in approximately the same proportion as set forth in the table above.

The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters by the company. Such amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase 1,588,234 additional shares.

Paid by the Company

	No Exercise	Full Exercise
Per Share	$ 1.26	$ 1.26
Total	$ 13,341,174	$ 15,342,348

Shares sold by the underwriters to the public will initially be offered at the initial public offering price set forth on the cover of this prospectus. Any shares sold by the underwriters to securities dealers may be sold at a discount of up to $0.756 per share from the initial public offering price. After the initial offering of the shares, the representatives may change the offering price and the other selling terms. The offering of the shares by the underwriters is subject to receipt and acceptance and subject to the underwriters' right to reject any order in whole or in part.

The company and its officers, directors and holders of substantially all of the company's common stock have agreed with the underwriters, subject to certain exceptions, not to dispose of or hedge any of their common stock or securities convertible into or exchangeable for shares of common stock (the "Lock-Up Securities") during the period from the date of this prospectus continuing through the date 180 days after the date of this prospectus, except with the prior written consent of Goldman Sachs & Co. LLC and TD Securities (USA) LLC. See section titled "*Shares Eligible for Future Sale*" for a discussion of certain transfer restrictions.

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The restrictions described in the immediately preceding paragraph do not apply to our officers, directors and holders of substantially all of our capital stock and securities convertible into or exchangeable for our common stock with respect to:

Transfers of Lock-Up Securities (i) as one or more bona fide gifts or charitable contributions, or for bona fide estate planning purposes, (ii) upon death by will, testamentary document or intestate succession, (iii) if the lock-up party is a natural person, to any member of the lock-up party's immediate family or to any trust for the direct or indirect benefit of the lock-up party or the family of the lock-up party or, if the lock-up party is a trust, to a trustor, trustee (or co-trustee) or beneficiary of the trust or the estate of a beneficiary of such trust, (iv) to a corporation, partnership, limited liability company or other entity of which the lock-up party and the immediate family of the lock-up party are the legal and beneficial owner of all of the outstanding equity securities or similar interests, (v) to a nominee or custodian of a person or entity to whom a disposition or transfer would be permissible under clauses (i) through (iv) above, (vi) if the lock-up party is a corporation, partnership, limited liability company, trust or other business entity, (A) to another corporation, partnership, limited liability company, trust or other business entity that is a subsidiary or an affiliate (as defined in Rule 405 under the Securities Act) of the lock-up party, or to any investment fund or other entity which fund or entity is controlled or managed by or under common control or common investment management of the lock-up party or affiliates of the lock-up party or the immediate family of the lock-up party, or (B) as part of a disposition, transfer or distribution by the lock-up party to its stockholders, partners, members or other equityholders or to the estate of any such stockholders, partners, members or other equityholders, (vii) by operation of law, such as pursuant to a merger, acquisition, reorganization, qualified domestic order, divorce settlement, divorce decree or separation agreement, (viii) to the company (A) pursuant to any contractual arrangement in effect on the date of the lock-up agreement and described in this prospectus, the preliminary prospectus relating to the shares being offered in this prospectus immediately prior to the time the underwriting agreement is executed and the prospectus that provides for the repurchase of the lock-up party's common stock or other securities by the company or (B) from an employee of the company upon death, disability or termination of employment, in each case, of such employee, (ix) if the lock-up party is not an officer or director of the company, in connection with a sale of the lock-up party's shares of common stock acquired (A) from the underwriters in this offering or (B) in open market transactions after the closing date of this offering, (x) to the company in connection with the vesting, settlement or exercise of restricted stock units, options, warrants or other rights to purchase shares of common stock (including, in each case, by way of "net" or "cashless" exercise) that are scheduled to expire or automatically vest during the lock-up period, including any transfer to the company for the payment of tax withholdings or remittance payments due as a result of the vesting, settlement or exercise of such restricted stock units, options, warrants or other rights, or in connection with the conversion of convertible securities, in all such cases pursuant to equity awards granted under a stock incentive plan or other equity award plan, or pursuant to the terms of convertible securities, each as described in the prospectus, provided that any securities received upon such vesting, settlement, exercise or conversion shall be subject to the terms of the lock-up agreement or (xi) with the prior written consent of Goldman Sachs & Co. LLC and TD Securities (USA) LLC on behalf of the underwriters; provided that (A) in the case of clauses (i), (ii), (iii), (iv), (v) and (vi) above, such transfer or distribution shall not involve a disposition for value, (B) in the case of clauses (i), (ii), (iii), (iv), (v), (vi) and (vii) above, it shall be a condition to the transfer or distribution that the donee, devisee, transferee or distributee, as the case may be, shall sign and deliver a lock-up agreement in the form of the lock-up agreement, (C) in the case of clauses (ii), (iii), (iv), (v) and (vi) above, no filing by any party (including, without limitation, any donor, donee, devisee, transferor, transferee, distributor or distributee) under the Exchange Act, or other public filing, report or announcement reporting a reduction in beneficial ownership of Lock-Up Securities shall be required or shall be voluntarily made in connection with such transfer or distribution, and (D) in the case of clauses (i), (vii), (viii), (ix) and (x) above, no filing under the Exchange Act or other public filing, report or announcement shall be voluntarily made, and if any such filing, report or announcement shall be legally required during the lock-up period, such filing,

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report or announcement shall clearly indicate in the footnotes thereto (A) the circumstances of such transfer or distribution and (B) in the case of a transfer or distribution pursuant to clauses (a)(i) or (vii) above, that the donee, devisee, transferee or distributee has agreed to be bound by a lock-up agreement containing the same restrictions set forth above.

In addition, the lock-up party may (a) enter into a written plan meeting the requirements of Rule 10b5-1 under the Exchange Act relating to the transfer, sale or other disposition of the lock-up party's Lock-Up Securities, if then permitted by the company, provided that none of the securities subject to such plan may be transferred, sold or otherwise disposed of until after the expiration of the lock-up period and no public announcement, report or filing under the Exchange Act, or any other public filing, report or announcement, shall be voluntarily made regarding the establishment of such plan during the lock-up period, and if any such filing, report or announcement shall be legally required during the lock-up period, such filing, report or announcement shall clearly indicate in the footnotes thereto that that none of the securities subject to such plan may be transferred, sold or otherwise disposed of pursuant to such plan until after the expiration of the lock-up period; (b)(i) transfer the lock-up party's Lock-Up Securities pursuant to a bona fide third-party tender offer, merger, consolidation or other similar transaction that is approved by the board of directors of the company and made to all holders of the company's capital stock involving a change of control of the company, in one transaction or a series of related transactions, to a person or group of affiliated persons, of shares of capital stock if, after such transfer, such person or group of affiliated persons would hold at least a majority of the outstanding voting securities of the company (or the surviving entity) and (ii) enter into any lock-up, voting or similar agreement pursuant to which the lock-up party may agree to transfer, sell, tender or otherwise dispose of common stock or such other securities in connection with a transaction described in clause (b)(i) above; provided that in the event that such tender offer, merger, consolidation or other similar transaction is not completed, the lock-up party's Lock-Up Securities shall remain subject to the provisions of the lock-up agreement; and (c) convert outstanding preferred stock of the company into shares of common stock, provided that any such shares received upon such conversion shall remain subject to the provisions of the lock-up agreement.

The restrictions on transfers or other dispositions by us described above do not apply to us with respect to (i) the shares to be sold in this offering, (ii) shares of common stock or any securities convertible into, or exercisable for, shares of common stock (including, without limitation, options, warrants, restricted stock or restricted stock units) outstanding as of the date of this prospectus and described in this prospectus, (iii) the grant of any shares of common stock or any securities convertible into, or exercisable for, shares of common stock (including, without limitation, options, warrants, restricted stock or restricted stock units) pursuant to employee equity-based compensation plans, incentive plans, stock plans, dividend reinvestment plans or other arrangements in place as of the date of this prospectus and described in this prospectus, (iv) the filing of a registration statement on Form S-8 in connection with the registration of securities issuable under any employee equity-based compensation plan, incentive plan, stock plan or dividend reinvestment plan adopted and approved by the company's board of directors prior to the date of this prospectus and as described in this prospectus and (v) shares of common stock or other securities issued in connection with any bona fide merger, joint venture, strategic alliance, commercial or other collaborative transaction, or the acquisition or license by the company of the business, property, technology or other assets of another individual or entity that is an unaffiliated third party of the company, or the assumption of an employee benefit plan in connection with such a merger or acquisition. In the case of clause (v), the aggregate number of shares of our common stock that we may sell or issue or agree to sell or issue may not exceed 5.0% of the total number of shares of our common stock issued and outstanding immediately following this offering, and each recipient of any such shares of our common stock issued pursuant to clause (v) during such period must execute and deliver to the representatives an agreement having substantially the same terms as those agreements governing the Lock-Up Securities described above prior to, or concurrently with, such issuance or sale.

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Prior to the offering, there has been no public market for the shares. The initial public offering price has been negotiated among the company and the representatives. Among the factors to be considered in determining the initial public offering price of the shares, in addition to prevailing market conditions, will be the company's historical performance, estimates of the business potential and earnings prospects of the company, an assessment of the company's management and the consideration of the above factors in relation to market valuation of companies in related businesses.

We have been approved to list the common stock on The Nasdaq Global Market under the symbol "SION."

In connection with the offering, the underwriters may purchase and sell shares of common stock in the open market. These transactions may include short sales, stabilizing transactions and purchases to cover positions created by short sales. Short sales involve the sale by the underwriters of a greater number of shares than they are required to purchase in the offering, and a short position represents the amount of such sales that have not been covered by subsequent purchases. A "covered short position" is a short position that is not greater than the amount of additional shares for which the underwriters' option described above may be exercised. The underwriters may cover any covered short position by either exercising their option to purchase additional shares or purchasing shares in the open market. In determining the source of shares to cover the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase additional shares pursuant to the option described above. "Naked" short sales are any short sales that create a short position greater than the amount of additional shares for which the option described above may be exercised. The underwriters must cover any such naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market after pricing that could adversely affect investors who purchase in the offering. Stabilizing transactions consist of various bids for or purchases of common stock made by the underwriters in the open market prior to the completion of the offering.

The underwriters may also impose a penalty bid. This occurs when a particular underwriter repays to the underwriters a portion of the underwriting discount received by it because the representatives have repurchased shares sold by or for the account of such underwriter in stabilizing or short covering transactions.

Purchases to cover a short position and stabilizing transactions, as well as other purchases by the underwriters for their own accounts, may have the effect of preventing or retarding a decline in the market price of the company's stock, and together with the imposition of the penalty bid, may stabilize, maintain or otherwise affect the market price of the common stock. As a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. The underwriters are not required to engage in these activities and may end any of these activities at any time. These transactions may be effected on Nasdaq, in the over-the-counter market or otherwise.

The company estimates that their share of the total expenses of the offering, including registration, filing and listing fees, printing fees and legal and accounting expenses, but excluding underwriting discounts and commissions, will be approximately $4.2 million. We have agreed to reimburse the underwriters for certain of their expenses in an amount up to $40,000.

The company has agreed to indemnify the several underwriters against certain liabilities, including liabilities under the Securities Act.

The underwriters and their respective affiliates are full service financial institutions engaged in various activities, which may include sales and trading, commercial and investment banking, advisory,

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investment management, investment research, principal investment, hedging, market making, brokerage and other financial and non-financial activities and services. Certain of the underwriters and their respective affiliates have provided, and may in the future provide, a variety of these services to the issuer and to persons and entities with relationships with the issuer, for which they received or will receive customary fees and expenses.

In the ordinary course of their various business activities, the underwriters and their respective affiliates, officers, directors and employees may purchase, sell or hold a broad array of investments and actively trade securities, derivatives, loans, commodities, currencies, credit default swaps and other financial instruments for their own account and for the accounts of their customers, and such investment and trading activities may involve or relate to assets, securities and/or instruments of the issuer (directly, as collateral securing other obligations or otherwise) and/or persons and entities with relationships with the issuer. The underwriters and their respective affiliates may also communicate independent investment recommendations, market color or trading ideas and/or publish or express independent research views in respect of such assets, securities or instruments and may at any time hold, or recommend to clients that they should acquire, long and/or short positions in such assets, securities and instruments.

Selling Restrictions

European Economic Area

In relation to each Member State of the European Economic Area (each, a "Relevant Member"), no shares have been offered or will be offered pursuant to the offering to the public in that Relevant Member prior to the publication of a prospectus in relation to the shares which has been approved by the competent authority in that Relevant Member or, where appropriate, approved in another Relevant Member and notified to the competent authority in that Relevant Member, all in accordance with the Prospectus Regulation, except that the shares may be offered to the public in that Relevant Member at any time:

(i) to any legal entity which is a qualified investor as defined under Article 2 of the Prospectus Regulation;

(ii) to fewer than 150 natural or legal persons (other than qualified investors as defined under Article 2 of the Prospectus Regulation), subject to obtaining the prior consent of the representatives for any such offer; or

(iii) in any other circumstances falling within Article 1(4) of the Prospectus Regulation,

provided that no such offer of the shares shall require us or any of the representatives to publish a prospectus pursuant to Article 3 of the Prospectus Regulation or supplement a prospectus pursuant to Article 23 of the Prospectus Regulation.

For the purposes of this provision, the expression an "offer to the public" in relation to the shares in any Relevant Member means the communication in any form and by any means of sufficient information on the terms of the offer and any shares to be offered so as to enable an investor to decide to purchase or subscribe for any shares, and the expression "Prospectus Regulation" means Regulation (EU) 2017/1129.

United Kingdom

No shares have been offered or will be offered pursuant to the offering to the public in the United Kingdom prior to the publication of a prospectus in relation to the shares which has been approved by

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the Financial Conduct Authority, except that the shares may be offered to the public in the United Kingdom at any time:

(i) to any legal entity which is a qualified investor as defined under Article 2 of the UK Prospectus Regulation;

(ii) to fewer than 150 natural or legal persons (other than qualified investors as defined under Article 2 of the UK Prospectus Regulation), subject to obtaining the prior consent of the representatives for any such offer; or

(iii) in any other circumstances falling within Section 86 of the FSMA,

provided that no such offer of the shares shall require the Issuer or Manager to publish a prospectus pursuant to Section 85 of the FSMA or supplement a prospectus pursuant to Article 23 of the UK Prospectus Regulation. For the purposes of this provision, the expression an "offer to the public" in relation to the shares in the United Kingdom means the communication in any form and by any means of sufficient information on the terms of the offer and any shares to be offered so as to enable an investor to decide to purchase or subscribe for any shares and the expression. "UK Prospectus Regulation" means Regulation (EU) 2017/1129 as it forms part of domestic law by virtue of the European Union (Withdrawal) Act 2018.

Canada

The securities may be sold in Canada only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions, and Ongoing Registrant Obligations. Any resale of the securities must be made in accordance with an exemption form, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this offering memorandum (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

Hong Kong

The shares may not be offered or sold in Hong Kong by means of any document other than (i) in circumstances which do not constitute an offer to the public within the meaning of the Companies (Winding Up and Miscellaneous Provisions) Ordinance (Cap. 32 of the Laws of Hong Kong) ("Companies (Winding Up and Miscellaneous Provisions) Ordinance") or which do not constitute an invitation to the public within the meaning of the Securities and Futures Ordinance (Cap. 571 of the Laws of Hong Kong) ("Securities and Futures Ordinance"), or (ii) to "professional investors" as defined in the Securities and Futures Ordinance and any rules made thereunder, or (iii) in other circumstances which do not result in the document being a "prospectus" as defined in the Companies (Winding Up and Miscellaneous Provisions) Ordinance, and no advertisement, invitation or document relating to the shares may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or elsewhere), which is directed at, or the contents of which are likely to be

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accessed or read by, the public in Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to shares which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" in Hong Kong as defined in the Securities and Futures Ordinance and any rules made thereunder.

Singapore

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares may not be circulated or distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor (as defined under Section 4A of the Securities and Futures Act, Chapter 289 of Singapore (the "SFA")) under Section 274 of the SFA, (ii) to a relevant person (as defined in Section 275(2) of the SFA) pursuant to Section 275(1) of the SFA, or any person pursuant to Section 275(1A) of the SFA, and in accordance with the conditions specified in Section 275 of the SFA or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA, in each case subject to conditions set forth in the SFA.

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor, the securities (as defined in Section 239(1) of the SFA) of that corporation shall not be transferable for 6 months after that corporation has acquired the shares under Section 275 of the SFA except: (1) to an institutional investor under Section 274 of the SFA or to a relevant person (as defined in Section 275(2) of the SFA), (2) where such transfer arises from an offer in that corporation's securities pursuant to Section 275(1A) of the SFA, (3) where no consideration is or will be given for the transfer, (4) where the transfer is by operation of law, (5) as specified in Section 276(7) of the SFA or (6) as specified in Regulation 32 of the Securities and Futures (Offers of Investments) (Shares and Debentures) Regulations 2005 of Singapore ("Regulation 32").

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is a trust (where the trustee is not an accredited investor (as defined in Section 4A of the SFA)) whose sole purpose is to hold investments and each beneficiary of the trust is an accredited investor, the beneficiaries' rights and interest (howsoever described) in that trust shall not be transferable for 6 months after that trust has acquired the shares under Section 275 of the SFA except: (1) to an institutional investor under Section 274 of the SFA or to a relevant person (as defined in Section 275(2) of the SFA), (2) where such transfer arises from an offer that is made on terms that such rights or interest are acquired at a consideration of not less than S$200,000 (or its equivalent in a foreign currency) for each transaction (whether such amount is to be paid for in cash or by exchange of securities or other assets), (3) where no consideration is or will be given for the transfer, (4) where the transfer is by operation of law, (5) as specified in Section 276(7) of the SFA or (6) as specified in Regulation 32.

Solely for the purposes of its obligations pursuant to Section 309B of the SFA, we have determined, and hereby notify all relevant persons (as defined in the CMP Regulations 2018), that the shares are "prescribed capital markets products" (as defined in the CMP Regulations 2018) and Excluded Investment Products (as defined in MAS Notice SFA 04-N12: Notice on the Sale of Investment Products and MAS Notice FAA-N16: Notice on Recommendations on Investment Products).

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Japan

The securities have not been and will not be registered under the Financial Instruments and Exchange Act of Japan (Act No. 25 of 1948, as amended) ("the FIEA"). The securities may not be offered or sold, directly or indirectly, in Japan or to or for the benefit of any resident of Japan (including any person resident in Japan or any corporation or other entity organized under the laws of Japan) or to others for reoffering or resale, directly or indirectly, in Japan or to or for the benefit of any resident of Japan, except pursuant to an exemption from the registration requirements of the FIEA and otherwise in compliance with any relevant laws and regulations of Japan.

Australia

No placement document, prospectus, product disclosure statement or other disclosure document has been lodged with the Australian Securities and Investments Commission, in relation to the offering. This offering document does not constitute a prospectus, product disclosure statement or other disclosure document under the Corporations Act 2001 (the "Corporations Act"), and does not purport to include the information required for a prospectus, product disclosure statement or other disclosure document under the Corporations Act.

Any offer in Australia of the shares may only be made to persons ("Exempt Investors") who are "sophisticated investors" (within the meaning of Section 708(8) of the Corporations Act), "professional investors" (within the meaning of Section 708(11) of the Corporations Act) or otherwise pursuant to one or more exemptions contained in Section 708 of the Corporations Act so that it is lawful to offer the shares without disclosure to investors under Chapter 6D of the Corporations Act.

The shares applied for by Exempt Investors in Australia must not be offered for sale in Australia in the period of 12 months after the date of allotment under the offering, except in circumstances where disclosure to investors under Chapter 6D of the Corporations Act would not be required pursuant to an exemption under Section 708 of the Corporations Act or otherwise or where the offer is pursuant to a disclosure document which complies with Chapter 6D of the Corporations Act. Any person acquiring shares must observe such Australian on-sale restrictions.

This offering document contains general information only and does not take account of the investment objectives, financial situation or particular needs of any particular person. It does not contain any securities recommendations or financial product advice. Before making an investment decision, investors need to consider whether the information in this offering document is appropriate to their needs, objectives and circumstances, and, if necessary, seek expert advice on those matters.

Dubai International Financial Centre

This offering document relates to an Exempt Offer in accordance with the Offered Securities Rules of the Dubai Financial Services Authority ("DFSA"). This offering document is intended for distribution only to persons of a type specified in the Offered Securities Rules of the DFSA. It must not be delivered to, or relied on by, any other person. The DFSA has no responsibility for reviewing or verifying any documents in connection with Exempt Offers. The DFSA has not approved this prospectus nor taken steps to verify the information set forth in this prospectus and has no responsibility for the offering document. The securities to which this offering document relates may be illiquid and/or subject to restrictions on their resale. Prospective purchasers of the securities offered should conduct their own due diligence on the securities. If you do not understand the contents of this offering document you should consult an authorized financial advisor.

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Switzerland

This offering document is not intended to constitute an offer or solicitation to purchase or invest in the shares of our common stock. The shares of common stock may not be publicly offered, directly or indirectly, in Switzerland within the meaning of the Swiss Financial Services Act ("FinSA"), and no application has or will be made to admit the shares of common stock to trading on any trading venue (exchange or multilateral trading facility) in Switzerland. Neither this offering document nor any other offering or marketing material relating to the shares of common stock constitutes a prospectus pursuant to the FinSA, and neither this offering document nor any other offering or marketing material relating to the shares of common stock may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this offering document nor any other offering or marketing material relating to the offering, us or the securities have been or will be filed with or approved by any Swiss regulatory authority. In particular, this offering document will not be filed with, and the offer of securities will not be supervised by, the Swiss Financial Market Supervisory Authority, and the offer of securities has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes ("CISA"). The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of securities.

Brazil

The offer and sale of the securities have not been and will not be registered with the Brazilian Securities Commission (Comissão de Valores Mobiliários, or "CVM") and, therefore, will not be carried out by any means that would constitute a public offering in Brazil under CVM Resolution No 160, dated 13 July 2022, as amended ("CVM Resolution 160") or unauthorized distribution under Brazilian laws and regulations. The securities may only be offered to Brazilian professional investors (as defined by applicable CVM regulation), who may only acquire the securities through a non-Brazilian account, with settlement outside Brazil in non-Brazilian currency. the trading of these securities on regulated securities markets in Brazil is prohibited.

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LEGAL MATTERS

The validity of the shares of our common stock being offered in this prospectus will be passed upon for us by Goodwin Procter LLP, Boston, Massachusetts. Ropes & Gray LLP, Boston, Massachusetts is representing the underwriters in this offering.

EXPERTS

The financial statements of Sionna Therapeutics, Inc. as of December 31, 2023 and 2022, and for the years then ended included in this prospectus, have been audited by Deloitte & Touche LLP, an independent registered public accounting firm, as stated in their report. Such financial statements are included in reliance upon the report of such firm given their authority as experts in accounting and auditing.

WHERE YOU CAN FIND ADDITIONAL INFORMATION

We have filed with the SEC a registration statement on Form S-1 (File Number 333-284352) under the Securities Act with respect to the shares of common stock offered by this prospectus. This prospectus, which constitutes a part of the registration statement, does not contain all the information set forth in the registration statement, some of which is contained in exhibits to the registration statement as permitted by the rules and regulations of the SEC. For further information with respect to us and our common stock, we refer you to the registration statement, including the exhibits filed as a part of the registration statement. Statements contained in this prospectus concerning the contents of any contract or any other document are not necessarily complete. If a contract or document has been filed as an exhibit to the registration statement, please see the copy of the contract or document that has been filed. Each statement in this prospectus relating to a contract or document filed as an exhibit is qualified in all respects by the filed exhibit. The SEC also maintains an internet website that contains reports and other information about issuers, like us, that file electronically with the SEC. The address of that website is *www.sec.gov*.

We currently do not file periodic reports with the SEC. On the completion of this offering, we will be subject to the information reporting requirements of the Exchange Act, and we will file reports, proxy statements and other information with the SEC. These reports, proxy statements and other information will be available for review at the website of the SEC referred to above.

We also maintain a website at *www.sionnatx.com*. Information contained in, or accessible through, our website is not a part of this prospectus, and the inclusion of our website address in this prospectus is only as an inactive textual reference. Upon completion of this offering, you may access, free of charge, our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and amendment to those reported filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC.

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Consolidated Financial Statements for the Years Ended December 31, 2023 and 2022:

Unaudited Interim Condensed Consolidated Financial Statements for the Nine Months Ended September 30, 2024 and 2023:

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the stockholders and the Board of Directors of Sionna Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Sionna Therapeutics, Inc., and subsidiary (the "Company") as of December 31, 2023 and 2022, the related consolidated statements of operations and comprehensive loss, convertible preferred stock and stockholders' deficit, and cash flows, for the years then ended, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2023 and 2022, and the results of its operations and its cash flows for the years then ended in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits, we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Deloitte & Touche LLP

Boston, Massachusetts

September 12, 2024 (February 3, 2025, as to the effects of the reverse stock split discussed in Note 16)

We have served as the Company's auditor since 2021.

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SIONNA THERAPEUTICS, INC.
CONSOLIDATED BALANCE SHEETS
(in thousands, except share and per share data)

	Year Ended December 31, 2023	2022
Assets		
Current assets:		
Cash and cash equivalents	$ 38,521	$ 54,837
Marketable securities	—	27,773
Prepaid expenses and other current assets	686	1,223
Total current assets	39,207	83,833
Property and equipment, net	3,107	2,574
Restricted cash	962	962
Operating lease right-of-use asset	8,625	—
Other assets	44	—
Total assets	$ 51,945	$ 87,369
Liabilities, convertible preferred stock and stockholders' deficit		
Current liabilities:		
Accounts payable	$ 719	$ 557
Accrued expenses and other current liabilities	6,590	7,696
Operating lease liability, current	728	—
Total current liabilities	8,037	8,253
Operating lease liability, noncurrent	9,749	—
Total liabilities	17,786	8,253
Commitments and contingencies (Note 8)		
Series Seed convertible preferred stock, $0.001 par value, 3,963,963 shares authorized, issued and outstanding as of December 31, 2023 and 2022, liquidation value of $13,200 as of December 31, 2023 and 2022	13,014	13,014
Series A convertible preferred stock, $0.001 par value, 5,704,161 shares authorized, issued and outstanding as of December 31, 2023 and 2022; liquidation value of $25,263 as of December 31, 2023 and 2022	25,235	25,235
Series B convertible preferred stock, $0.001 par value, 11,370,621 shares authorized, issued and outstanding as of December 31, 2023 and 2022; liquidation value of $111,000 as of December 31, 2023 and 2022	110,791	110,791
Total convertible preferred stock	149,040	149,040
Stockholders' deficit:		
Common stock, $0.001 par value, 30,000,000 shares authorized as of December 31, 2023 and 2022, respectively; 3,313,012 and 3,277,460 shares issued, 3,050,863 and 2,792,929 shares outstanding as of December 31, 2023 and 2022, respectively	2	2
Additional paid-in capital	4,515	2,225
Accumulated other comprehensive loss	—	(16)
Accumulated deficit	(119,398)	(72,135)
Total stockholders' deficit	(114,881)	(69,924)
Total liabilities, convertible preferred stock and stockholders' deficit	$ 51,945	$ 87,369

The accompanying notes are an integral part of these consolidated financial statements.

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SIONNA THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except share and per share data)

	Year Ended December 31,	
	2023	2022
Operating expenses:		
Research and development	$ 40,626	$ 34,605
General and administrative	9,707	6,767
Total operating expenses	50,333	41,372
Loss from operations	(50,333)	(41,372)
Other income:		
Interest income	2,769	1,132
Other income	301	—
Total other income	3,070	1,132
Net loss	$ (47,263)	$ (40,240)
Net loss per share—basic and diluted	$ (16.11)	$ (15.61)
Weighted-average common shares outstanding, basic and diluted	2,933,218	2,577,544
Comprehensive loss:		
Net loss	$ (47,263)	$ (40,240)
Other comprehensive income:		
Unrealized gain (loss) on marketable debt securities	16	(16)
Comprehensive loss	$ (47,247)	$ (40,256)

The accompanying notes are an integral part of these consolidated financial statements.

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SIONNA THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' DEFICIT
(in thousands, except share amounts)

	Series Seed Preferred Stock		Series A Preferred Stock		Series B Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount				
Balance as of December 31, 2021	3,963,963	$13,014	5,704,161	$25,235	—	$ —	2,370,559	$ 1	$ 475	$ —	$ (31,895)	$ (31,419)
Issuance of Series B Preferred Stock, net of issuance costs of $0.2 million	—	—	—	—	11,370,621	110,791	—	—	—	—	—	—
Exercise of common stock options	—	—	—	—	—	—	83,368	—	64	—	—	64
Vesting of restricted stock awards	—	—	—	—	—	—	339,002	1	—	—	—	1
Stock-based compensation expense	—	—	—	—	—	—	—	—	1,686	—	—	1,686
Unrealized loss on marketable debt securities	—	—	—	—	—	—	—	—	—	(16)	—	(16)
Net loss	—	—	—	—	—	—	—	—	—	—	(40,240)	(40,240)
Balance as of December 31, 2022	3,963,963	$13,014	5,704,161	$25,235	11,370,621	$110,791	2,792,929	$ 2	$ 2,225	$ (16)	$ (72,135)	$ (69,924)
Exercise of common stock options	—	—	—	—	—	—	35,552	—	31	—	—	31
Vesting of restricted stock awards	—	—	—	—	—	—	222,382	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	2,259	—	—	2,259
Unrealized gain on marketable debt securities	—	—	—	—	—	—	—	—	—	16	—	16
Net loss	—	—	—	—	—	—	—	—	—	—	(47,263)	(47,263)
Balance as of December 31, 2023	3,963,963	$13,014	5,704,161	$25,235	11,370,621	$110,791	3,050,863	$ 2	$ 4,515	$ —	$ (119,398)	$ (114,881)

The accompanying notes are an integral part of these consolidated financial statements.

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SIONNA THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)

	Year Ended December 31, 2023	2022
Cash flows from operating activities:		
Net loss	$ (47,263)	$ (40,240)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	2,259	1,686
Non-cash operating lease expense	2,184	—
Amortization of premium and accretion of discount on marketable securities	(679)	(400)
Depreciation expense	583	122
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	333	(753)
Accounts payable	162	(1,514)
Accrued expenses and other current liabilities	(1,106)	5,031
Operating lease liabilities	(172)	—
Net cash used in operating activities	(43,699)	(36,068)
Cash flows from investing activities:		
Purchases of property and equipment	(1,116)	(1,874)
Purchases of marketable securities	(23,532)	(40,389)
Maturities of marketable securities	52,000	13,000
Net cash provided by (used in) investing activities	27,352	(29,263)
Cash flows from financing activities:		
Proceeds from issuance of Series B convertible preferred stock, net of issuance costs	—	110,791
Exercise of common stock options	31	64
Net cash provided by financing activities	31	110,855
Net (decrease) increase in cash, cash equivalents and restricted cash	(16,316)	45,524
Cash, cash equivalents and restricted cash at beginning of period	55,799	10,275
Cash, cash equivalents and restricted cash at end of period	$ 39,483	$ 55,799
Supplemental disclosure of non-cash investing and financing activities:		
Fixed asset additions included in accounts payable and accrued expenses	$ —	$ 427
Supplemental cash flow information:		
Right-of use asset obtained in exchange for new operating lease liability	$ 9,516	$ —
Reconciliation of cash and restricted cash:		
Cash and cash equivalents	$ 38,521	$ 54,837
Restricted cash	962	962
Total cash, cash equivalents, and restricted cash	$ 39,483	$ 55,799

The accompanying notes are an integral part of these consolidated financial statements.

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SIONNA THERAPEUTICS, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. Nature of the Business

Organization

Sionna Therapeutics, Inc. (the "Company"), formerly known as Sling Therapeutics, Inc., is a clinical-stage biopharmaceutical company dedicated to researching and developing novel medicines for cystic fibrosis. The Company was incorporated in Delaware in August 2019 and is headquartered in Waltham, Massachusetts.

Risks and Uncertainties

The Company is subject to a number of risks common to other companies in the biotechnology industry, including but not limited to, development by competitors of new technological innovations, risks of failure of preclinical studies and clinical trials, development and manufacturing of product candidates, obtaining regulatory approval for product candidates, competition from substitute products, the need to successfully commercialize and gain market acceptance of its product candidates, protection of proprietary technology, dependence on key personnel, the ability to attract and retain qualified employees, reliance on third party organizations compliance with government regulations, and the need to obtain additional financing. Product candidates currently under development will require significant additional research and development efforts, including extensive clinical testing and regulatory approval, prior to commercialization. These efforts will require significant amounts of additional capital, adequate personnel infrastructure, and extensive compliance-reporting capabilities. Even if the Company's development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

Liquidity and Going Concern

The Company has funded its operations primarily with proceeds from the sale of convertible preferred stock and has not generated revenue from product sales or other sources. As of December 31, 2023, the Company has raised an aggregate of $149.0 million in net proceeds through the sale of convertible preferred stock. In March 2024, the Company issued and sold 18,628,970 shares of Series C convertible preferred stock, par value $0.001 per share ("Series C Preferred Stock"), for gross proceeds of $181.9 million (the "Series C Financing"). The Company has incurred annual net operating losses and has generated negative operating cash flows in every year since inception. As of December 31, 2023, the Company had an accumulated deficit of $119.4 million. The Company expects its operating losses to continue into the foreseeable future as it continues to pursue its research and development efforts.

The Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that these consolidated financial statements are issued. The Company believes that its existing cash and cash equivalents of $38.5 million as of December 31, 2023, together with the additional proceeds received from the Series C Financing of $181.9 million in March 2024, will be sufficient to allow the Company to fund operations beyond twelve months from the date that the financial statements are available for issuance.

The Company is seeking to complete an initial public offering of its common stock. Upon the completion of a qualified public offering on specified terms, the Company's outstanding convertible preferred stock will automatically convert into shares of common stock (see Note 10).

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2. Summary of Significant Accounting Policies

Basis of Presentation and Consolidation

The accompanying consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP") as found in the Accounting Standards Codification ("ASC") of the Financial Accounting Standards Board ("FASB"). The accompanying consolidated financial statements include the accounts of Sionna Therapeutics, Inc., and its wholly owned subsidiary, Sionna Therapeutics Securities Corporation. All intercompany balances and transactions have been eliminated in consolidation.

Use of Estimates

The preparation of consolidated financial statements in conformity with GAAP requires management to make judgments, assumptions, and estimates that affect the reported amounts of assets, liabilities and expenses and the related disclosure of contingent assets and liabilities as of the date of the consolidated financial statements and the reported amounts of expenses during the reporting periods. Significant estimate and assumptions reflected within the consolidated financial statements include, but are not limited to, research and development expenses and accruals and the valuation of the Company's common shares in connection with the accounting for stock-based awards. The Company bases its estimates on historical experience and various other assumptions that are believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. On an ongoing basis, management evaluates its estimates, as there are changes in circumstances and facts. Changes in estimates are recorded in the period in which they become known. Actual results may differ from those estimates or assumptions.

Cash, Cash Equivalents and Restricted Cash

The Company considers all highly liquid investments with original maturities of ninety days or less at the time of purchase to be cash equivalents. Cash and cash equivalents typically include cash held in deposit accounts and money market funds.

Cash accounts with any type of restriction are classified as restricted cash. For the years ended December 31, 2023 and 2022, the Company had restricted cash of $1.0 million held to secure a letter of credit associated with the Company's leased corporate headquarters. The restricted funds are maintained in a certificate of deposit account. The Company classified this amount as restricted cash in the accompanying consolidated balance sheets within non-current assets based on the release date of the restrictions.

Marketable Securities

The Company classifies marketable securities with a remaining maturity greater than three months at the time of purchase and less than one year from the balance sheet date as current. Marketable securities would be classified as long-term assets on the consolidated balance sheets if the maturity exceeded one year, and the Company did not intend to utilize the marketable securities to fund current operations.

The Company classifies all of its investments as available-for-sale securities. Accordingly, these investments are recorded at fair value. Realized gains and losses and amortization and accretion of discounts and premiums are included in interest income, which is a component of other income. Unrealized gain and losses on available-for-sale securities are included in "Accumulated other comprehensive loss" as a component of stockholders' deficit until realized.

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At each balance sheet date, the Company assesses available-for-sale debt securities in an unrealized loss position to determine whether the unrealized loss or any potential credit losses should be recognized. The Company evaluates whether it intends to sell, or it is more likely than not that it will be required to sell the security before recovery of its amortized cost basis. The Company also evaluates whether the decline in fair value has resulted from credit losses or other factors. In making this assessment, the Company considers the severity of the impairment, any changes in interest rates, changes to the underlying credit ratings and forecasted recovery, among other factors. The credit-related portion of unrealized losses, and any subsequent improvements, are recorded in other income. There have been no impairment or credit losses recognized during any of the periods presented.

Concentration of Credit Risk and Off-Balance Sheet Risk

Financial instruments that potentially subject the Company to significant concentration of credit risk consist primarily of cash, cash equivalents and marketable securities. Periodically, the Company may maintain deposits in financial institutions in excess of government insured limits. The Company believes that it is not exposed to significant credit risk as its deposits are held at financial institutions that management believes to be of high credit quality and the Company has not experienced any losses on these deposits. The Company regularly invests excess cash with major financial institutions in money market funds, U.S. Treasury securities and government agency securities, and commercial paper, all of which can be readily purchased and sold using established markets. As of December 31, 2023, the Company's cash and cash equivalents were held with three financial institutions. The Company believes that the market risk arising from its holdings of these financial instruments is mitigated based on the fact that many of these securities are either government-backed or of high credit rating.

Fair Value of Financial Instruments

Certain assets and liabilities of the Company are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

Level 1—Quoted market prices in active markets for identical assets or liabilities.

Level 2—Inputs other than Level 1 inputs that are either directly or indirectly observable, such as quoted market prices, interest rates and yield curves.

Level 3—Unobservable inputs for the asset or liability (i.e., supported by little or no market activity). Level 3 inputs include management's own assumptions about the assumptions that market participants would use in pricing the asset or liability (including assumptions about risk).

To the extent that the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of the fair value requires more judgement. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of input that is significant to the fair value measurement.

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Property and Equipment, net

Property and equipment is stated at cost net of accumulated depreciation. Costs of major additions and betterments are capitalized. Maintenance and repairs to an asset that do not improve or extend its life are expensed in the period incurred. Depreciation expense is recognized using the straight-line method over the estimated useful life of each asset as follows:

	Estimated Useful Life (in years)
Lab equipment	5
Furniture & fixtures	5
Hardware & software	3
Leasehold improvements	Shorter of useful life or remaining lease term

Construction-in-progress is stated at cost, which includes direct costs attributable to the construction of the related asset. Depreciation expense is not recorded on construction-in-progress until the relevant assets are completed and placed into service. When an item is sold or retired, the costs and related accumulated depreciation are eliminated, and the resulting gain or loss, if any, is credited or charged to the consolidated statement of operations and comprehensive loss. Property and equipment to be disposed of are carried at fair value less costs to sell.

Impairment of Long-Lived Assets

Long-lived assets consist primarily of property and equipment and right-of-use assets. The Company continually evaluates whether events or circumstances have occurred that indicate that the estimated remaining useful life of its long-lived assets may warrant revision or that the carrying value of these assets may be impaired. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use or disposition of an asset group are less than its carrying amount. The impairment loss would be based on the excess of the carrying value of the impaired asset group over its fair value, determined based on discounted cash flows. In the event that such cash flows are not expected to be sufficient to recover the carrying amount of the assets, the assets are written down to their fair values. The Company has not recognized any impairment charges during the years ended December 31, 2023 and 2022.

Leases

At the inception of an arrangement the Company determines whether the arrangement contains a lease. Operating leases are included in the operating lease right-of-use lease asset (“ROU asset”), operating lease liability, current, and operating lease liability, noncurrent, on the Company’s consolidated balance sheets. Assets subject to finance leases are included in property and equipment, and the related lease obligation is included in other current liabilities and other long-term liabilities on the Company’s consolidated balance sheets. Lease expense for operating leases is recognized on a straight-line basis over the lease term as an operating expense. As of December 31, 2023 and 2022, the Company did not have any finance leases. The Company has elected the short-term lease recognition exemption for short-term leases, which allows the Company not to recognize lease liabilities and ROU assets on the consolidated balance sheets for leases with an original term of twelve months or less.

ROU assets represent the Company’s right to use an underlying asset for the lease term, and lease obligations represent the Company’s obligation to make lease payments arising from the lease. Operating lease liabilities and their corresponding ROU assets are initially recognized based on the present value of lease payments over the expected remaining lease term. When determining the lease

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term, the Company includes options to extend or terminate the lease when it is reasonably certain that the Company will exercise the option. Certain adjustments to the ROU asset may be required for items such as incentives received from the lessor. The interest rate implicit in lease contracts is typically not readily determinable. Therefore, the Company utilizes its incremental borrowing rate to discount lease payments. The incremental borrowing rate reflects the fixed rate at which the Company could borrow, on a collateralized basis, the amount of the lease payments in the same currency, for a similar term, in a similar economic environment. Leases generally provide for payments of non-lease components, such as common area maintenance, real estate taxes and other costs associated with the leased property. The Company accounts for non-lease components together with lease components. Variable lease payments, such as periodic adjustments for inflation, reimbursement of real estate taxes, and variable common area maintenance are expensed as incurred as variable lease costs and are not recorded on the consolidated balance sheets.

Sublease income is recognized on a straight-line basis over the term of the sublease agreement and is recorded within Other Income on the consolidated statements of operations and comprehensive loss.

Research and Development Expenses and Accruals

Research and development costs include (i) employee-related expenses, including salaries, benefits, and stock-based compensation expense; (ii) external research and development expenses incurred under arrangements with third parties, such as contract research organizations ("CRO") agreements, contract development and manufacturing organizations ("CDMOs"), consultants and scientific advisors; (iii) costs associated with preclinical and clinical activities and (iv) lab supplies, lab expenses and an allocation of rent, depreciation, and infrastructure.

The Company enters into contracts in the normal course of business with CROs, CDMOs, and other vendors to assist in research and development activities. These contracts generally provide for termination at any time upon a certain amount of prior notice and payment of costs incurred.

The Company recognizes research and development costs in the periods in which they are incurred. Typically, external expenses are recognized based on an evaluation of the progress to completion of specific tasks using information provided to the Company by their service providers as of each reporting date. Advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses, which are expensed as the related goods are delivered or the services are performed, or when it is no longer expected that the goods will be delivered, or the services rendered. Significant judgments and estimates are made in determining the accrued, or prepaid expense balances at the end of any reporting period.

Asset Acquisitions and Acquired In-Process Research and Development Expense

The Company accounts for acquisitions of assets or a group of assets that do not meet the definition of a business as asset acquisitions based on the cost to acquire the asset or group of assets, which include certain transaction costs. In an asset acquisition, the cost to acquire is allocated to the identifiable assets acquired and liabilities assumed based on their relative fair values as of the acquisition date. No goodwill is recorded in an asset acquisition. Assets that are acquired in an asset acquisition for use in research and development activities that have an alternative future use are capitalized as in process research and development ("IPR&D"). Acquired IPR&D that has no alternative future use as of the acquisition date is recognized as research and development expense as of the acquisition date. The Company will recognize additional research and development expenses in the future if and when the Company becomes obligated to make contingent milestone payments under the terms of the agreements by which it acquired the IPR&D assets.

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Contingent consideration in the form of milestone payments related to IPR&D with no alternative future use are charged to expense when the related milestone is achieved and becomes payable. For the years ended December 31, 2023 and 2022, the Company did not recognize any milestones or IPR&D expense in connection with the consideration due under its license agreements (see Note 9).

Patent Costs

All patent-related costs incurred in connection with filing and prosecuting patent applications such as direct application fees, and legal and consulting expenses are expensed as incurred due to the uncertainty about the recovery of the expenditure. Patent-related costs are classified as general and administrative expenses within the Company's consolidated statements of operations and comprehensive loss.

Convertible Preferred Stock

The Company's convertible preferred stock is classified as temporary equity in the accompanying consolidated balance sheets and excluded from stockholders' deficit as the potential redemption of such stock is outside the Company's control and would require the redemption of the then-outstanding convertible preferred stock. The convertible preferred stock is not redeemable except for in the event of a liquidation, dissolution or winding up of the Company (see Note 11). Costs incurred in connection with the issuance of convertible preferred stock are recorded as a reduction of gross proceeds from issuance. Subsequent adjustments of the carrying values to the ultimate redemption values will be made only when it becomes probable that these events will occur.

Stock-Based Compensation

The Company measures all stock options and other stock-based awards granted to employees, non-employees, and directors based on the fair value on the date of the grant and recognizes compensation expense of those awards over the requisite service period, which is generally the vesting period of the respective award. The Company's stock-based payments include stock options and grants of restricted common stock awards. Generally, the Company issues stock-based awards with only service-based vesting conditions. The measurement date for employee awards is the date of grant, and stock-based compensation costs are recognized as expense over the employees' requisite service period, which is the vesting period, on a straight-line basis. The Company's policy is to account for forfeitures when they occur. Stock-based compensation expense is classified in the accompanying consolidated statements of operations and comprehensive loss in the same manner in which the award recipient's payroll costs are classified or in which the award recipients service payments are classified.

Restricted common stock awards are subject to repurchase rights until such awards meet all vesting conditions. Accordingly, the Company has recorded the proceeds from the issuance of restricted stock awards as a liability in the consolidated balance sheets. The restricted stock liability is reclassified into stockholders' equity as the restricted stock vests.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. The Board of Directors of the Company (the "Board") determines the fair value of the Company's common stock, par value $0.001 per share ("common stock"), taking into consideration its most recently available third-party valuations of common stock as well as additional factors which may have changed since the date of the most recent contemporaneous valuation through grant date. Due to the lack of company-specific historical and implied volatility information, the Company estimates its expected stock volatility based on the historical volatility of a publicly traded set of peer companies and expects to continue to do so until it has adequate historical data regarding the volatility of its own traded stock price. The Company uses the simplified method as prescribed by the

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Securities and Exchange Commission's Staff Accounting Bulletin No. 107, *Share-Based Payment*, to calculate the expected term for options granted to employees and non-employees as the Company does not have sufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is zero because the Company has never paid cash dividends on common shares and does not expect to pay any cash dividends in the foreseeable future.

Income Taxes

The Company's provision for income taxes, deferred tax assets and liabilities, and reserves for unrecognized tax benefits reflect our best assessment of estimated future taxes to be paid. Significant judgments and estimates based on interpretations of existing tax laws or regulations in the United States are required in determining our provision for income taxes. Changes in tax laws, statutory tax rates, and estimates of our future taxable income could impact the deferred tax assets and liabilities provided for in the consolidated financial statements and would require an adjustment to the provision for income taxes.

The Company accounts for income taxes using the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the consolidated financial statements or in the Company's tax returns. Deferred tax assets and liabilities are determined on the basis of the differences between the financial statements and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes, based upon the weight of available evidence, that it is more likely than not that all or a portion of the deferred tax assets will not be realized, a valuation allowance is established through a charge to income tax expense. Potential for recovery of deferred tax assets is evaluated by estimating the future taxable profits expected and considering prudent and feasible tax planning strategies.

The Company accounts for uncertainty in income taxes recognized in the consolidated financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more-likely-than-not to be sustained, the tax position is then assessed to determine the amount of benefit to recognize in the consolidated financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, which are considered appropriate as well as the related net interest and penalties.

Net Loss Per Share

Basic net loss per share is computed by dividing net loss by the weighted-average number of common shares outstanding during the period. Diluted net loss per share is computed using the weighted-average number of common shares outstanding during the period and, if dilutive, the weighted-average number of potential shares of common shares. Net loss per share attributable to common stockholders is calculated using the two-class method, which is an earnings allocation formula that determines net loss per share for the holders of the Company's common shares and participating securities. The Company's convertible preferred stock contains participation rights in any dividend paid by the Company and is deemed to be a participating security. The participating securities do not include a contractual obligation to share in losses of the Company and are not included in the calculation of net loss per share in the periods in which a net loss is recorded.

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Diluted net loss per share is computed using the more dilutive of (a) the two-class method or (b) the if-converted method. The Company allocates earnings first to preferred stockholders based on dividend rights and then to common and preferred stockholders based on ownership interests. The weighted-average number of common shares included in the computation of diluted net loss per share gives effect to all potentially dilutive common equivalent shares.

Common stock equivalent shares are excluded from the computation of diluted net loss per share if their effect is antidilutive. In periods in which the Company reports a net loss attributable to common stockholders, diluted net loss per share attributable to common stockholders is the same as basic net loss per share attributable to common stockholders since dilutive common shares are not assumed to have been issued if their effect is antidilutive. The Company reported a net loss attributable to common stockholders for the years ended December 31, 2023 and 2022.

Comprehensive Loss

Comprehensive loss includes net loss as well as other changes in stockholder's deficit that result from transactions and events other than those with stockholders. The Company's unrealized gains and losses on marketable securities represent the only component of other comprehensive loss that are excluded from the reported net loss and that are presented in the consolidated statements of operations and comprehensive loss.

Segment Information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker in deciding how to allocate resources and assess performance. The Company and the Company's chief operating decision maker, the Company's Chief Executive Officer, view the Company's operations and manage the Company's business as a single operating segment, which is the business of developing and commercializing cystic fibrosis drugs.

Emerging Growth Company Status

The Company is an emerging growth company, as defined in the Jumpstart Our Business Startups Act (the "JOBS Act"). Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies. The Company has elected to use this extended transition period for complying with new or revised accounting standards that have different effective dates for public and private companies until the earlier of the date that it (i) is no longer an emerging growth company or (ii) affirmatively and irrevocably opts out of the extended transition period provided in the JOBS Act. As a result, these consolidated financial statements may not be comparable to companies that comply with the new or revised accounting pronouncements as of public company effective dates.

Recently Adopted Accounting Pronouncements

In June 2016, the FASB issued Accounting Standards Update ("ASU") 2016-13, *Financial Instruments—Credit Losses (Topic 326: Measurement of Credit Losses on Financial Instruments)* ("ASU 2016-13"). The standard requires that credit losses be reported using an expected losses model rather than the incurred losses model that is currently used, and establishes additional disclosures related to credit risks. For available-for-sale debt securities with unrealized losses, this standard requires allowances to be recorded instead of reducing the amortized cost of the investment. The Company adopted this guidance effective January 1, 2023. The adoption of this standard did not have a material impact on the Company's consolidated financial position and results of operations.

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Recently Issued Accounting Pronouncements Not Yet Adopted

In November 2023, the FASB issued ASU 2023-07, *Segment Reporting (Topic 280: Improvements to Reportable Segment Disclosures)* ("ASU 2023-07"). The amendments in this update improve reportable segment disclosure requirements through enhanced disclosures about significant segment expenses. All disclosure requirements of the update are required for entities with a single reportable segment. The amendments are effective for fiscal years beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024, and should be applied on a retrospective basis to all periods presented. For purposes of its annual reporting requirements, the Company has adopted this standard as of January 1, 2024. As of December 31, 2023, the Company only has one reportable segment. The Company is currently in the process of evaluating the effects of this pronouncement on the related disclosures.

In December 2023, the FASB issued ASU 2023-09, *Income Taxes (Topic 740: Improvements to Income Tax Disclosures)* ("ASU 2023-09"). ASU 2023-09 provides more transparency about income tax information through improvements to income tax disclosures primarily related to the rate reconciliation and incomes taxes paid information. For public companies, the amendments are effective for annual periods beginning after December 15, 2024 and should be applied prospectively. The Company has determined that the effects of adopting the amendments in ASU 2023-09 will not have a material impact on its consolidated financial position, the results of its operations, or related disclosures when such amendment is adopted.

3. Marketable Securities

The following table summarizes the amortized cost and estimated fair value of the Company's commercial paper and U.S. Treasury securities, which are considered to be available-for-sale investments and were included in marketable securities (in thousands):

	December 31, 2022			
	Amortized Cost	Unrealized Gain	Unrealized Loss	Fair Value
Marketable securities:				
Commercial paper	$ 11,886	$ —	$ —	$ 11,886
U.S. treasuries	15,903	—	(16)	15,887
Total	$ 27,789	$ —	$ (16)	$ 27,773

As of December 31, 2023, the Company held commercial paper within cash and cash equivalents as it had a maturities of less than 90 days and no marketable securities were held as of December 31, 2023.

As of December 31, 2022, all marketable securities had contractual maturities within one year and are considered short-term investments. The Company has determined that there were no material changes in the credit risk, therefore the Company has not recognized any credit allowances on its debt securities.

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4. Fair Value Measurements

The following table presents information about the Company's financial instruments that are measured at fair value on a recurring basis and indicates the fair value hierarchy of the inputs the Company utilized to determine such fair value (in thousands):

	December 31, 2023			
	Total	Level 1	Level 2	Level 3
Assets				
Cash equivalents:				
Money market funds	$23,660	$23,660	$ —	$ —
Commercial paper	989	—	989	—
Total financial assets	$24,649	$23,660	$ 989	$ —

	December 31, 2022			
	Total	Level 1	Level 2	Level 3
Assets				
Cash equivalents:				
Money market funds	$42,106	$42,106	$ —	$ —
Government agency securities	1,918	—	1,918	—
Marketable securities:				
Commercial paper	11,886	—	11,886	—
U.S. Treasury securities	15,887	15,887	—	—
Total financial assets	$71,797	$57,993	$13,804	$ —

As of December 31, 2023 and 2022, the Company had no financial liabilities that required fair value measurement. As of December 31, 2023, the Company had cash equivalents consisting of money market funds classified as Level 1 financial assets, as these assets are valued using quoted market prices in active markets without any valuation adjustments. Additionally, the Company holds commercial paper which were considered Level 2 category assets as these have quoted prices in markets that are not considered to be active.

As of December 31, 2022, the Company held both marketable securities as well as U.S. Treasuries which were included in cash equivalents and are considered Level 1 category assets. Additionally, the Company held both government agency securities and commercial paper which are considered Level 2 category assets.

During the year ended December 31, 2023 and 2022, there were no transfers or reclassifications between fair value measurement levels of assets or liabilities. The carrying values of prepaid expenses and other current assets, accounts payable and accrued expenses and other current liabilities approximate their fair values due to the short-term nature of these assets and liabilities.

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5. Property and Equipment, net

Property and equipment, net consisted of the following (in thousands):

	December 31,	
	2023	2022
Deposits on lab and office equipment	$ 116	$ 780
Construction-in-progress	—	1,521
Laboratory equipment	1,329	617
Leasehold improvements	1,854	—
Furniture & fixtures	722	—
Total property and equipment	4,021	2,918
Less: accumulated depreciation	(914)	(344)
Property and equipment, net	$3,107	$2,574

Depreciation expense was $0.6 million and $0.1 million for the years ended December 31, 2023 and 2022, respectively.

6. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following (in thousands):

	December 31,	
	2023	2022
Accrued research and development	$3,065	$5,107
Accrued compensation and benefits	3,112	1,953
Accrued professional fees	362	147
Accrued other	51	489
Total accrued expenses and other current liabilities	$6,590	$7,696

7. Leases

Operating Leases Summary

Natick, Massachusetts

In January 2020, the Company entered into a noncancelable facility lease agreement to lease 1,700 square feet of laboratory space in Natick, Massachusetts (“Natick Lease”). The original term of the Natick Lease commenced in February 2020 for a 12-month period and was classified as a short-term lease. The Natick Lease was subsequently extended three times and the lease term ended in June 2023.

Waltham, Massachusetts

In May 2022, the Company entered into a noncancelable facility lease agreement to lease 24,051 square feet of office and laboratory space in Waltham, Massachusetts (“Waltham Lease”), which became the Company headquarters in January 2023 following the completion of construction of the prebuilt office and lab space, and when the Company received full access and control over the leased space. Upon execution of the Waltham Lease, the Company was required to prepay $0.2 million, which was included in the prepaid expenses and other current assets as of December 31, 2022 and included in the initial measurement of the right-of-use asset in 2023 when the Waltham Lease commenced. At commencement of the lease the right-of-use asset was $9.5 million, which was reduced by $0.4 million of lease incentives, offset by $0.2 million of lease prepayments, and the lease liability was $9.7 million.

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The original term for the Waltham Lease is for the period from January 2023 through November 2030, and the Company has the option to extend the Waltham Lease for an additional five-year term at market-based rates. The renewal option is not reasonably certain to occur, and as such, it was not considered in the measurement of the right-of-use asset and lease liability. The Waltham Lease has escalating base rent payments, which commenced in September 2023 and continue through the expiration of the lease. The Company received eight months of free rent and five additional months of 50% rent abatement. The Company is responsible for other fixed and variable payments including operating expenses and property taxes.

Per the terms of the Waltham Lease, the Landlord agreed to construct $0.4 million of leasehold improvements that the Company concluded were the landlord's asset and therefore were not recorded by the Company. In addition, the Landlord provided the Company an option to finance up to $0.4 million (the "TI Option") in additional leasehold improvements over the lease term at a 9% per annum interest rate through higher rent payments. The Company exercised the TI Option in June 2022, and the Waltham Lease was amended to account for the increase in rental payments. The Company concluded that the leasehold improvements that were constructed with the TI Option funds were for Company-owned assets and therefore the TI Option represented a lease incentive. Upon the lease commencement of the Waltham Lease in January 2023, the lease incentive was recorded as a reduction to the initial measurement of the right-of-use asset.

Sublease Agreement

In June 2023, the Company entered into a sublease agreement to sublet 6,399 square feet of its office and laboratory space under the Waltham Lease (the "Sublease"). The Company was not relieved of its obligation to the lessor for the head lease as a result of its entry into the Sublease. The Sublease is classified as an operating lease and commenced in July 2023 and has an original term of 26 months with the option to extend for an additional six months. The Sublease has escalating rent payments and is secured by a letter of credit of $0.2 million with the Company named as the beneficiary. Sublease income is recognized on a straight-line basis and the rental income and reimbursement for operating expenses and taxes is included in other income on the consolidated statements of operations and comprehensive loss. As of December 31, 2023, the Company recognized $0.3 million of sublease income within other income on the consolidated statements of operations and comprehensive loss, and recognized no sublease income for the year ended December 31, 2022.

Operating Lease Tables

The following table presents weighted average remaining lease term and discount rates which are used in the calculation of the Company's right of use asset and lease liabilities at the end of each reporting period:

	December 31,	
	2023	2022
Weighted average remaining lease term (years)	6.9	—
Weighted average discount rate	11.0%	9.0%
Cash paid for amounts included in the measurement of operating lease liabilities (in thousands)	$ 172	$ —

The components of lease costs were as follows (in thousands):

	December 31,	
	2023	2022
Operating lease costs	$1,824	$ —
Variable lease costs	468	—
Short-term lease costs	88	207
Total lease cost	$2,380	$207

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Lease commitments

Future minimum lease payments were as follows (in thousands):

Year ended December 31,	Amount
2024	$ 1,897
2025	2,128
2026	2,190
2027	2,253
2028	2,319
Thereafter	4,632
Total future minimum lease payments	15,419
Less imputed interest	(4,942)
Present value of operating lease liabilities	$10,477

8. Commitments and Contingencies

Operating Leases

The Company has entered into arrangements for leases of office space; see Note 7, “Leases,” for details.

Legal Proceedings

The Company was not subject to any material legal proceedings during the years ended December 31, 2023 and 2022, and the Company is not aware of any material legal proceedings that are currently pending or threatened.

License Agreements

The Company entered into licenses agreements under which it is obligated to make fixed and contingent payments; see Note 9 “License Agreements” and Note 16 “Subsequent Events” for details.

Other Contracts

The Company is party to various contracts with CROs and contract manufacturing organizations that generally provide for termination on notice, with the exact amounts in the event of termination to be based on the timing of the termination and the terms of the agreement.

Based on our development plans as of December 31, 2023, the Company may be obligated to make future development and commercial milestone payments, and royalty payments on future sales of specified products associated with the Company’s license agreements (refer to Note 9). Payments under these agreements generally become due and payable upon achievement of such milestones or sales. When the achievement of these milestones or sales have not occurred, such contingencies are not recorded in the Company’s consolidated financial statements.

There were no contractual obligations arising from these arrangements as of December 31, 2023 and 2022.

Indemnification Agreements

As permitted under Delaware law, the Company indemnifies its officers, directors, and employees for certain events or occurrences while the officer or director is, or was, serving at the Company’s

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request in such capacity. The term of the indemnification is for the officer's or director's lifetime. Further, in the ordinary course of business the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. To date however, the Company has not incurred any material costs as a result of such indemnifications nor experienced any losses related to them. As of December 31, 2023 and 2022, the Company was not aware of any claims under indemnification arrangements and does not expect significant claims related to these indemnification obligations and, consequently, concluded that the fair value of these obligations is negligible; therefore, no related reserves were established.

9. License Agreements

Sanofi License Agreement

In December 2019, the Company entered into a license agreement, which has been subsequently amended (as amended, the "Sanofi License Agreement"), with Sanofi SA (f/k/a Genzyme) ("Sanofi"), pursuant to which the Company has been granted an exclusive, sub-licensable, royalty-bearing, worldwide license to develop and commercialize products using the licensed compounds and know-how for cystic fibrosis transmembrane conductance regulator ("CFTR") correctors.

As initial consideration for the license, the Company paid a non-refundable, upfront payment of $1.5 million, as well as a reimbursement of $0.3 million for Sanofi's research and development expenses, which was recorded as research and development expense in the consolidated statements of operations and comprehensive loss because the acquired license represented in-process research and development with no alternative future use. In addition, the Company is required to pay Sanofi a total of up to $40.0 million upon achievement of certain late-stage developmental and commercial milestones. The developmental milestone payment will be recorded when the milestone is achieved, and the commercial milestone payment and royalties will be recorded when the sales occur. As of December 31, 2023, none of such milestones have been achieved. The Company is also required to pay royalties to Sanofi in the low single-digit percentage range based on net sales of licensed products, subject to customary reductions and offsets. Such royalty payments shall be reduced for products covered by derived patents.

CFF Payment Agreement

In December 2019, the Company entered into a payment agreement (the "CFF Payment Agreement") with the Cystic Fibrosis Foundation ("CFF"), pursuant to which the Company agreed to provide CFF with compensation in exchange for the grant of, or forbearance from exercising, certain of CFF's rights existing under the license agreement by and between CFF (through an assignment by Cystic Fibrosis Foundation Therapeutics, Inc.) and Genzyme Corporation, an affiliate of Sanofi (the "CFFT-Genzyme Agreement"). Under the CFF Payment Agreement, the Company is obligated to compensate CFF in connection with the Company's development and commercialization of licensed products under the Sanofi License Agreement.

As initial consideration for CFF's grant of, and forbearance from exercising, its rights under the CFFT-Genzyme Agreement, the Company paid an upfront fee of $0.2 million and issued CFF 300,300 shares of its Series Seed preferred stock, valued at $1.0 million. In addition, the Company agreed to pay CFF a sub-teen double-digit percentage of any amounts paid by it to Sanofi under the Sanofi License Agreement, other than milestone, royalty or reimbursement payments. In addition, the

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Company is required to pay CFF a total of up to $40.0 million upon achievement of certain late-stage developmental and commercial milestones. Such milestone and royalty payments shall be reduced for products covered by derived patents. Further, a side letter was executed between the Company and Sanofi, which clarifies the relationship between the Company, Sanofi and CFF, under which the Company is obligated to pay Sanofi 20% of the milestones it would have been obligated to pay CFF, net of the milestone amounts it is obligated to pay under the Sanofi License Agreement.

The Company records the milestone payments as research and development expenses when the milestones occur and consideration is paid or becomes payable. As of December 31, 2023, none of such milestones have been achieved. The Company is also required to pay revenue-shares of royalty payments to CFF in the low single-digit percentage range based on net sales of licensed products subject to customary reductions and offsets. Royalties will be recorded when the sales occur.

10. Convertible Preferred Stock

Series Seed

In December 2019, the Company issued and sold 2,762,762 shares of Series Seed convertible preferred stock, par value $0.001 per share ("Series Seed Preferred Stock") to certain investors at a purchase price of $3.33 per share, for gross proceeds of $8.2 million (the "Series Seed Financing"). As disclosed in Note 9, the Company issued Series Seed Preferred Stock to CFF in connection with the CFF Payment Agreement, and the fair value of the Series Seed Preferred Stock was recorded as research and development expense in the consolidated statements of operations and comprehensive loss. One investor was also issued 684,415 shares of common stock in connection with the initial Series Seed Financing closing. The proceeds from that investor were allocated between the Series Seed Preferred Stock and common stock on a relative fair value basis. There were no material issuance costs incurred related to this financing.

In June 2020, upon the Board's approval, the Company issued an additional 1,201,202 shares of Series Seed Preferred Stock to an additional investor at $3.33 per share for gross proceeds of $4.0 million. The Company incurred $38,887 of issuance costs in connection with this issuance.

Series A

In September 2020, the Company issued and sold 5,704,161 shares of Series A convertible preferred stock, par value $0.001 per share ("Series A Preferred Stock"), at a purchase price of $4.4289 per share, for gross proceeds of $25.3 million (the "Series A Financing"). The Company incurred $0.1 million of issuance costs in connection with the Series A Financing.

Series B

In February 2022, the Company issued and sold 11,370,621 shares of Series B convertible preferred stock, par value $0.001 per share ("Series B Preferred Stock"), at a purchase price of $9.762 per share, for gross proceeds of $111.0 million (the "Series B Financing"). The Company incurred $0.2 million of issuance costs in connection with the Series B Financing.

Upon the issuance of Series Seed Preferred Stock, Series A Preferred Stock and Series B Preferred Stock (together with the Series C Preferred Stock, the "Preferred Stock"), the Company assessed the embedded conversion and liquidation features of the shares and determined that the Company was not required to separately account for these features.

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Preferred Stock consists of the following (in thousands, except share amounts):

	December 31, 2023				
	Preferred Stock Authorized	Preferred Stock Issued and Outstanding	Carrying Value	Liquidation Value	Stock Issuable Upon Conversion
Series Seed Preferred Stock	3,963,963	3,963,963	$ 13,014	$ 13,200	2,712,998
Series A Preferred Stock	5,704,161	5,704,161	25,235	25,263	3,904,017
Series B Preferred Stock	11,370,621	11,370,621	110,791	111,000	7,782,230
Total	21,038,745	21,038,745	$ 149,040	$ 149,463	14,399,245

	December 31, 2022				
	Preferred Stock Authorized	Preferred Stock Issued and Outstanding	Carrying Value	Liquidation Value	Stock Issuable Upon Conversion
Series Seed Preferred Stock	3,963,963	3,963,963	$ 13,014	$ 13,200	2,712,998
Series A Preferred Stock	5,704,161	5,704,161	25,235	25,263	3,904,017
Series B Preferred Stock	11,370,621	11,370,621	110,791	111,000	7,782,230
Total	21,038,745	21,038,745	$ 149,040	$ 149,463	14,399,245

The holders of Preferred Stock have the following rights, preferences and privileges:

Voting Rights

The holders of each series of Preferred Stock are entitled to vote on all matters and shall have the number of votes equal to the number of shares of common stock into which the Preferred Stock is convertible.

Dividends

Dividends shall be payable only when, as, and if declared by the Board of Directors, and the Company shall be under no obligation to pay any dividends. The Company shall not declare, pay or set aside any dividends on shares of any other class or series of capital stock unless the Preferred Stockholders then outstanding first receive the dividend amounts calculated based upon the dividend on the class or series of capital stock that would result in the highest Preferred Stock dividend.

Liquidation Preference

The holders of the Preferred Stock have first preference in the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company, including a merger or sale of the Company. The amount to be paid is equal to greater of the Series B Preferred Stock original issue price in the case of the Series B Preferred Stock, the Series A Preferred Stock original issue price in the case of the Series A Preferred Stock, and the Series Seed Preferred Stock original issue price in the case of the Series Seed Preferred Stock, plus any dividends declared but unpaid thereon, or such amount per share as would have been payable had all shares of the Preferred Stock been converted into common stock.

Redemption

The Preferred Stock does not have redemption rights, except for the contingent redemption upon the occurrence of a liquidation event, which would constitute a merger or consolidation, or the sale, lease, transfer, exclusive license or other disposition, of all or substantially all of the Company's or its subsidiaries' assets.

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Conversion

Each share of Preferred Stock shall be convertible, at the option of the holder (without payment) into common stock as determined by dividing the Preferred Stock's original issue price by the conversion price in effect at the time of conversion. The Series B Preferred Stock conversion price shall initially be equal to $9.762, the Series A Preferred Stock conversion price shall initially be equal to $4.4289, and the Series Seed Preferred Stock conversion price shall initially be equal to $3.33. In the event of a liquidation, dissolution or winding up of the Company or a Deemed Liquidation Event, the conversion right shall terminate at the close of business on the last full day preceding the date fixed for the payment of any such amounts distributable on such event to the holders of Preferred Stock.

All shares of Preferred Stock will automatically convert into shares of common stock upon a qualified initial public offering on a one-to-one basis.

11. Common Stock

The Company was authorized to issue 30,000,000 of common stock as of December 31, 2023 and 2022, respectively. As of December 31, 2023 and 2022, there were 3,313,012 and 3,277,460 shares, legally issued and outstanding, which includes 262,149 and 484,531 shares of unvested restricted stock.

As of December 31, 2023 and 2022, there were 3,050,863 and 2,792,929 shares of common stock outstanding for accounting purposes as reported on the consolidated statement of convertible preferred stock and stockholders' deficit. This excludes unvested restricted stock which are legally outstanding but are not considered outstanding for accounting purposes.

The voting, dividend and liquidation rights of the holders of the Company's common stock are subject to and qualified by the rights, powers and preference of the holders of the Preferred Stock set forth in Note 10 above.

The number of shares of common stock that have been reserved for the potential conversion of Preferred Stock, exercise of stock options, and vesting of restricted stock is as follows:

	Year Ended December 31,	
	2023	2022
Convertible preferred stock	14,399,245	14,399,245
Options to purchase common stock	1,914,362	1,945,186
Unvested restricted stock	262,149	484,531
Total	16,575,756	16,828,962

12. Stock-Based Compensation

2020 Stock Option and Grant Plan

The Company's 2020 Stock Option and Grant Plan, as amended (the "2020 Plan"), provides for the Company to sell or issue common stock or restricted common stock, or to grant incentive stock options or nonqualified stock options for the purchase of common stock, to employees, members of the Board and consultants of the Company. The 2020 Plan is administered by the Board, or at the discretion of the Board, by a committee of the Board. The exercise prices, vesting and other restrictions are determined at the discretion of the Board, or their committee if so delegated, except that the exercise price per share of stock options may not be less than 100% of the fair market value of the share of common stock on the date of grant and the term of stock option may not be greater than ten years.

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The total number of shares of common stock that may be issued under the 2020 Plan is 3,949,579 shares, of which 775,423 remained available for future grant as of December 31, 2023. Vesting periods are determined at the discretion of the Board. Stock options and restricted stock granted to-date typically vest over four years.

Restricted Common Stock

The Company did not issue shares of restricted common stock to founders and officers during the years ended December 31, 2023 or 2022. The outstanding restricted shares are subject to a vesting schedule that varies among the grants, and vesting may be accelerated upon a change in control, as defined in the agreements. If the holders cease to have a business relationship with the Company, the Company may repurchase any unvested shares of common stock held by these individuals at their original purchase price. The unvested shares of common stock are not considered outstanding shares and are recorded as a liability until the shares vest.

A summary of the Company's restricted stock activity during the year ended December 31, 2023 is presented below:

	Number of Restricted Shares	Weighted-Average Per-Share Grant-Date Fair Value
Unvested as of December 31, 2022	484,531	$ 0.76
Vested	(222,382)	$ 0.75
Unvested as of December 31, 2023	262,149	$ 0.78

For the years ended December 31, 2023 and 2022, the liability related to the payments received for shares of unvested restricted stock was de minimis. The aggregate fair value of restricted stock awards that vested during the years ended December 31, 2023 and 2022 was $1.4 million and $2.0 million, respectively.

Stock Option Valuation

The assumptions used to determine the fair values of stock options granted to employees and directors during the years ended December 31, 2023 and 2022 are presented as follows:

	Year Ended December 31,	
	2023	2022
Expected term (in years)	6.0	6.0
Volatility	107.0%	96.4%
Interest rate	4.1%	2.2%
Dividend yield	—	—

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Stock Option Activity

The following table summarizes the Company's stock option activity under the 2020 Plan for the year ended December 31, 2023:

	Number of Shares	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding as of December 31, 2022	1,945,186	$ 5.19	9.1	$ 1,797
Granted	179,636	6.11		
Exercised	(35,552)	0.87		
Cancelled or forfeited	(174,908)	5.75		
Outstanding as of December 31, 2023	1,914,362	$ 5.30	7.9	$ 1,549
Exercisable as of December 31, 2023	848,368	$ 4.89	7.2	$ 1,032

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the stock options and the estimated fair value of the Company's common stock for those stock options that had exercise prices lower than the fair value of the Company's common stock.

The weighted-average grant date fair value in the years ended December 31, 2023 and 2022, was $5.10 and $4.76, respectively. The total intrinsic value of options exercised in the years ended December 31, 2023 and 2022 was $0.2 million and $0.4 million, respectively.

Stock-Based Compensation Expense

During the years ended December 31, 2023 and 2022, the Company recorded stock-based compensation in the accompanying consolidated statements of operations and comprehensive loss as follows (in thousands):

	Year Ended December 31,	
	2023	2022
Research and development expense	$ 1,135	$ 859
General and administrative expense	1,124	827
Total	$ 2,259	$ 1,686

As of December 31, 2023, there was $4.4 million of unrecognized stock-based compensation expense for stock option awards that are expected to be recognized over a weighted average period of 2.4 years. As of December 31, 2023, there was $0.2 million of unrecognized stock-based compensation expense for restricted stock awards that are expected to be recognized over a weighted average period of 1.3 years.

13. Income Taxes

The Company had no federal or state income tax expense due to operating losses incurred for the years ended December 31, 2023 and 2022.

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A reconciliation of income tax expense (benefit) from continuing operations to the amount computed by applying the statutory federal income tax rate to the net income (loss) from continuing operations is summarized as follows:

	Year Ended December 31,	
	2023	2022
Tax expense (benefit) at the statutory rate	21.0%	21.0%
State income taxes, net of federal benefit	6.3%	5.5%
Stock-based compensation	(0.4%)	(0.7%)
Permanent differences	0.1%	0.1%
Difference and changes in tax rates	0.7%	0.0%
Tax credits	2.4%	2.4%
Return to provision true up	(0.1%)	(0.2%)
Change in valuation allowance	(30.0%)	(28.1%)
Effective tax rate	0.0%	0.0%

The components of the Company's deferred tax assets and liabilities are comprised of the following (in thousands):

	Year Ended December 31,	
	2023	2022
Net operating loss carryforward	$ 13,249	$ 9,719
Tax credits	2,204	1,124
Fixed assets	21	—
Stock-based compensation	296	—
Capitalized research costs	16,737	8,330
Accrued expenses	465	209
Intangibles	830	790
Lease liability	2,862	—
Other temporary differences	8	5
Total deferred tax assets	$ 36,672	$ 20,177
Less valuation allowance	(34,297)	(20,092)
Net deferred tax asset	$ 2,375	$ 85
Right of use assets	$ (2,375)	$ —
Stock-based compensation	—	(60)
Fixed assets	—	(25)
Total deferred tax liabilities	$ (2,375)	$ (85)
Net deferred tax asset (liability)	$ —	$ —

As of December 31, 2023 and 2022, the Company had federal net operating loss carryforwards of approximately $48.2 million and $35.9 million, respectively, which may be available to offset future income tax liabilities. The 2017 Tax Cuts and Jobs Act ("TCJA") will generally allow losses incurred after 2017 to be carried over indefinitely but will generally limit the net operating loss deduction to the lesser of the net operating loss carryover or 80% of a corporation's taxable income (subject to Section 382 of the Internal Revenue Code of 1986, as amended (the "Internal Revenue Code")). Also, there are no carryback for losses incurred after 2017. All net operating loss carryforwards were generated after 2017.

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In addition, the Company has state net operating loss carryforwards of $47.1 million and $34.3 million as of December 31, 2023 and 2022, respectively, which will expire at various dates through 2043.

As of December 31, 2023 and 2022, the Company had federal research and development tax credit ("Federal R&D Tax Credit") carryforwards of $1.5 million and $0.8 million, respectively, available to reduce future tax liabilities, which will expire at various dates through 2043.

As of December 31, 2023 and 2022, the Company had state research and development credit ("State R&D Tax Credit") carryforwards of approximately $1.4 million and $0.8 million, respectively, available to reduce future tax liabilities, which will expire at various dates through 2038.

The Company has evaluated the positive and negative evidence bearing upon its ability to realize its deferred tax assets, which are comprised primarily of net operating loss carryforwards, the capitalization of research and experimental expenditures, and tax credits. Management has considered the Company's history of cumulative net losses in the United States, estimated future taxable income and prudent and feasible tax planning strategies and has concluded that it is more likely than not that the Company will not realize the benefits of its U.S. federal and state deferred tax assets. Accordingly, a full valuation allowance of $34.3 million and $20.1 million has been established against these net deferred tax assets as of December 31, 2023 and 2022, respectively. The Company reevaluates the positive and negative evidence at each reporting period. The Company's valuation allowance increased during 2023 by approximately $14.2 million, primarily due to the capitalization of research and experimental expenditures and the increase in net operating loss and tax credit carryforwards.

Under the provision of the Internal Revenue Code, the net operating loss and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. Net operating loss and tax credit carryforwards may become subject to an annual limitation in the event of certain cumulative change in the ownership interest of significant shareholders over a three-year period in excess of 50%, as defined under Section 382 and 383 of the Internal Revenue Code, respectively, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of the Company immediately prior to the ownership change. Subsequent ownership change may further affect the limitation in future years. The Company has not yet completed a change in control analysis, as defined under Section 382 and 383 of the Internal Revenue Code, through December 31, 2023, and the Company has not determined whether the future utilization of net operating loss carryforwards may be materially limited based on past financings. In addition, the Company may complete future financings that could result in a change in control in the future which may limit the amount of tax attributes available to offset future tax liabilities.

We account for income taxes by evaluating a probability threshold that a tax position must meet before a financial statement benefit is recognized. The minimum threshold is a tax position that is more likely than not to be sustained upon examination by the applicable taxing authority, including resolution of any related appeals or litigation processes, based on the technical merits of the position.

The Company didn't have any unrecognized tax benefits as of December 31, 2023 or 2022, respectively. The Company does not expect its unrecognized tax benefits to change significantly over the next year. Realization of the future tax benefits is dependent on the many factors, including the Company's ability to generate taxable income. The Company recognizes interest and penalties related to uncertain tax positions in income tax expense. As of December 31, 2023 and 2022, the Company's balance of interest and penalties was zero.

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The Company files tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In a normal course of business, the Company is subject to examination by U.S. federal and states. The Company's tax years are still open since inception. To the extent that the Company has tax attribute carryforwards, the tax year in which the attributes were generated may still be adjusted upon examination by the U.S. Internal Revenue Services or state tax authorities to the extent utilized in a future period. The Company is not currently under examination by any tax authorities.

14. Net Loss Per Share

Basic and diluted net loss per share attributable to common stockholders was calculated as follows for the periods presented (in thousands, except share and per share amounts):

	Year Ended December 31,	
	2023	2022
Numerator:		
Net loss	$ (47,263)	$ (40,240)
Net loss attributable to common stockholders, basic and diluted	$ (47,263)	$ (40,240)
Denominator:		
Weighted-average number of common shares used in net loss per share, basic and diluted	2,933,218	2,577,544
Net loss per share of common shares, basic and diluted	$ (16.11)	$ (15.61)

The Company's potentially dilutive securities, which include convertible Preferred Stock, restricted stock and stock options, have been excluded from the computation of diluted net loss per share as the effect would be to reduce the net loss per share. Therefore, the weighted-average number of common stock outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same. The Company excluded the following shares from the computation of diluted net loss per share attributable to common stockholders as of December 31, 2023 and 2022 because including them would have had an anti-dilutive effect:

	Year Ended December 31,	
	2023	2022
Convertible preferred stock	14,399,245	14,399,245
Unvested restricted stock	262,149	484,531
Options to purchase common stock	1,914,362	1,945,186
Total	16,575,756	16,828,962

15. Employee Benefit Plan

The Company established a defined contribution savings plan under Section 401(k) of the Internal Revenue Code. This plan covers all employees and allows participants to defer a portion of their annual compensation on a pre-tax basis. Company contributions to the plan may be made at the discretion of the Board. For the years ended December 31, 2023 and 2022, no contributions have been made to the plan by the Company.

16. Subsequent Events

The Company has evaluated subsequent events after December 31, 2023, through September 12, 2024, the date these financial statements were available to be issued, and February 3, 2025, for the reverse stock split described below.

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Series C Financing

In March 2024, the Company authorized, issued and sold 18,628,970 shares of Series C Preferred Stock to certain investors at a purchase price of $9.762 per share, for gross proceeds of $181.9 million. The Series C preferred stockholders have senior liquidation preference to the holders of the Preferred Stock upon a deemed liquidation event. The Company is required to obtain certain approvals, including the largest Series C preferred stockholders' vote if a deemed liquidation event would result in a per share payment with respect to each share of Series C Preferred Stock that is less than the Series C Preferred Stock original issuance price. All other material terms are substantively similar to the Preferred Stock (see Note 10). In connection with the financing, the Company increased its authorized shares to 55,200,000 of common stock.

Following the financing, the Company subsequently issued 1,631,350 stock options granted to employees and non-employees.

AbbVie License Agreement

In July 2024, the Company entered into a license agreement (the "AbbVie License Agreement") with AbbVie Global Enterprises Ltd. ("AbbVie"), pursuant to which the Company has been granted an exclusive worldwide, royalty-bearing, sublicensable license to research and develop certain CFTR compounds. The licensed rights are directed, among other things, to three clinical-stage CFTR modulator therapies: galicaftor (ABBV-2222, now referred to as SION-2222), a TMD1-directed CFTR corrector, navocaftor (ABBV-3067, now referred to as SION-3067), a CFTR potentiator, and ABBV-2851 (now referred to as SION-2851), a TMD1-directed corrector. The license granted to us under the AbbVie License Agreement is subject to certain preexisting rights held by AbbVie and Galapagos NV ("Galapagos"). In particular, certain of the licensed patents and other intellectual property rights were developed by or on behalf of Galapagos and are sublicensed to us subject to the terms of the second amended and restated collaboration agreement between Galapagos and AbbVie in October 2018 (the "Galapagos License Agreement"), as amended by a side letter between Galapagos and AbbVie in July 2024.

As initial consideration for the license, the Company paid a non-refundable, upfront payment of $5.0 million and issued 1,414,445 shares of its common stock to AbbVie. The Company determined that the AbbVie License Agreement represented an asset acquisition as it did not meet the definition of the business. The Company recorded the total initial consideration as research and development expense because the acquired license represented in-process research and development with no alternative future use. In addition, the Company is required to pay AbbVie a total of up to $360.0 million upon achievement of certain development and commercial milestones, consisting of up to $70.0 million in late-stage development milestones and up to $290.0 million in commercial milestones. The Company is also required to pay royalties to AbbVie in the low to mid single-digit percentage range based on net sales of licensed products, subject to customary reductions and offsets.

In addition, we are required to pay AbbVie up to $130.0 million in commercial and sales-based milestone payments, mid to high single-digit royalties on the licensed products, or other payments due to Galapagos pursuant to the Galapagos License Agreement, to the extent such payments are triggered by our use of the licensed rights owned by Galapagos under the AbbVie License Agreement.

Stock Split

The Company's Board approved a 1-for-1.4611 reverse stock split of its issued and outstanding common stock, which also resulted in a proportional adjustment to the conversion price for each series of its convertible Preferred Stock, and to the exercise prices and number of shares of common stock underlying the outstanding stock options, which became effective on January 31, 2025. Accordingly, all share and per share amounts for all periods presented in the accompanying financial statements and notes thereto have been retroactively adjusted, where applicable, to reflect the reverse stock split.

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SIONNA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(in thousands, except share and per share data)
(Unaudited)

	September 30, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 35,749	$ 38,521
Marketable securities, current	101,725	—
Prepaid expenses and other current assets	2,863	686
Total current assets	140,337	39,207
Property and equipment, net	2,606	3,107
Marketable securities, noncurrent	43,400	—
Restricted cash	962	962
Operating lease right-of-use asset	8,040	8,625
Other assets	1,920	44
Total assets	$ 197,265	$ 51,945
Liabilities, convertible preferred stock and stockholders' deficit		
Current liabilities:		
Accounts payable	$ 104	$ 719
Accrued expenses	5,347	6,590
Operating lease liability, current	1,027	728
Total current liabilities	6,478	8,037
Operating lease liability, noncurrent	8,968	9,749
Total liabilities	15,446	17,786
Commitments and contingencies (Note 7)		
Series Seed convertible preferred stock, $0.001 par value, 3,963,963 shares authorized, issued and outstanding as of September 30, 2024 and December 31, 2023, liquidation value of $13,200 as of September 30, 2024 and December 31, 2023	13,014	13,014
Series A convertible preferred stock, $0.001 par value, 5,704,161 shares authorized, issued and outstanding as of September 30, 2024 and December 31, 2023; liquidation value of $25,263 as of September 30, 2024 and December 31, 2023	25,235	25,235
Series B convertible preferred stock, $0.001 par value, 11,370,621 shares authorized, issued and outstanding as of September 30, 2024 and December 31, 2023; liquidation value of $111,000 as of September 30, 2024 and December 31, 2023	110,791	110,791
Series C convertible preferred stock, $0.001 par value, 18,628,970 and 0 shares authorized, issued and outstanding as of September 30, 2024 and December 31, 2023, respectively; liquidation value of $181,856 as of September 30, 2024	181,328	—
Total convertible preferred stock	330,368	149,040
Stockholders' deficit:		
Common stock, $0.001 par value, 55,200,000 and 30,000,000 shares authorized as of September 30, 2024 and December 31 2023, respectively; 4,783,261 and 3,313,012 shares issued, 4,663,624 and 3,050,863 shares outstanding as of September 30, 2024 and December 31, 2023, respectively	5	2
Additional paid-in capital	15,911	4,515
Accumulated other comprehensive income	773	—
Accumulated deficit	(165,238)	(119,398)
Total stockholders' deficit	(148,549)	(114,881)
Total liabilities, convertible preferred stock and stockholders' deficit	$ 197,265	$ 51,945

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

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SIONNA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except share and per share data)
(Unaudited)

	Nine Months Ended September 30,	
	2024	2023
Operating expenses:		
Research and development	$ 43,035	$ 30,736
General and administrative	9,388	7,002
Total operating expenses	52,423	37,738
Loss from operations	(52,423)	(37,738)
Other income:		
Interest income	6,051	2,216
Other income	532	135
Total other income	6,583	2,351
Net loss	$ (45,840)	$ (35,387)
Net loss per share—basic and diluted	$ (12.81)	$ (12.20)
Weighted-average common shares outstanding, basic and diluted	3,579,423	2,901,137
Comprehensive loss:		
Net loss	$ (45,840)	$ (35,387)
Other comprehensive income:		
Unrealized gain on marketable securities	773	18
Comprehensive loss	$ (45,067)	$ (35,369)

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

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SIONNA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' DEFICIT
(in thousands, except share amounts)
(Unaudited)

	Series Seed Preferred Stock		Series A Preferred Stock		Series B Preferred Stock		Series C Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount				
Balance as of December 31, 2023	3,963,963	$13,014	5,704,161	$25,235	11,370,621	$110,791	—	$ —	3,050,863	$ 2	$ 4,515	$ —	$ (119,398)	$ (114,881)
Issuance of Series C Preferred Stock, net of issuance costs of $528	—	—	—	—	—	—	18,628,970	181,328	—	—	—	—	—	—
Issuance of common stock from IPR&D acquisition	—	—	—	—	—	—	—	—	1,414,445	2	8,637	—	—	8,639
Exercise of common stock options	—	—	—	—	—	—	—	—	55,806	1	115	—	—	116
Vesting of restricted common stock awards	—	—	—	—	—	—	—	—	142,510	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	2,644	—	—	2,644
Unrealized gain on marketable securities	—	—	—	—	—	—	—	—	—	—	—	773	—	773
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	(45,840)	(45,840)
Balance as of September 30, 2024	3,963,963	$13,014	5,704,161	$25,235	11,370,621	$110,791	18,628,970	$181,328	4,663,624	$ 5	$ 15,911	$ 773	$ (165,238)	$ (148,549)

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

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SIONNA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' DEFICIT
(in thousands, except share amounts)
(Unaudited)

	Series Seed Preferred Stock		Series A Preferred Stock		Series B Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount				
Balance as of December 31, 2022	3,963,963	$13,014	5,704,161	$25,235	11,370,621	$110,791	2,792,929	$ 2	$ 2,225	$ (16)	$ (72,135)	$ (69,924)
Exercise of common stock options	—	—	—	—	—	—	35,552	—	31	—	—	31
Vesting of restricted common stock awards	—	—	—	—	—	—	166,786	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	1,687	—	—	1,687
Unrealized gain on marketable securities	—	—	—	—	—	—	—	—	—	18	—	18
Net loss	—	—	—	—	—	—	—	—	—	—	(35,387)	(35,387)
Balance as of September 30, 2023	3,963,963	$13,014	5,704,161	$25,235	11,370,621	$110,791	2,995,267	$ 2	$ 3,943	$ 2	$ (107,522)	$ (103,575)

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

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SIONNA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)
(Unaudited)

	Nine Months Ended September 30,	
	2024	2023
Cash flows from operating activities:		
Net loss	$ (45,840)	$ (35,387)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	2,644	1,687
Non-cash operating lease expense	1,472	1,557
Amortization of discount on marketable securities	(2,211)	(624)
Non-cash expense related to IPR&D acquisition	8,639	—
Depreciation expense	501	422
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(2,177)	(125)
Accounts payable	(615)	(70)
Accrued expenses	(2,094)	(3,666)
Operating lease liabilities	(1,369)	(41)
Other assets	44	—
Net cash used in operating activities	(41,006)	(36,247)
Cash flows from investing activities:		
Purchases of property and equipment	—	(994)
Purchases of marketable securities	(175,641)	(23,532)
Maturities of marketable securities	33,500	44,750
Net cash (used in) provided by investing activities	(142,141)	20,224
Cash flows from financing activities:		
Proceeds from issuance of Series C convertible preferred stock, net of issuance costs	181,328	—
Payment of deferred offering costs	(1,069)	—
Exercise of common stock options	116	31
Net cash provided by financing activities	180,375	31
Net decrease in cash, cash equivalents and restricted cash	(2,772)	(15,992)
Cash, cash equivalents and restricted cash at beginning of period	39,483	55,799
Cash, cash equivalents and restricted cash at end of period	$ 36,711	$ 39,807
Supplemental cash flow information:		
Right-of use asset obtained in exchange for new operating lease liability	$ —	$ 9,516
Deferred offering costs in accrued expenses	851	—
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	$ 35,749	$ 38,845
Restricted cash	962	962
Total cash, cash equivalents, and restricted cash	$ 36,711	$ 39,807

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

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SIONNA THERAPEUTICS, INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(Unaudited)

1. Nature of the Business

Organization

Sionna Therapeutics, Inc. (the "Company"), formerly known as Sling Therapeutics, Inc., is a clinical-stage biopharmaceutical company dedicated to researching and developing novel medicines for cystic fibrosis. The Company was incorporated in Delaware in August 2019 and is headquartered in Waltham, Massachusetts.

Risks and Uncertainties

The Company is subject to a number of risks common to other companies in the biotechnology industry, including but not limited to, development by competitors of new technological innovations, risks of failure of preclinical studies and clinical trials, development and manufacturing of product candidates, obtaining regulatory approval for product candidates, competition from substitute products, the need to successfully commercialize and gain market acceptance of its product candidates, protection of proprietary technology, dependence on key personnel, the ability to attract and retain qualified employees, reliance on third party organizations' compliance with government regulations, and the need to obtain additional financing. Product candidates currently under development will require significant additional research and development efforts, including extensive clinical testing and regulatory approval, prior to commercialization. These efforts will require significant amounts of additional capital, adequate personnel infrastructure, and extensive compliance-reporting capabilities. Even if the Company's development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

Liquidity and Going Concern

The Company has funded its operations primarily with proceeds from the sale of convertible preferred stock and has not generated revenue from product sales or other sources. As of September 30, 2024, the Company has raised an aggregate of $330.4 million in net proceeds through the sale of convertible preferred stock. The Company has incurred annual net operating losses and has generated negative operating cash flows in every year since inception. As of September 30, 2024, the Company had an accumulated deficit of $165.2 million. The Company expects its operating losses to continue into the foreseeable future as it continues to pursue its research and development efforts.

The Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that these condensed consolidated financial statements are issued. The Company believes that its existing cash, cash equivalents and marketable securities of $180.9 million as of September 30, 2024, will be sufficient to allow the Company to fund operations beyond twelve months from the date that the financial statements are available for issuance.

The Company is seeking to complete an initial public offering ("IPO") of its common stock. Upon the completion of a qualified public offering on specified terms, the Company's outstanding convertible preferred stock will automatically convert into shares of common stock (see Note 9).

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2. Summary of Significant Accounting Policies

The Company's significant accounting policies are disclosed in Note 2, "*Summary of Significant Accounting Policies*," in the audited consolidated annual financial statements for the years ended December 31, 2023 and 2022 included elsewhere in this prospectus. Since the date of those annual financial statements, there have been no changes to the Company's significant accounting policies, except as noted below.

Unaudited Interim Financial Information

The accompanying condensed consolidated balance sheet as of September 30, 2024, and the condensed consolidated statements of operations and comprehensive loss, statements of convertible preferred stock and stockholders' deficit and statements of cash flows for the nine months ended September 30, 2024 and 2023 are unaudited. The condensed consolidated interim financial statements have been prepared on the same basis as the December 31, 2023 and 2022 audited annual financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments necessary for the fair presentation of the Company's financial position as of September 30, 2024 and the results of its operations and its cash flows for the nine months ended September 30, 2024 and 2023. The financial data and other information disclosed in these notes related to the nine months ended September 30, 2024 and 2023 are also unaudited. The results for the nine months ended September 30, 2024 are not necessarily indicative of results to be expected for the year ending December 31, 2024, or for any other subsequent period.

Deferred Offering Costs

The Company capitalizes certain legal, professional accounting and other third-party fees that are direct and incremental costs associated with the planned IPO and other in-process equity financing as deferred offering costs until such financing is consummated. After consummation of the IPO or equity financing, these costs are recorded as a reduction of the proceeds generated as a result of the offering within additional paid-in capital. Should the planned IPO or equity financing be abandoned, the deferred offering costs will be expensed immediately as a charge to operating expenses in the condensed consolidated statements of operations. The Company did not record any deferred offering costs as of December 31, 2023, and recorded deferred offering costs of $1.9 million within other assets as of September 30, 2024.

Recently Issued Accounting Pronouncements Not Yet Adopted

In November 2023, the FASB issued ASU 2023-07, *Segment Reporting (Topic 280: Improvements to Reportable Segment Disclosures)* ("ASU 2023-07"). The amendments in this update improve reportable segment disclosure requirements through enhanced disclosures about significant segment expenses. All disclosure requirements of the update are required for entities with a single reportable segment. The amendments are effective for fiscal years beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024, and should be applied on a retrospective basis to all periods presented. For purposes of its annual reporting requirements, the Company has adopted this standard as of January 1, 2024, but has not yet adopted the standard in these interim financial statements and disclosures. As of September 30, 2024, the Company only has one reportable segment. The Company is currently in the process of evaluating the effects of this pronouncement on the related disclosures.

In December 2023, the FASB issued ASU 2023-09, *Income Taxes (Topic 740: Improvements to Income Tax Disclosures)* ("ASU 2023-09"). ASU 2023-09 provides more transparency about income tax information through improvements to income tax disclosures primarily related to the rate reconciliation

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and incomes taxes paid information. For public companies, the amendments are effective for annual periods beginning after December 15, 2024 and should be applied prospectively. The Company has determined that the effects of adopting the amendments in ASU 2023-09 will not have a material impact on its condensed consolidated financial position, the results of its operations, or related disclosures when such amendment is adopted.

In November 2024, the FASB issued ASU 2024-03, *Income Statement—Reporting Comprehensive Income—Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses ("ASU 2024-03")*, which requires entities to disclose additional information about specific expense categories in the notes to the financial statements. ASU 2024-03 is effective for annual periods beginning after December 15, 2026 and for interim periods within fiscal years beginning after December 15, 2027. Early adoption is permitted. ASU 2024-03 may be applied retrospectively or prospectively. The Company is currently evaluating the effect of this update on its consolidated financial statements and related disclosures.

3. Marketable Securities

The following table summarizes the amortized cost and estimated fair value of the Company's current and noncurrent marketable securities, which are considered to be available-for-sale investments and were included in marketable securities (in thousands):

	September 30, 2024			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Marketable securities, current:				
U.S. Treasury securities	$ 45,956	$ 180	$ —	$ 46,136
Government agency securities	25,265	59	—	25,324
Corporate debt	17,113	49	—	17,162
Commercial paper	13,094	9	—	13,103
Total marketable securities, current	101,428	297	—	101,725
Marketable securities, noncurrent:				
U.S. Treasury securities	26,862	326	—	27,188
Government agency securities	16,062	150	—	16,212
Total marketable securities, noncurrent	42,924	476	—	43,400
Total marketable securities	$144,352	$ 773	$ —	$145,125

The Company has determined that there were no material changes in the credit risk, therefore the Company has not recognized any allowance for credit losses on its debt securities. As of September 30, 2024, the Company holds marketable securities with an aggregate fair value of $43.4 million that had remaining maturities between one and two years. All other securities had a contractual maturity of less than a year. Excluded from the balances above is an aggregate balance of $1.0 million of interest receivable, which is recorded as a component of prepaid expenses and other current assets. There were no realized gains or losses in the nine months ended September 30, 2024 or 2023.

There were no marketable securities held as of December 31, 2023.

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4. Fair Value Measurements

The following table presents information about the Company's financial instruments that are measured at fair value on a recurring basis and indicates the fair value hierarchy of the inputs the Company utilized to determine such fair value (in thousands):

	September 30, 2024			
	Total	Level 1	Level 2	Level 3
Assets				
Cash equivalents:				
Money market funds	$ 21,095	$21,095	$ —	$ —
Commercial paper	2,488	—	2,488	—
Marketable securities:				
U.S. Treasury securities	73,324	73,324	—	—
Government agency securities	41,536	—	41,536	—
Corporate debt	17,162	—	17,162	—
Commercial paper	13,103	—	13,103	—
Total financial assets	$168,708	$94,419	$74,289	$ —

	December 31, 2023			
	Total	Level 1	Level 2	Level 3
Assets				
Cash equivalents:				
Money market funds	$ 23,660	$23,660	$ —	$ —
Commercial paper	989	—	989	—
Total financial assets	$ 24,649	$23,660	$ 989	$ —

During the nine months ended September 30, 2024 and 2023, there were no transfers or reclassifications between fair value measurement levels of assets or liabilities. The carrying values of prepaid expenses and other current assets, accounts payable and accrued expenses approximate their fair values due to the short-term nature of these assets and liabilities.

5. Property and Equipment, net

Property and equipment, net consisted of the following (in thousands):

	September 30, 2024	December 31, 2023
Deposits on lab and office equipment	$ —	$ 116
Laboratory equipment	1,434	1,329
Leasehold improvements	1,854	1,854
Furniture & fixtures	722	722
Total property and equipment	4,010	4,021
Less: accumulated depreciation	(1,404)	(914)
Property and equipment, net	$ 2,606	$ 3,107

Depreciation expense was $0.5 million and $0.4 million for the nine months ended September 30, 2024 and 2023, respectively.

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6. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	September 30, 2024	December 31, 2023
Accrued research and development	$ 1,998	$ 3,065
Accrued compensation and benefits	2,033	3,112
Accrued deferred offering costs	851	—
Accrued professional fees	404	362
Accrued other	61	51
Total accrued expenses	$ 5,347	$ 6,590

7. Commitments and Contingencies

Operating Leases

The Company leases office space under a noncancelable operating lease. There have been no material changes to the Company's lease during the nine months ended September 30, 2024. For additional information, see Note 7, "*Leases*" in the audited consolidated annual financial statements for the years ended December 31, 2023 and 2022 included elsewhere in this prospectus.

Legal Proceedings

The Company was not subject to any material legal proceedings as of September 30, 2024, and the Company is not aware of any material legal proceedings that are currently pending or threatened.

Other Contracts

The Company is party to various contracts with contract research organizations and contract manufacturing organizations that generally provide for termination on notice, with the exact amounts in the event of termination to be based on the timing of the termination and the terms of the agreement.

There were no contractual obligations arising from these arrangements as of September 30, 2024 and 2023.

Indemnification Agreements

As permitted under Delaware law, the Company will indemnify its officers, directors, and employees for certain events or occurrences while the officer, director, or employee is, or was, serving at the Company's request in such capacity. The term of the indemnification is for the officer's or director's lifetime. Further, in the ordinary course of business the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. To date however, the Company has not incurred any significant costs as a result of such indemnifications nor experienced any losses related to them. As of September 30, 2024, the Company was not aware of any claims under indemnification arrangements and does not expect significant claims related to these indemnification obligations and, consequently, concluded that the fair value of these obligations is negligible; therefore, no related reserves were established.

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8. License Agreements

The Company has entered into several license agreements with third parties that typically involve payments from the Company, including up-front payments, milestone payments and royalty payments. The terms and conditions as well as accounting analysis for the Company's significant license agreements are described in Note 9, "*License Agreements*" in the audited consolidated annual financial statements for the years ended December 31, 2023 and 2022 included elsewhere in this prospectus. There have been no other material changes to the terms and conditions or accounting conclusions except as noted below.

AbbVie Agreement

In July 2024, the Company entered into a license agreement (the "AbbVie License Agreement") with AbbVie Global Enterprises Ltd. ("AbbVie"), pursuant to which the Company has been granted an exclusive worldwide, royalty-bearing, sublicensable license to research, develop and commercialize certain CFTR compounds. The licensed rights are directed, among other things, to three clinical-stage CFTR modulator assets. The license granted to us under the AbbVie License Agreement is subject to certain preexisting rights held by AbbVie and Galapagos NV ("Galapagos"). In particular, certain of the licensed patents and other intellectual property rights were developed by or on behalf of Galapagos and are sublicensed to us subject to the terms of the second amended and restated collaboration agreement between Galapagos and AbbVie in October 2018 (the "Galapagos License Agreement"), as amended by a side letter between Galapagos and AbbVie in July 2024.

As initial consideration for the license, the Company paid a non-refundable, upfront payment of $5.0 million and issued 1,414,445 shares of its common stock with a fair value of $8.6 million to AbbVie. The Company determined that the AbbVie License Agreement represented an acquisition of an asset, which was the in-process research and development. The Company recorded the total initial consideration of $13.6 million as research and development expense during the nine months ended September 30, 2024 because the acquired license represented in-process research and development with no alternative future use. In addition, the Company is required to pay AbbVie a total of up to $360.0 million upon achievement of certain development and commercial milestones, consisting of up to $70.0 million in late-stage development milestones and up to $290.0 million in commercial milestones. The Company is also required to pay royalties to AbbVie in the low to mid single-digit percentage range based on net sales of licensed products, subject to customary reductions and offsets. The royalty term will terminate on a product-by-product and country-by-country basis upon the later of (i) the expiration of the last-to-expire valid claim within the relevant licensed patent rights, (ii) the expiration of regulatory exclusivity in such country for such licensed product and (iii) the tenth anniversary of the first commercial sale of a licensed product in such country.

In addition, the Company is required to pay AbbVie up to $130.0 million in commercial and sales-based milestone payments, mid to high single-digit royalties on the licensed products, or other payments due to Galapagos pursuant to the Galapagos License Agreement, to the extent such payments are triggered by the Company's use of the licensed rights owned by Galapagos under the AbbVie License Agreement. As of September 30, 2024, none of such milestones under the AbbVie License Agreement and Galapagos License Agreement have been achieved.

9. Convertible Preferred Stock

The Company has previously issued Series Seed convertible preferred stock (the "Series Seed Preferred Stock"), Series A convertible preferred stock (the "Series A Preferred Stock"), and Series B convertible preferred stock (the "Series B Preferred Stock"). There have been no material changes to the previously issued convertible preferred stock during the nine months ended September 30, 2024. For additional information, see Note 10, "*Convertible Preferred Stock*" in the audited consolidated annual financial statements for the years ended December 31, 2023 and 2022 included elsewhere in this prospectus.

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Series C

In March 2024, the Company issued and sold 18,628,970 shares of Series C convertible preferred stock, par value $0.001 per share ("Series C Preferred Stock") at a purchase price of $9.762 per share, for gross proceeds of $181.9 million (the "Series C Financing"). The Company incurred $0.5 million of issuance costs in connection with the Series C Financing.

Upon the issuance of Series Seed Preferred Stock, Series A Preferred Stock, Series B Preferred Stock and Series C Preferred Stock (the "Preferred Stock"), the Company assessed the embedded conversion and liquidation features of the shares and determined that the Company was not required to separately account for these features.

Preferred Stock consists of the following (in thousands, except share amounts):

	September 30, 2024				
	Authorized	Issued and Outstanding	Carrying Value	Liquidation Value	Common Stock Issuable Upon Conversion
Series Seed Preferred Stock	3,963,963	3,963,963	$ 13,014	$ 13,200	2,712,998
Series A Preferred Stock	5,704,161	5,704,161	25,235	25,263	3,904,017
Series B Preferred Stock	11,370,621	11,370,621	110,791	111,000	7,782,230
Series C Preferred Stock	18,628,970	18,628,970	181,328	181,856	12,749,961
Total	39,667,715	39,667,715	$ 330,368	$ 331,319	27,149,206

	December 31, 2023				
	Authorized	Issued and Outstanding	Carrying Value	Liquidation Value	Common Stock Issuable Upon Conversion
Series Seed Preferred Stock	3,963,963	3,963,963	$ 13,014	$ 13,200	2,712,998
Series A Preferred Stock	5,704,161	5,704,161	25,235	25,263	3,904,017
Series B Preferred Stock	11,370,621	11,370,621	110,791	111,000	7,782,230
Total	21,038,745	21,038,745	$ 149,040	$ 149,463	14,399,245

The holders of Preferred Stock have rights, preferences and privileges which are disclosed within Note 10, "*Convertible Preferred Stock*" in the audited consolidated annual financial statements for the years ended December 31, 2023 and 2022 included elsewhere in this prospectus. There have been no material changes to rights, preferences and privileges during the nine months ended September 30, 2024.

Liquidation Preference

The holders of the Preferred Stock have first preference in the event of any voluntary or involuntary liquidation, dissolution, or winding up of the Company, including a merger or sale of the Company. The amount to be paid is equal to greater of the Series C Preferred Stock original issue price in the case of the Series C Preferred Stock, Series B Preferred Stock original issue price in the case of the Series B Preferred Stock, the Series A Preferred Stock original issue price in the case of the Series A Preferred Stock, and the Series Seed Preferred Stock original issue price in the case of the Series Seed Preferred Stock, plus any dividends declared but unpaid thereon, or such amount per share as would have been payable had all shares of the Preferred Stock been converted into common stock.

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Conversion

Each share of Preferred Stock shall be convertible, at the option of the holder (without payment) into common stock as determined by dividing the Preferred Stock's original issue price by the conversion price in effect at the time of conversion. The Series C Preferred Stock and Series B Preferred Stock conversion price shall initially be equal to $9.762, the Series A Preferred Stock conversion price shall initially be equal to $4.4289, and the Series Seed Preferred Stock conversion price shall initially be equal to $3.33. In the event of a liquidation, dissolution or winding up of the Company or a Deemed Liquidation Event, the conversion right shall terminate at the close of business on the last full day preceding the date fixed for the payment of any such amounts distributable on such event to the holders of Preferred Stock.

All shares of Preferred Stock will automatically convert into shares of common stock upon a qualified initial public offering, which based on the conversion prices currently in effect, would result in a conversion on a one-to-one basis.

10. Common Stock

The voting, dividend and liquidation rights of the holders of the Company's common stock are subject to and qualified by the rights, powers and preference of the holders of the Preferred Stock set forth in Note 10, "*Convertible Preferred Stock*" in the audited consolidated annual financial statements for the years ended December 31, 2023 and 2022 included elsewhere in this prospectus.

The number of shares of common stock that have been reserved for future issuances is as follows:

	September 30, 2024	December 31, 2023
Convertible Preferred Stock	27,149,206	14,399,245
Options to purchase common stock	3,652,051	1,914,362
Remaining shares reserved for future issuance	1,099,636	775,423
Unvested restricted common stock	119,639	262,149
Total	32,020,532	17,351,179

11. Stock-Based Compensation

2020 Stock Option and Grant Plan

The Company's 2020 Stock Option and Grant Plan was amended by the Board of Directors of the Company (the "Board") during the nine months ended September 30, 2024 and provided for the issuance of 55,200,000 shares of common stock as of September 30, 2024.

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Restricted Common Stock

A summary of the Company's restricted common stock activity during the nine months ended September 30, 2024 is presented below:

	Number of Restricted Common Stock Awards	Weighted-Average Per-Share Grant-Date Fair Value
Unvested as of December 31, 2023	262,149	$ 0.78
Vested	(142,510)	$ 0.78
Unvested as of September 30, 2024	119,639	$ 0.78

Common Stock Option Activity

The following table summarizes the Company's common stock option activity under the 2020 Plan for the nine months ended September 30, 2024:

	Number of Common Stock Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding as of December 31, 2023	1,914,362	$ 5.30	7.9	$ 1,549
Granted	1,871,895	6.16		
Exercised	(55,806)	2.07		
Cancelled or forfeited	(78,400)	6.11		
Outstanding as of September 30, 2024	3,652,051	$ 5.77	8.5	$ 8,439
Exercisable as of September 30, 2024	1,289,683	$ 5.26	7.6	$ 3,643

Stock-Based Compensation Expense

The Company recorded stock-based compensation in the accompanying condensed consolidated statements of operations and comprehensive loss as follows (in thousands):

	Nine Months Ended September 30,	
	2024	2023
Research and development expense	$ 1,082	$ 849
General and administrative expense	1,562	838
Total	$ 2,644	$ 1,687

As of September 30, 2024, there was $10.4 million of unrecognized stock-based compensation expense for common stock option awards that are expected to be recognized over a weighted average period of 2.9 years. As of September 30, 2024, there was $0.1 million of unrecognized stock-based compensation expense for restricted common stock awards that are expected to be recognized over a weighted average period of 0.6 years.

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12. Net Loss Per Share

Basic and diluted net loss per share attributable to common stockholders was calculated as follows for the periods presented (in thousands, except share and per share amounts):

	Nine Months Ended September 30,	
	2024	2023
Numerator:		
Net loss	$ (45,840)	$ (35,387)
Net loss attributable to common stockholders, basic and diluted	$ (45,840)	$ (35,387)
Denominator:		
Weighted-average number of common shares used in net loss per share, basic and diluted	3,579,423	2,901,137
Net loss per share of common shares, basic and diluted	$ (12.81)	$ (12.20)

The Company's potentially dilutive securities, which include convertible Preferred Stock, options to purchase common stock, and unvested restricted common stock, have been excluded from the computation of diluted net loss per share as the effect would be to reduce the net loss per share. Therefore, the weighted-average number of common stock outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same.

The Company excluded the following shares from the computation of diluted net loss per share attributable to common stockholders because including them would have had an anti-dilutive effect:

	Nine Months Ended September 30,	
	2024	2023
Convertible Preferred Stock	27,149,206	14,399,245
Options to purchase common stock	3,652,051	2,001,849
Unvested restricted common stock	119,639	317,745
Total	30,920,896	16,718,839

13. Subsequent Events

The Company has evaluated subsequent events after September 30, 2024, through November 26, 2024, the date these financial statements were available to be issued, and February 3, 2025, for the reverse stock split described below.

Stock Split

The Company's Board approved a 1-for-1.4611 reverse stock split of its issued and outstanding common stock, which also resulted in a proportional adjustment to the conversion price for each series of its convertible Preferred Stock, and to the exercise prices and number of shares of common stock underlying the outstanding stock options, which became effective on January 31, 2025. Accordingly, all share and per share amounts for all periods presented in the accompanying financial statements and notes thereto have been retroactively adjusted, where applicable, to reflect the reverse stock split.

2025 Equity Plans

In January 2025, the Board adopted and approved the 2025 Stock Incentive Plan (the "2025 Plan"), which will become effective upon the date immediately preceding the date on which the registration statement for the proposed IPO is declared effective by the SEC. The 2025 Plan provides for the grant of awards with respect to an additional 5,060,000 shares of common stock. Subject to the effectiveness of the 2025 Plan, the Company will cease the grant of additional awards under the 2020 Plan.

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In January 2025, the Board adopted and approved the 2025 Employee Stock Purchase Plan (the "2025 ESPP"), which will become effective upon the date immediately preceding the date on which the registration statement for the proposed IPO is declared effective by the SEC. The 2025 ESPP initially provides participating employees with the opportunity to purchase up to an aggregate of 390,127 shares of common stock.

Grant of stock options under the 2025 Plan

In January 2025, the Board approved the grant of options with service-based vesting criteria for the purchase of an aggregate of 1,946,388 shares of common stock, at an exercise price per share equal to the per share "price to the public" or (equivalent), under the 2025 Plan.

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10,588,233 Shares

Common Stock

Prospectus

Goldman Sachs & Co. LLC	**TD Cowen**	**Stifel**	**Guggenheim Securities**

Until March 3, 2025 (25 days after the date of this prospectus), all dealers that effect transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to the dealers' obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.

February 6, 2025