LeonaBio

2025 Annual Report

Developing Meaningful
New Therapies for Those
Who Need Them Most

To Our Shareholders,

At LeonaBio, we are energized by the transformation our company has undergone and deeply optimistic about what lies ahead.

2025 was a truly pivotal year for LeonaBio. In December, we acquired rights to develop and commercialize lasofoxifene, a late-stage selective estrogen receptor modulator (SERM) currently in a Phase 3 clinical trial for treatment-resistant estrogen receptor-positive, HER2-negative, ESR1-mutated metastatic breast cancer — a patient population with limited options and urgent need. This transaction diversified our pipeline with a late-stage oncology asset and positioned LeonaBio squarely at the intersection of endocrine biology, precision medicine, and combination therapy strategies.

Supported by compelling Phase 2 clinical data, including a median progression-free survival of approximately 13 months in heavily pretreated patients in the combination setting, we believe lasofoxifene has the potential to become the endocrine therapy of choice for patients who have progressed on aromatase inhibitors and CDK4/6 inhibitors. We are advancing the ongoing Phase 3 ELAINE-3 trial with clear conviction and expect to complete enrollment in the fourth quarter of 2026, with topline data anticipated in the second half of 2027.

To fund this vision, we successfully completed a $90 million common stock and warrant private placement financing, co-led by Commodore Capital, Perceptive Advisors, and TCGX, with participation from an exceptional group of blue-chip life science investors. Together with the potential additional $146 million upon exercise of the cash-exercisable warrants, we believe we are well positioned to advance both lasofoxifene and our neurodegenerative disease program through key milestones in 2026 and beyond.

Our ATH-1105 program for ALS also continues to advance meaningfully. In August 2025, we presented encouraging results from our completed Phase 1 first-in-human clinical trial, which demonstrated a favorable safety and tolerability profile, dose-proportional pharmacokinetics, and CNS penetration. We are on track to initiate dosing of ALS patients in a Phase 2 proof-of-concept study in the second half of 2026.

Our transition from Athira Pharma to LeonaBio reflects far more than a name change. Our new name with Greek and Latin roots, meaning lioness, embodies strength, leadership, resilience, and the company's commitment to stand with patients facing the most serious diseases. We now have two clinical-stage programs, an experienced and aligned team, and the financial resources to execute with confidence.

We are grateful for the continued dedication of our clinical collaborators and the patients and families who participate in our trials. Without their courage and commitment, none of this work would be possible.

We enter 2026 with momentum, purpose, and an unwavering belief in what we are building — a company with the potential to change the treatment landscape for patients who need better options.

Sincerely,

Mark J. Litton, Ph.D.
President and Chief Executive Officer, LeonaBio, Inc.

Certain statements contained in this letter may constitute forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. A detailed discussion of such forward-looking statements and the related risks and uncertainties is included in our Annual Report on Form 10-K included herewith.

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-K

(Mark One)

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2025

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934 FOR THE TRANSITION PERIOD FROM TO**

Commission File Number 001-39503

LeonaBio, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**45-3368487**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**

18706 North Creek Parkway, Suite 104
Bothell, Washington 98011
(Address of principal executive officer)
(425) 620-8501
(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Trading Symbol(s)**	**Name of each exchange on which registered**
Common Stock, $0.0001 par value per share	LONA	The Nasdaq Stock Market LLC (The Nasdaq Capital Market)

Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the Registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. YES ☐ NO ☒

Indicate by check mark if the Registrant is not required to file reports pursuant to Section 13 or 15(d) of the Act. YES ☐ NO ☒

Indicate by check mark whether the Registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the Registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. YES ☒ NO ☐

Indicate by check mark whether the Registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the Registrant was required to submit such files). YES ☒ NO ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
Emerging growth company	☐		

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☐

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to §240.10D-1(b). ☐

Indicate by check mark whether the Registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). YES ☐ NO ☒

The aggregate market value of the common stock held by non-affiliates of the registrant, based on the closing price of the shares of common stock on June 30, 2025 (the last business day of the registrant's most recently completed second fiscal quarter) as reported by The Nasdaq Stock Market LLC on such date was approximately $9.9 million. Shares of the registrant's common stock held by each executive officer and director and by each other person who may be deemed to be an affiliate of the registrant have been excluded from this computation. This calculation does not reflect a determination that certain persons are affiliates of the registrant for any other purpose.

The number of shares of Registrant's common stock outstanding as of March 27, 2026 was 9,393,514.

DOCUMENTS INCORPORATED BY REFERENCE

None.

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Summary Risk Factors

Our business is subject to numerous risks and uncertainties, including those highlighted in the section of this report captioned “Risk Factors.” The following is a summary of the principal risks we face:

- We are a clinical-stage biopharmaceutical company with a limited operating history.
- Our approach to inhibiting estrogen receptor signaling in both wild-type and ESR1-mutated breast cancer through treatment by lasofoxifene exposes us to unforeseen risks. We have limited data from preclinical studies and clinical trials to date, and we cannot be certain that future trials will yield data in support of the safety, efficacy and tolerability of our drug candidates.
- Our approach to targeting neurotrophic factors through the use of small molecules like ATH-1105 is based on a novel therapeutic approach, which exposes us to unforeseen risks. We have limited data from preclinical studies and clinical trials to date, including for ATH-1105, and we cannot be certain that future trials will yield data in support of the safety, efficacy and tolerability of our drug candidates.
- Our development of lasofoxifene or ATH-1105 may never lead to marketable products.
- Our ability to generate revenue and achieve profitability depends significantly on our ability to achieve a number of objectives.
- Treatment of central and peripheral nervous system degenerative disorders is a field that has seen very limited success in product development and our research and development efforts may be unsuccessful.
- Clinical development involves a lengthy and expensive process with an uncertain outcome, and results of preclinical studies and earlier clinical trials with a single or few clinical trial sites may not be predictive of eventual safety or effectiveness in large-scale potentially pivotal clinical trials across multiple clinical trial sites. We may encounter substantial delays in clinical trials, or may not be able to conduct or complete clinical trials on the expected timelines, if at all.
- We may not be successful in integrating lasofoxifene into our pipeline of product candidates.
- We have no marketed proprietary products and have not yet completed any Phase 3 clinical trials, which makes it difficult to assess our ability to develop our future product candidates and commercialize any resulting products independently.
- We may expend our limited resources to pursue a particular drug candidate or indication and fail to capitalize on drug candidates or indications that may be more profitable or for which there is a greater likelihood of success.
- We have been and may in the future be subject to claims, lawsuits, arbitration proceedings, government investigations, securities class action litigation and other legal, regulatory and administrative proceedings and face potential liability and expenses related thereto, which could divert management's attention, and insurance coverage may not be sufficient to cover all costs and damages. This could have a material adverse effect on our business, operating results and financial condition.
- Any “topline”, interim, initial, or preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.
- Our reporting of topline or final data for our clinical trials may be delayed and our regulatory submissions or receipt of necessary marketing approvals could be delayed or prevented.

- We face significant competition, and if our competitors develop and market technologies or products more rapidly than we do or that are more effective, safer, or less expensive than the drug candidates we develop, our commercial opportunities will be negatively impacted.
- We will require substantial additional funding to finance our operations, complete the development and commercialization of our product candidates and develop and commercialize other current and potential drug candidates. If we are unable to raise this funding and access capital when needed, we may be forced to delay, reduce, or eliminate our drug product development programs, commercialization efforts or other operations.
- As a result of our failure to timely file a Current Report on Form 8-K, we are currently ineligible to file or use registration statements on Form S-3, which may impair our ability to raise capital on terms favorable to us, in a timely manner or at all.
- We have incurred significant losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future.
- The regulatory approval processes of the U.S. Food and Drug Administration (the "FDA") and other comparable foreign regulatory authorities are lengthy, time consuming and inherently unpredictable. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals for our drug candidates, we will not be able to commercialize, or will be delayed in commercializing, our drug candidates, and our ability to generate revenue will be materially impaired.
- If we engage in future acquisitions or strategic partnerships, this may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities, and subject us to other risks.
- We rely on third parties to conduct our nonclinical studies and clinical trials. If these third parties do not properly and successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval of or commercialize our drug candidates.
- Even if approved, our drug candidates may not achieve adequate market acceptance among physicians, patients, healthcare payors and others in the medical community necessary for commercial success.
- We have never commercialized a drug candidate before and may lack the necessary expertise, personnel and resources to successfully commercialize any drug products on our own or together with suitable collaborators.
- Our success depends on our ability to protect our intellectual property and our proprietary technologies.
- If the scope of any patent protection we obtain is not sufficiently broad or the term is not sufficiently long, or if we lose any of our patent protection, our ability to prevent our competitors from commercializing similar or identical product candidates would be adversely affected.
- Intellectual property discovered or developed through government funded programs may be subject to federal regulations such as “march-in” rights, certain reporting requirements and a manufacturing preference for U.S.-based companies. Compliance with such regulations may limit our exclusive rights and limit our ability to contract with non-U.S. manufacturers, which could adversely affect our ability to successfully develop and commercialize our drug products.

- The market price of our common stock has been and may continue to be volatile, which could result in substantial losses for investors.
- Future sales, or the perception of future sales, of a substantial amount of our common stock could depress the trading price of our common stock.
- We and certain of our directors and executive officers have previously been, and may in the future be, named as defendants in lawsuits that could result in substantial costs and divert management's attention.
- Actions by activist stockholders have in the past been, and may in the future be, disruptive and could cause uncertainty about the strategic direction of our business.
- The loss of any of our key personnel could significantly harm our business, results of operations and competitive position.

Our Risk Factors are not guarantees that no such conditions exist as of the date of this report and should not be interpreted as an affirmative statement that such risks or conditions have not materialized, in whole or in part.

PART I

Special Note Regarding Forward-Looking Statements

This Annual Report on Form 10-K contains forward-looking statements that are based on our management's beliefs and assumptions and on information currently available to our management. This section should be read in conjunction with our audited consolidated financial statements and related notes included in Part II, Item 8 of this report. The statements contained in this report that are not purely historical are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act").

In some cases, you can identify forward-looking statements by the following words: "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "ongoing," "plan," "possible," "potential," "predict," "project," "should," "target", "will," "would" or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words. You should read these statements carefully because they discuss future expectations, contain projections of future results of operations or financial condition, or state other "forward-looking" information. These statements relate to our future plans, objectives, expectations, intentions and financial performance and the assumptions that underlie these statements. These forward-looking statements include, but are not limited to, statements about:

- the sufficiency of our existing cash, cash equivalents and investments to fund our future operating expenses and capital expenditure requirements;
- our financial performance;
- our ability to obtain funding for our operations, including funding necessary to develop and commercialize our drug candidates and funding necessary for the payment of future milestone and other payments that may be earned by our collaboration partners;
- the ability of our nonclinical studies and clinical trials to demonstrate safety and efficacy of our drug candidates;
- the success, cost and timing of our development activities, nonclinical studies and clinical trials;
- our potential to complete the Phase 3 ELAINE-3 clinical trial for lasofoxifene and any subsequent clinical trials to show the clinical benefits of lasofoxifene;
- the potential of our Phase 2 ATH-1105 clinical trial and any subsequent clinical trials to show the beneficial characteristics, safety and efficacy of ATH-1105;
- the rate and degree of market acceptance of our drug candidates;
- the potential learnings from our ongoing clinical trials and their ability to inform and improve future clinical development plans;
- the timing and focus of our future clinical trials, and the reporting of data from those trials;
- anticipated milestone timelines, such as the timing of data releases, and our ability to meet such timelines;
- our plans relating to commercializing our drug candidates, if approved;

- our plans and ability to establish sales, marketing and distribution infrastructure to commercialize any drug candidates for which we obtain approval;
- our ability to attract and retain key managerial, scientific and clinical personnel and the expected contributions of such personnel;
- our ability to contract with third-party suppliers and manufacturers and their ability to perform adequately;
- the potential for lasofoxifene to be a new standard of care in the genetically defined patient group;
- the accuracy of our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;
- the pricing and reimbursement of our drug candidates, if approved;
- our reliance on third parties to conduct clinical trials of our drug candidates, and for the manufacture of our drug candidates for nonclinical studies and clinical trials;
- the success of competing therapies that are or may become available;
- the beneficial characteristics, safety and efficacy of our drug candidates;
- regulatory developments in the United States and other jurisdictions;
- our ability to obtain and maintain regulatory approval of our drug candidates in the United States and other jurisdictions, and any related restrictions, limitations or warnings in the label of any approved drug candidate;
- future agreements with third parties in connection with the commercialization of our drug candidates;
- our plans, capacity and capability relating to the further development and manufacturing of our drug candidates, including additional indications for which we may pursue regulatory approval;
- our plans and ability to obtain or protect intellectual property rights, including extensions of existing patent terms where available;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our drug candidates and technology;
- the outcome of legal proceedings which may in the future be instituted against us and certain of our directors and officers;
- the actions by activist stockholders, which have in the past been, and may in the future be, disruptive and could cause uncertainty about the strategic direction of our business;
- the size and growth potential of the markets for our drug candidates, if approved for commercial use, and our ability to serve those markets; and
- the potential benefits of any strategic collaborations, partnerships, or other transactions we may enter into.

These forward-looking statements are subject to certain risks and uncertainties that could cause actual results to differ materially from those anticipated in the forward-looking statements. Factors that might cause such a difference include, but are not limited to, those discussed in this report in Part I, Item 1A — “Risk Factors,” and elsewhere in this report. These statements, like all statements in this report,

speak only as of their date, and we undertake no obligation to update or revise these statements in light of future developments. Our Risk Factors are not guarantees that no such conditions exist as of the date of this report and should not be interpreted as an affirmative statement that such risks or conditions have not materialized, in whole or in part.

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 report, and although 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 a thorough 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.

This report includes our trademarks and registered trademarks, including LeonaBio, the LeonaBio logo, and other trademarks, trade names, or service marks of LeonaBio. Each other trademark, trade name or service mark appearing in this report belongs to its holder. Solely for convenience, trademarks, trade names, and service marks referred to in this report are listed without ® or TM symbols, but we will assert, to the fullest extent under applicable law, our or the rights of the applicable licensors to these trademarks, trade names, and service marks.

In this report, "we," "our," "us," "LeonaBio," and "the Company" refer to LeonaBio, Inc. and its wholly-owned subsidiary.

Item 1. Business.

Overview

We are a clinical-stage biopharmaceutical company dedicated to the development of novel therapeutics for high unmet medical needs, including treatment-resistant metastatic breast cancer and amyotrophic lateral sclerosis ("ALS"), with the goal of improving patients' lives.

Following a review of potential therapeutic and business opportunities, we acquired rights to a promising late-stage asset, lasofoxifene, for the potential treatment of ESR1-mutated ("mESR1") metastatic breast cancer in December 2025. This late-stage program has generated promising clinical data and we believe it represents a unique and exciting opportunity to diversify our pipeline. We believe this program fits well with our later-stage clinical development experience, and complements our in-house asset, ATH-1105, a Phase-2 ready program for the potential treatment of ALS.

The drug development landscape in both oncology and neuroscience is evolving rapidly, driven by breakthroughs in genetics, disease biology, and molecular pathway research. We are leveraging this scientific momentum to advance a pipeline of innovative, late-stage assets both internally developed and strategically in-licensed with the goal of accelerating their path to market and maximizing their clinical and commercial impact.

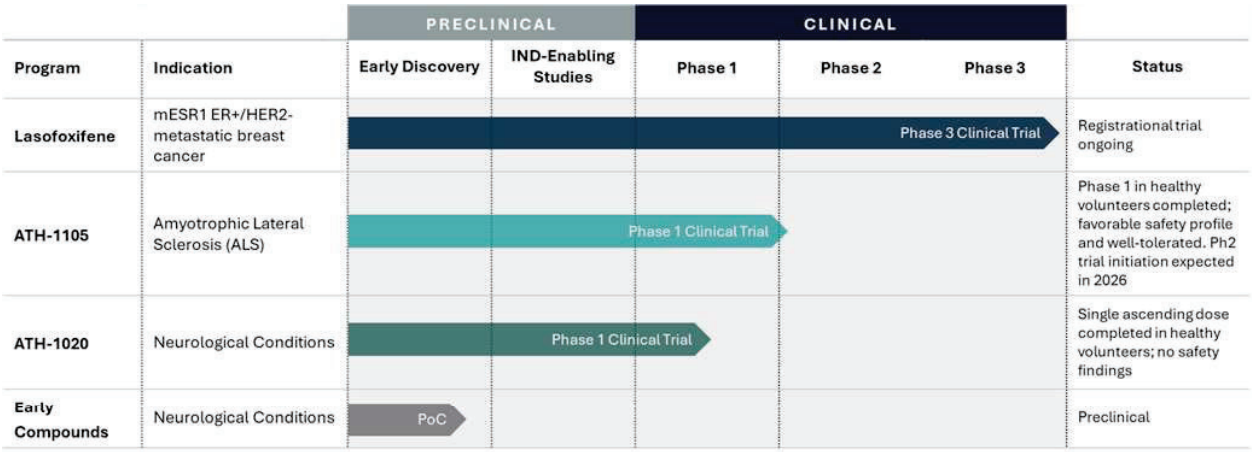
Our lead drug candidates, lasofoxifene and ATH-1105, are novel, small molecule therapies with the potential to address devastating diseases where current treatment options are limited or ineffective. With a strong commitment to scientific excellence and patient-centered innovation, we aim to advance meaningful new therapies that are designed to treat patients with treatment-resistant metastatic breast cancer and ALS.

Our Pipeline

Figure 1 below illustrates the current development stage of our proprietary drug candidates and early discovery and development programs, of which only lasofoxifene and ATH-1105 are currently in clinical development. Each program targets unmet needs in either oncology or neurology, leveraging

differentiated mechanisms of action supported by preclinical and clinical evidence. Lasofoxifene, a selective estrogen receptor modulator ("SERM"), is currently being evaluated in an ongoing registrational Phase 3 trial for metastatic breast cancer with ESR1 mutations. ATH-1105, a small-molecule positive modulator of the neurotrophic HGF system, has completed a first-in-human Phase 1 trial, and planning for a Phase 2 clinical trial for the treatment of ALS is underway. We aim to advance these programs with a focus on disciplined clinical strategy and execution, data-driven decision making, and potential commercialization following receipt of applicable regulatory approvals. We are exploring the use of our drug candidates that enhance the neurotrophic HGF system, including ATH-1020, with the goal of improving neuronal health and function in multiple neurological diseases. In addition, our drug discovery efforts have focused on designing and testing new early compounds directed towards novel targets for a variety of clinical applications.

Figure 1. Summary of Our Preclinical and Clinical Programs.



Lasofoxifene

Lasofoxifene is a highly potent, orally bioavailable, small molecule, selective estrogen receptor modulator that inhibits estrogen receptor signaling in both wild-type ("WT") and mESR1 breast cancer.

Lasofoxifene was discovered through a research collaboration between Pfizer Inc. ("Pfizer") and Ligand Pharmaceuticals ("Ligand"). Pfizer continued development of the drug in osteoporosis, female sexual dysfunction, and vaginal atrophy. In 2009, lasofoxifene was approved by the European Medicines Agency ("EMA") in Europe for the treatment of osteoporosis, under the drug name Fablyn. Multiple NDAs were submitted by Pfizer for the prevention of osteoporosis, and vulvar and vaginal atrophy, but no approvals were granted in the United States for these indications. Pfizer determined not to launch Fablyn in Europe and marketing authorization was withdrawn automatically under the Sunset Provision in 2012. Currently, lasofoxifene is not authorized for marketing anywhere in the world.

In 2011, Pfizer returned global rights of lasofoxifene to Ligand. In 2015, Sermonix Pharmaceuticals, Inc. ("Sermonix") subsequently in-licensed exclusive rights to oral lasofoxifene from Ligand, and in 2016, in-licensed intellectual property in ESR1 mutations from Duke University. In 2018, patents were issued for lasofoxifene in ER+ breast and ovarian cancers with an ESR1 mutation. Sermonix initiated a Phase 2/3 development program of lasofoxifene for the treatment of men or women with locally advanced or metastatic estrogen receptor-positive (ER+) breast cancer with an ESR1 mutation (ELAINE-1 and ELAINE-2 studies). In May 2019, the FDA granted lasofoxifene Fast Track Designation. A pivotal Phase 3 trial (ELAINE-3) is underway with topline data expected in the second half of 2027. The primary analysis

will be performed after 285 progression-free survival ("PFS") events per RECIST 1.1 criteria are observed by blinded independent central review.

In December 2025, we in-licensed lasofoxifene globally, excluding Asia and certain countries in the Middle East. We plan to complete the ELAINE-3 trial to position lasofoxifene for potential approval in the United States and Europe, if successful.

We are amending the ELAINE-3 trial protocol to increase the sample size from 500 participants to up to 600 participants. The primary goal of the amendment is to help ensure that the trial will have the appropriate number of disease progression events.

Mechanism of Disease

Estrogen receptor–positive (“ER^+”) breast cancer accounts for roughly 70% of all breast cancer cases and relies on estrogen-mediated signaling for tumor growth and survival. Endocrine therapy, through aromatase inhibitors (“AIs”), selective estrogen receptor modulators, or selective estrogen receptor degraders (“SERDs”), remains the standard of care, but a majority of patients with metastatic disease eventually develop resistance. A key mechanism underlying this acquired resistance is mutation of the ESR1 gene, which encodes the estrogen receptor alpha (ERα). ESR1 mutation arise primarily due to the selective pressure from endocrine therapies. While endocrine therapies are initially effective, over time, a portion of tumor cells enter a quiet state where they rewire survival pathways, often in the form of ESR1 mutations that are resistant to therapies. The result is persistent estrogen-mediated activation, uncontrolled cell proliferation, and metastatic potential despite ongoing endocrine therapy. Tumors harboring these mutations exhibit diminished sensitivity to standard agents such as fulvestrant, leading to relapse after CDK4/6 inhibitor–based combinations and limited PFS in the post-CDK4/6 setting, highlighting a critical unmet need.

Mechanism of Action

Lasofoxifene is a next-generation selective estrogen receptor modulator that inhibits estrogen receptor signaling in both WT and mESR1 breast cancer. Unlike other endocrine agents that lose potency in the presence of ESR1 mutations such as Y537S and D538G, lasofoxifene maintains full inhibitory activity by stabilizing the receptor in an inactive conformation. Structural and preclinical data show that lasofoxifene disrupts the activation helix within the ER ligand-binding domain, preventing downstream estrogen signaling. This mechanism effectively shuts down the constitutive signaling that drives proliferation and metastasis in mESR1 tumors.

In preclinical models, lasofoxifene demonstrated superior antitumor activity compared with fulvestrant, both as a single agent and in combination with CDK4/6 inhibitors, achieving robust tumor responses. Clinically, lasofoxifene acts as a tissue-selective ER modulator – antagonistic in breast tissue while maintaining beneficial estrogenic activity in bone tissues, supporting an improved tolerability and quality-of-life profile compared to SERDs. Lasofoxifene has also demonstrated excellent combinability with targeted agents such as CDK4/6 or PI3K inhibitors.

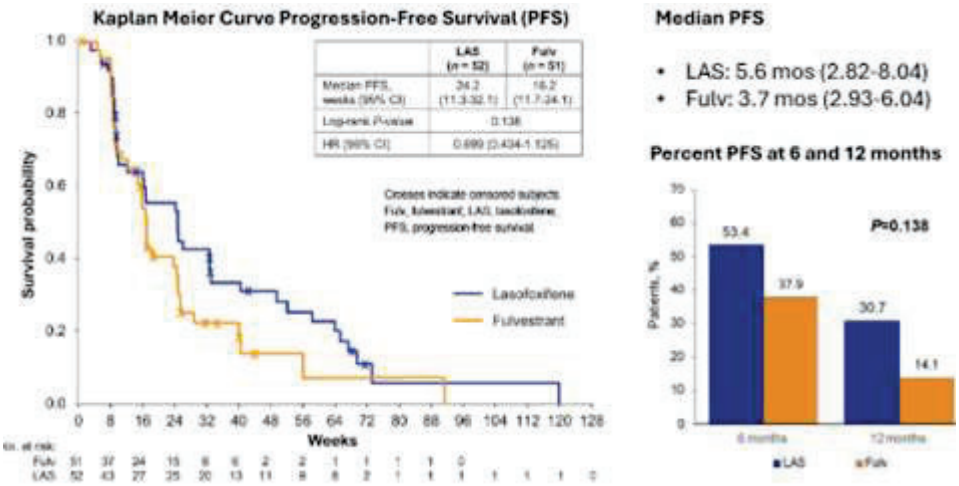
Through this unique receptor-stabilizing mechanism, lasofoxifene represents a mechanistically distinct and potent strategy to overcome ESR1-driven endocrine resistance and re-establish durable ER pathway control in metastatic $ER^+/HER2^-$ breast cancer.

Clinical Evidence for Treatment of mESR1 Metastatic Breast Cancer

A robust clinical efficacy and safety database was generated by Pfizer during their development of lasofoxifene, including 23 Phase 1 trials, 11 Phase 2 trials, and 6 Phase 3 trials. These data position lasofoxifene as one of the more well-characterized and novel endocrine therapies under development for advanced mBC. We are leveraging the existing data to advance the development of lasofoxifene. Of note, over 5,000 patients were treated with lasofoxifene in Pfizer's Phase 3 (PEARL; NCT00141323) osteoporosis trial for up to 5 years, which demonstrated reduction in bone fractures and a reduction in new-onset breast cancer. After 5 years, women treated with 0.5 mg lasofoxifene had an 81% ($p<0.001$) decrease in risk of ER+ breast cancer.

ELAINE-1 (NCT03781063) was an open-label, randomized, multicenter, Phase 2 trial, designed as a proof-of-concept of lasofoxifene compared to fulvestrant for the treatment of pre- and postmenopausal women with locally advanced or metastatic ER+/HER2− breast cancer with an acquired ESR1 mutation and who have disease progression on an AI in combination with a cyclin dependent kinase (CDK) 4/6 inhibitor. The primary outcome measure was PFS and the median PFS was 5.6 and 3.7 months for lasofoxifene treated participants and fulvestrant treated participants, respectively (Figure 12). Lasofoxifene and fulvestrant were both safe and well tolerated, with no significant differences between groups. While the trial was not powered, it sufficiently demonstrated the potential benefit of monotherapy lasofoxifene.

Figure 2. Kaplan-Meier estimates of PFS for lasofoxifene versus fulvestrant.

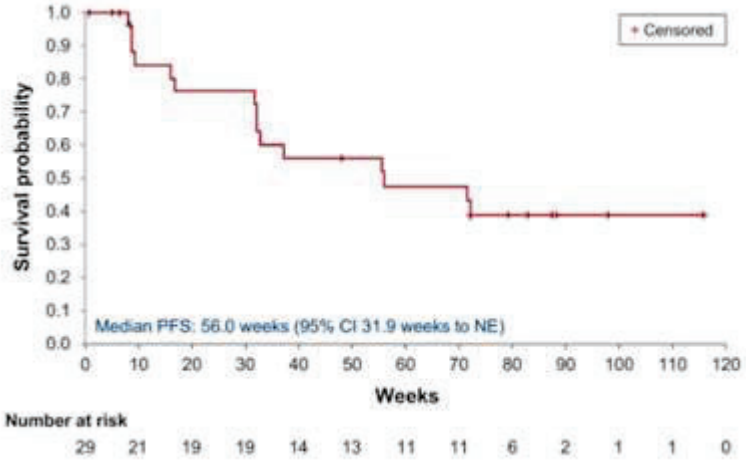


Crosses indicate censored patients. CI, confidence interval; Fulv, fulvestrant; HR, hazard ratio; LAS, lasofoxifene.

ELAINE-2 (NCT04432454) was an open-label, multicenter, single-arm Phase 2 trial, designed to evaluate the safety and tolerability of the lasofoxifene and abemaciclib (a subsequently approved treatment) combination for the treatment of pre- and postmenopausal women with locally advanced or metastatic ER+/HER2− breast cancer with an acquired ESR1 mutation who have disease progression while on first and/or second lines of hormonal treatment. The combination treatment of lasofoxifene with abemaciclib demonstrated a 13-month (56 weeks) median PFS, which we believe is the longest duration observed in 2L/3L post-CDK4/6i patients (Figure 13). The combined treatment delivered a high tumor response (56% ORR) and durable disease control (65.5% CBR). The deep ctDNA and ESR1 clearance correlated with PFS, which is suggestive of target engagement and clinical benefits for patients with co-

mutations. Safety outcomes of participants treated with abemaciclib and lasofoxifene resembled the established safety profile of abemaciclib and lasofoxifene, separately, with no notable increases in adverse event frequency.

Figure 3: Kaplan-Meier analysis of PFS.



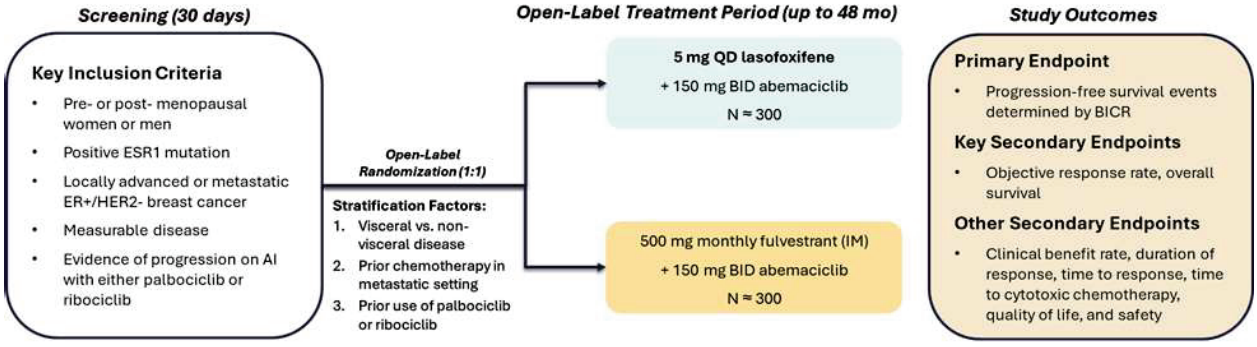
CI, confidence interval; NE, not estimable; PFS, progression-free survival.

Clinical Development Plan for Lasofoxifene in mESR1 Metastatic Breast Cancer

In September 2022, an End of Phase 2 (EOP2) meeting was held with the FDA. Following the completion of ELAINE-2, these data were discussed with FDA and a pivotal, global Phase 3 trial protocol (ELAINE-3; NCT05696626) to evaluate lasofoxifene with abemaciclib was agreed upon. ELAINE-3 aims to demonstrate the potential superiority of lasofoxifene with abemaciclib, as a new standard-of-care versus fulvestrant with abemaciclib for ESR1-driven post-CDK4/6i metastatic breast cancer (Figure 4). The primary endpoint is determined by a blinded independent review of centralized scans using industry standard RECIST criteria. The primary readout will be conducted when 285 participants have been determined to meet the RECIST criteria for progression.

ELAINE-3 was initiated in 2024. As of December 2025, over 50% of recruitment was completed and we anticipate completion of recruitment in 2026.

Figure 4: ELAINE-3 Trial Design



Market Opportunity

Current effective treatment options for ER+/HER2-, ESR1 mutated breast cancer remain limited, particularly after progression on AIs and CDK4/6 inhibitor therapy.

We believe that if lasofoxifene, in combination treatment with abemaciclib (Verzenio), demonstrates a competitive PFS, a substantial objective response rate, and a favorable tolerability and quality of life profile, it will represent a compelling profile for approval and commercialization. In a quantitative survey of 50 physicians, Sermonix reported that 86% of physicians indicated that they definitely would or probably would prescribe a lasofoxifene combination regimen to treat patients with ER+/HER2- advanced breast cancer with an ESR1 mutation. We believe lasofoxifene, if approved, will represent a desirable commercial option for patients with ER+/HER2-, ESR1 mutated breast cancer. Based on the prevalence of ER+/HER2- mBC cases in 2L+ therapy and rate of ESR1 mutations, our market research suggests that the U.S. market opportunity for lasofoxifene may exceed $1.0 billion in peak annual sales.

Potential Commercialization Plan

Lasofoxifene is currently in development for the potential treatment of ER+/HER2-, ESR1 mutated metastatic breast cancer, a genetically defined, high-value market segment with limited effective options following AI and CDK4/6 inhibitor therapy. The potential commercialization strategy is centered around lasofoxifene as a 2L/3L+ therapy in combination with abemaciclib (CDK4/6i) after progression on endocrine therapy + CDK4/6i. We aim to demonstrate that lasofoxifene provides compelling combination efficacy while offering quality-of-life advantages unmatched by current endocrine therapies. If successful, we may evaluate lasofoxifene in earlier-line use and in combinations with other targeted therapies such as mTOR or PI3K pathway inhibitors.

ATH-1105

ATH-1105 is a novel, orally available, brain-penetrant, next generation, small-molecule drug candidate designed to positively modulate the neurotrophic HGF system. We conducted a first-in-human Phase 1 double-blind, placebo-controlled trial that enrolled 80 healthy volunteers to evaluate single and multiple oral ascending doses of ATH-1105. The trial was completed in November 2024 and evaluated the safety and tolerability of ATH-1105 and included measurements of pharmacokinetic outcomes. The results of the Phase 1 trial showed that ATH-1105 demonstrated a favorable safety profile and was well-tolerated in healthy volunteers, supporting continued clinical development.

In preclinical models of ALS, treatment with ATH-1105 resulted in improvements in motor neuron survival and function. In vitro, ATH-1105 treatment protected primary spinal motor neurons from glutamate toxicity and prevented accumulation of toxic protein aggregates. Additionally, ATH-1105 induced target activation in cultures of ALS patient-derived motor neurons. In a preclinical mouse model of ALS, treatment with ATH-1105 significantly prevented loss of body weight, and improved motor function including balance, coordination and muscle strength. We additionally reported that treatment with ATH-1105 significantly improved electrophysiological measures of functional nerve signaling and protected against motor neuron axon degeneration and demyelination. ATH-1105 treatment also significantly reduced biomarkers of inflammation and neurodegeneration and prolonged survival. Study results were presented at the Motor Neurone Disease Association International Symposium in December 2022 and 2023, and published in *Frontiers in Neuroscience*, 2024. Weight loss, motor deficits, inflammation, loss of functional nerve signaling, and motor neurodegeneration and demyelination are all hallmarks of ALS disease; treatment with ATH-1105 significantly improved all of these aspects in the preclinical models tested.

ATH-1020

ATH-1020 is a novel, orally available, next generation, small molecule drug candidate designed to positively modulate the neurotrophic HGF system. This compound was originally assessed for neuropsychiatric indications in preclinical models as presented at the American Society for Experimental Therapeutics Annual Conference in February 2022.

In preclinical models of diabetic neuropathic pain, ATH-1020 demonstrated significant improvements in two aspects of disease that are prominent symptoms in people suffering from neuropathic pain: increased sensitivity to mechanical and thermal stimulation. The significant improvements in neuropathic pain were partially sustained after seven days of not receiving ATH-1020, suggesting persistent and potentially disease-modifying effects. Data from these studies were presented at the Society for Neuroscience Annual Conference in November 2022 and the American Society for Experimental Neurotherapeutics in March 2023. We have completed the SAD escalation portion of the Phase 1 trial, in which ATH-1020 demonstrated a favorable safety profile and was well-tolerated in healthy volunteers.

Early Compounds

In addition to the compounds described above, we have evaluated several other compounds in preclinical discovery and development for neurological diseases and other indications where we believe positive modulation of the neurotrophic HGF system may have therapeutic potential and we currently have novel compounds in early development for several molecular targets relevant to various clinical applications.

Positive Modulators of the Neurotrophic HGF System

Mechanism of Disease

Causes of neurodegenerative diseases are not fully understood as they are complex with several contributing factors including inflammation, oxidative stress, neurotoxicity, excitotoxicity, synaptic dysfunction, and protein pathologies that ultimately lead to neuronal damage, neuronal network degeneration and a decline in function. Intrinsic to these diseases is the disruption of a healthy neuronal network that can be overcome and repaired or maintained when the body's natural repair mechanisms are intact. However, a loss of or reduction in ability of the body to repair itself can lead to dysregulation that then manifests as symptoms and overall functional decline. One such naturally occurring repair mechanism is the neurotrophic HGF system.

Scientific evidence supporting the neurotrophic HGF system as a naturally occurring repair mechanism is backed by over 30 years of research. MET is one of the most stably expressed genes in the adult human brain and is essential to a healthy, functional nervous system. HGF/MET signaling plays a critical role in both the development and maintenance of nervous system tissue. In ALS, studies have highlighted the importance of HGF in key processes affected by the disease. HGF functions as a potent survival factor for motor neurons, and impaired HGF/MET signaling has detrimental effects on neuromuscular junction integrity. Preclinical studies in ALS animal models show that HGF delivery reduces motor neuron degeneration, improves motor function, and prolongs lifespan. Additionally, HGF signaling has been linked to muscle function, the loss of which is a key feature of ALS. And although evidence supports the neurotrophic HGF system as an attractive target for combating neurodegenerative diseases, such as ALS, with its multimodal mechanism of action, it has proven a difficult drug target. There are approved and in-development gene therapy approaches to increase HGF expression beyond

normal physiological levels, but these are limited as potentially viable treatment options for neurological disorders due to limited distribution and more invasive delivery requirements, such as intrathecal or intravenous, or locally restricted to the periphery, such as via intramuscular routes of administration.

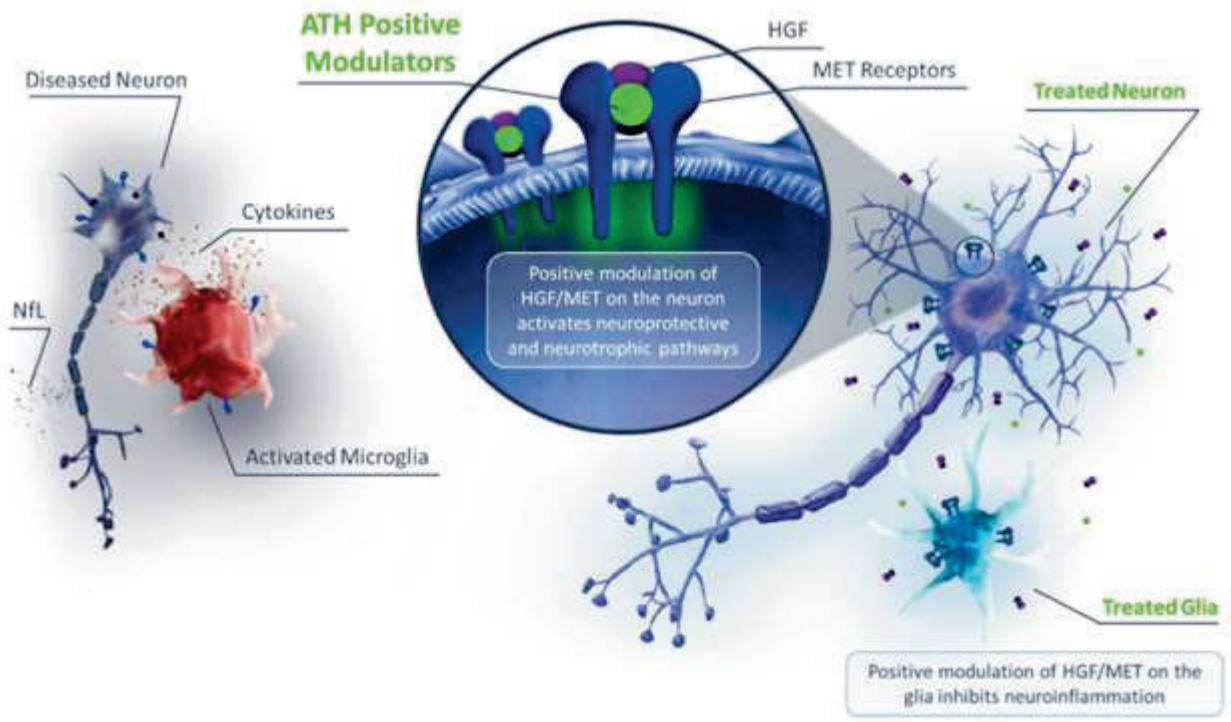
To date, drug developers have been deploying approaches that typically address only a single factor of the cascade of pathologies that lead to neurodegeneration, and translating early successful results to meaningful clinical benefit has been mixed. We believe that addressing such complex and multifactorial diseases requires a novel multimodal approach, such as targeting the neurotrophic HGF system through non-invasive small molecules that enhance levels of HGF system activation to protect and repair neuronal networks.

Mechanism of Action

We have developed a pipeline of proprietary small molecule compounds ("ATH positive modulators") designed to enhance the neurotrophic HGF system and promote its neuroprotective, neurotrophic and anti-inflammatory effects, including protection of neurons from a variety of insults. These novel small molecules are designed to cross the blood-brain barrier for CNS disorders or remain in the periphery for PNS and other indications, and mechanistically produce a series of multimodal effects that support their therapeutic promise to: reduce inflammation, promote regeneration, provide neuroprotection, and, ultimately, slow disease progression.

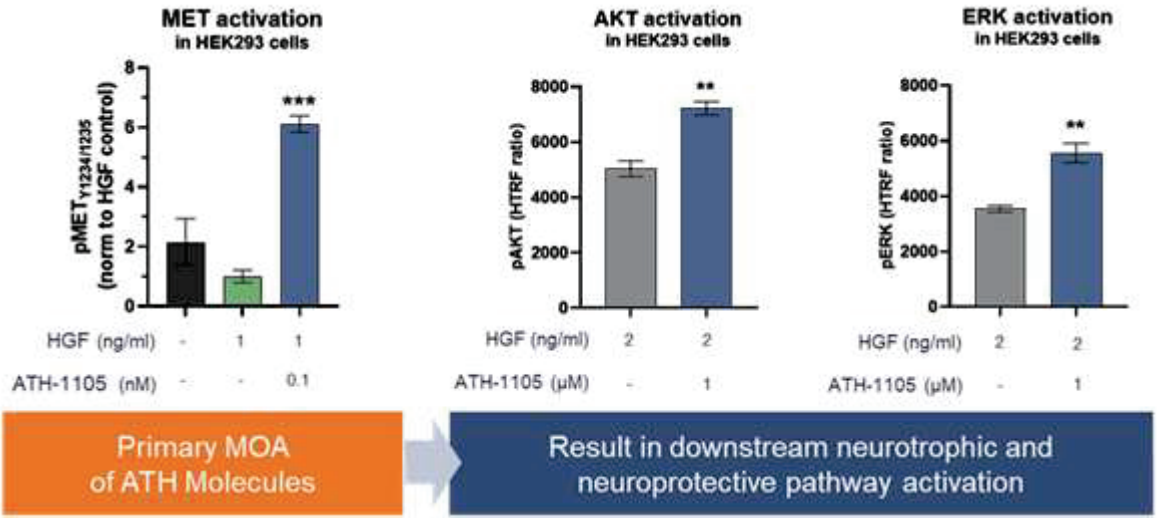
Figure 5 below illustrates the hypothesized mechanism of action of ATH positive modulators and the cellular disease state where diseased neurons generate a biomarker of neurodegeneration, NfL, as well as other signature markers of neuronal damage. Contributing to a diseased neuron are also proinflammatory cytokines produced by activated microglia. In the treated neuron state where ATH positive modulators are designed to enhance HGF/MET signaling to promote neuroprotective and neurotrophic pathways, reduced neuron degeneration and NfL production occur while the treated glia show reduced activation and production of proinflammatory cytokines, illustrating the potential reduction in neurodegeneration and inhibition of neuroinflammation through the enhancement of the neurotrophic HGF system.

Figure 5. Hypothesized Mechanism of Action of ATH Positive Modulators.



As ATH positive modulators interact with the HGF system, neurotrophic and neuroprotective pathways are activated downstream, including the activation of the extracellular-signal regulated kinase (“ERK”) and protein kinase B ("AKT pathways") which play critical roles in protecting neurons from damage and death, including from oxidative stress, excitotoxicity, and apoptosis. Figure 6 below shows cell culture data demonstrating that one of the key mechanisms of action of ATH positive modulators is through activation of AKT and ERK via MET activation.

Figure 6. ATH-1105 enhances HGF signaling and promotes neurotrophic and neuroprotective pathways.



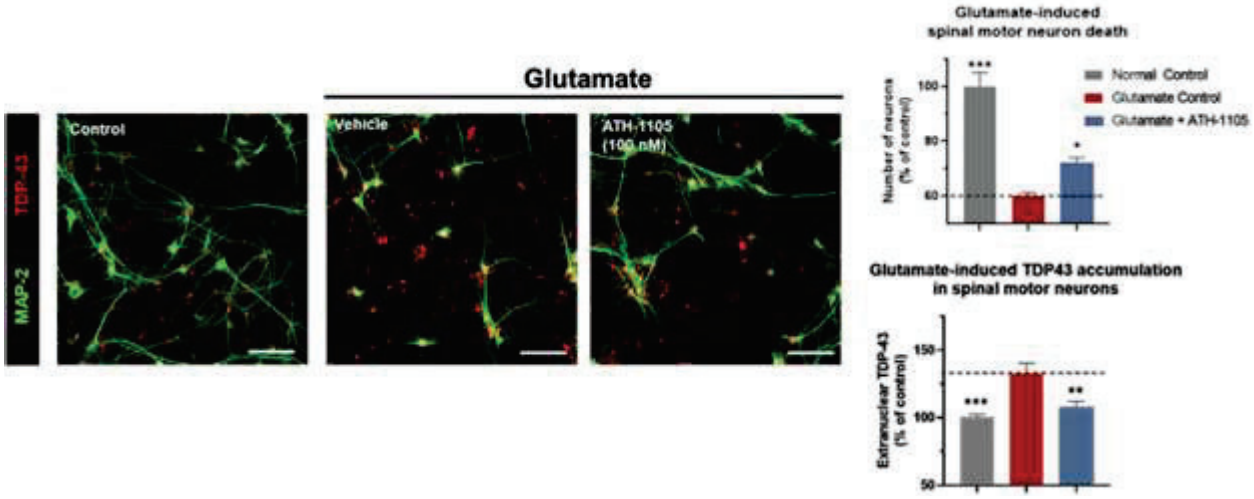
Positively modulating the neurotrophic HGF system promotes its multimodal effects, which we believe can potentially address the complex pathology in neurodegenerative diseases. These positive multimodal effects taken together may lead to improvement in function.

ATH-1105

ATH-1105 Preclinical Evidence for ALS

ATH-1105 is a novel oral small molecule drug candidate being assessed as a potential treatment for ALS. In a spinal motor neuron model, ATH-1105 significantly protected against neuron death by glutamate-induced excitotoxicity, while reducing pathological aggregation of Transactive response ("TAR") DNA-binding protein 43 ("TDP-43"). Figure 7 summarizes the data with images of microtubule-associated protein-2 ("MAP-2")-labeled spinal motor neurons in green, and extranuclear TDP-43 in red. In the control image on the left with no excitotoxic insult (glutamate), there is minimal overlap of MAP-2 neurons with extranuclear TDP-43. When glutamate is applied to the motor neuron cultures, there is an overall reduction in the number of neurons and increased extranuclear TDP-43 as shown by overlapping red-green staining in the middle image. When cell cultures were treated with ATH-1105, the effect of glutamate-induced excitotoxicity on the overall number of motor neurons and extranuclear TDP-43 protein aggregation was significantly reduced, as seen in the rightmost image and as quantified in the bar graphs.

Figure 7. ATH-1105 is Neuroprotective Against Excitotoxic Insult and Reduces Extranuclear TDP-43 Levels in Spinal Motor Neurons.

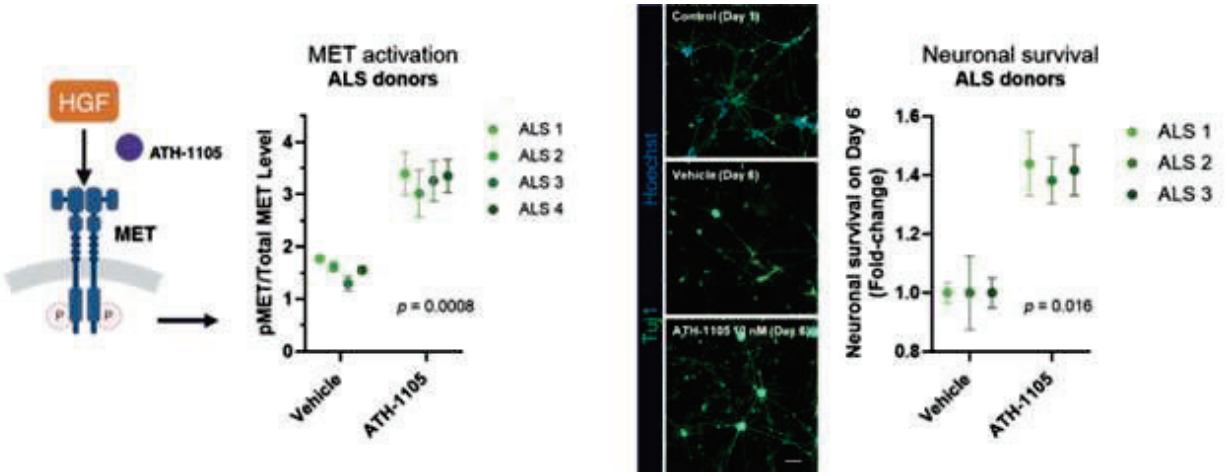


*Data presented as mean + SEM. Statistics applied: 1-way ANOVA with Fisher least significant difference test. *p<0.05; **p<0.01; ***p<0.001 versus Glutamate Control. Scale bar: 100 µm. n=5-6 per group.*

MAP-2, microtubule-associated protein-2; TDP-43, TAR DNA-binding protein 43.

ATH-1105 has also demonstrated target engagement in ALS patient-derived induced pluripotent stem cells (iPSCs) that were differentiated into motor neurons. In this model, as summarized in Figure 8, ALS patient-derived motor neurons treated with ATH-1105 10 nM exhibited a significant increase in MET activation (pMET) demonstrating engagement of HGF signaling, when compared to motor neurons treated with vehicle. Furthermore, treatment with ATH-1105 led to enhanced neuronal survival of ALS patient-derived motor neurons.

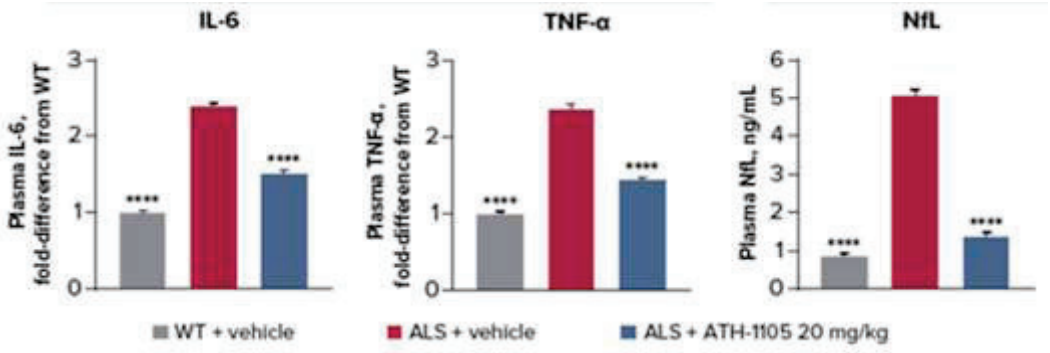
Figure 8. ATH-1105 enhances MET activation and survival in ALS patient-derived motor neurons.



Human iPSC-derived motor neurons derived from four people with sporadic ALS were sourced from Cedar Sinai via AnswerALS consortium. Motor neurons were cultured in growth factor deprived media and treated with vehicle or ATH-1105 every 48h for six days. MET activation: Data presented as mean ± SEM. Statistical significance was determined via paired *t*-test comparing MET activation (pMET/Total MET) in motor neurons treated with vehicle or ATH-1105 [10] nM for 24 hours; n = 2-3 technical replicates from each donor. Neuronal survival: Representative image highlighting effect of ATH-1105 on ALS patient-derived motor neurons (ALS 1) in culture. Neuronal survival (number of Tuji1+ neurons) was measured as surviving neurons on Day 6 divided by number of neurons on Day 1 (control expressed as 100%). Scale bar = 100 µm. Data expressed as fold-change between vehicle and ATH-1105 and presented as mean ± SEM. Statistical significance was determined via paired *t*-test comparing vehicle (containing HGF 0.05 ng/ml) with ATH-1105 [10] nM; n = 3-6 technical replicates from each donor. Select doses shown.

Plasma biomarkers of inflammation and neurodegeneration were significantly reduced following treatment with ATH-1105 in a transgenic TDP-43-driven mouse model of ALS (Figure 9). These results support the anti-inflammatory and neuroprotective effects of ATH-1105 through enhancement of neurotrophic HGF system signaling. In the TDP-43-driven ALS model, significant increases in the proinflammatory cytokines tumor necrosis factor alpha (“TNF-alpha”) and interleukin 6 (“IL-6”) were observed compared to healthy WT control animals. When ALS-model mice ("ALS mice") were treated with ATH-1105, significant reductions in both TNF-alpha and IL-6 were observed, demonstrating anti-inflammatory activity. Consistent with the neuroprotective effects demonstrated in cell-based models, a significant reduction in plasma levels of the neurodegeneration biomarker, NfL, was observed in ALS mice treated with ATH-1105, which is indicative of neuroprotection.

Figure 9. ATH-1105 Improves Biomarkers of Inflammation and Neurodegeneration in a Mouse Model of ALS.

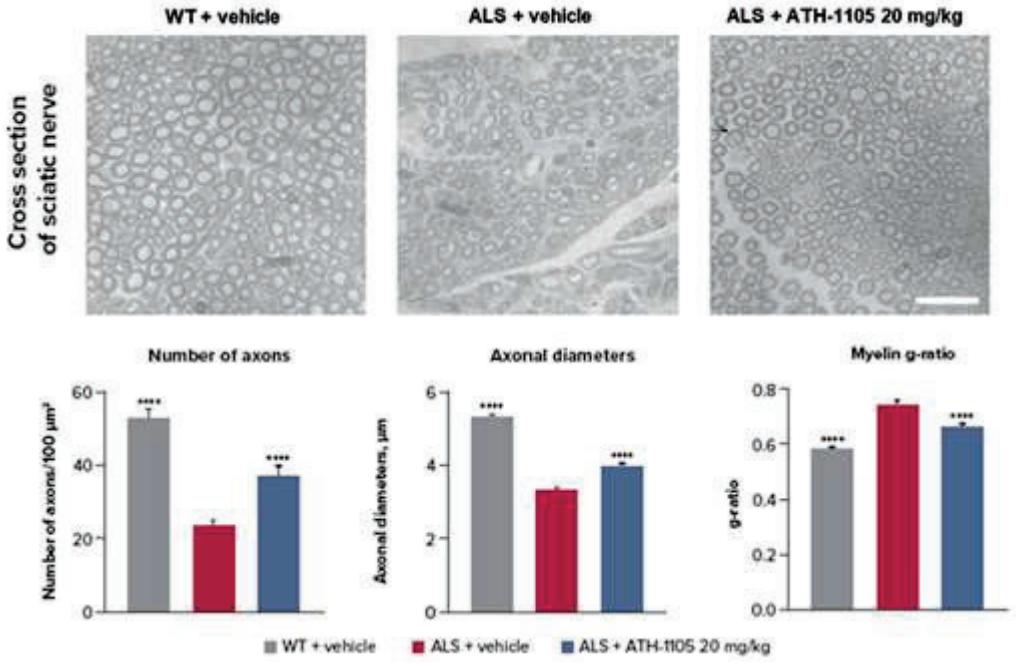


*Graphical representation of plasma IL-6, TNF-α in fold-difference over the WT + vehicle group, and NfL levels in ng/ml at two months of treatment. N=10 per group. Data presented as mean ± SEM. Statistical significance was determined by 1-way ANOVA with the Dunnett’s test versus ALS + vehicle. ****p<0.0001.*

ALS, amyotrophic lateral sclerosis; IL-6, interleukin 6; NfL, neurofilament light chain; TNF-α, tumor necrosis factor alpha; WT, wild-type.

Treatment with ATH-1105 in a mouse model of ALS protected against axon degeneration and demyelination as observed from histological examination of cross-sections of the sciatic nerve. Figure 10 below includes an image of the sciatic nerve from a WT healthy control animal, on the left, featuring a large number of large diameter axons surrounded by a consistent and highly regular coating of myelin sheath. In the middle, a sciatic nerve image from an ALS disease control animal is shown, where a marked reduction in the number of axons, a decrease in average axon diameter, and irregular myelination is observed. On the right, when ALS animals are treated with ATH-1105, sciatic nerve integrity is preserved with a greater population of large diameter axons and preservation of regular myelination. Graphs below the images are quantified data showing these effects of ATH-1105 in a mouse model of ALS.

Figure 10. Treatment with ATH-1105 Protected Against Axon Degeneration and Demyelination in a Mouse Model of ALS.



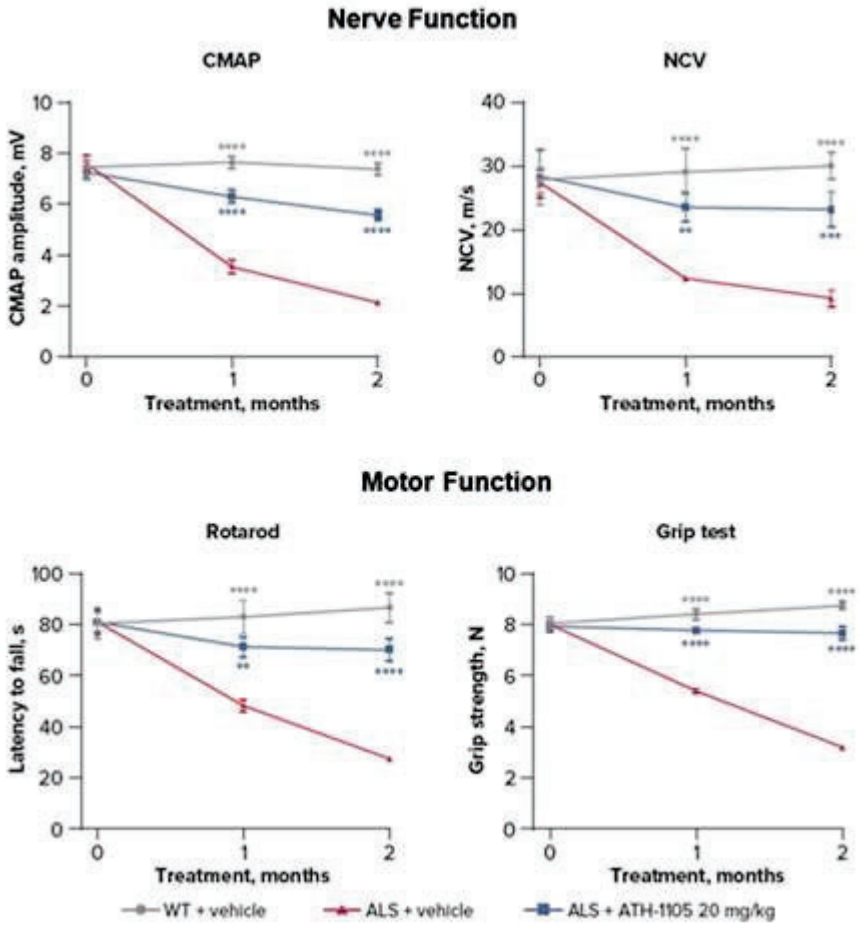
*Histology images of sciatic nerve cross-sections stained with toluidine blue to label myelin. Scale is 10 µm (all panels). Graphical representation of the number of axons (per 100 µm2), axonal diameter (in micrometers), and mean of myelin g-ratio, defined as the ratio of the inner axonal diameter to the total axonal diameter, following two months of treatment. Data presented as mean + SEM. Statistical significance was determined by 1-way ANOVA with the Dunnett's test versus ALS + vehicle. ****p<0.0001.*

ALS, amyotrophic lateral sclerosis; WT, wild-type.

Further analyses of electrophysiological and behavioral assessments indicated the protection of the motor neurons with ATH-1105 translated to improved nerve and motor function (Figure 11). Compound muscle action potential ("CMAP") and nerve conduction velocity ("NCV") are two electrophysiological measures of functional nerve signaling. Treatment with ATH-1105 in a mouse model of ALS demonstrated consistent and significant improvements of nerve function compared to ALS disease control animals.

Two examples of motor function improvements are shown by the rotarod, an assessment of balance and coordination, and the grip test, an assessment of strength. ALS disease control animals showed significant motor impairments compared to WT healthy control animals. ATH-1105 treatment in this mouse model of ALS led to significant improvements in both the rotarod and grip tests compared to the vehicle treated ALS disease control animals, demonstrating preservation of motor function. Other motor behavior tests assessing balance, coordination and muscle strength were the balance beam and Kondziela screen tests. Across all motor function measures, significant improvements were seen in ALS animals treated with ATH-1105 compared to ALS animals treated with vehicle.

Figure 11. Treatment with ATH-1105 Improves Nerve and Motor Function in a Mouse Model of ALS.



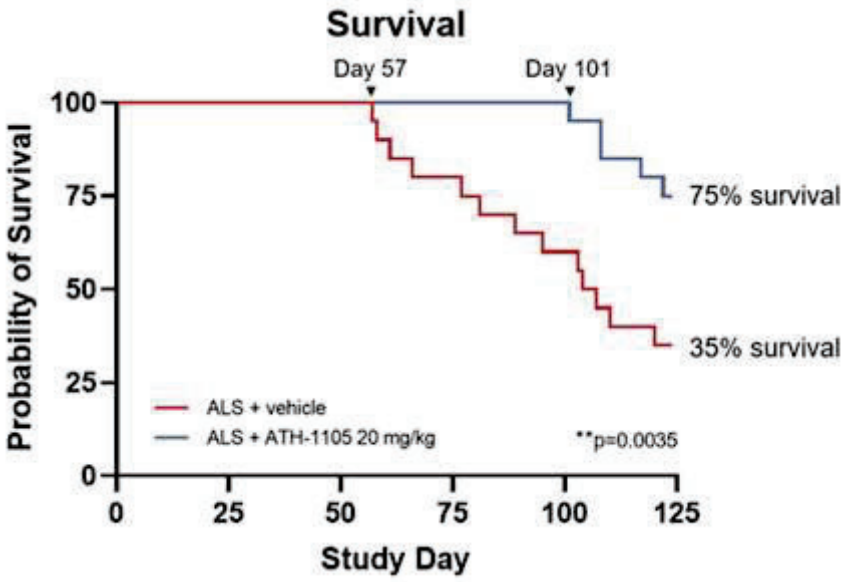
*Graphical representation of CMAP amplitude, NCV, rotarod latency, and grip strength at baseline and after one and two months of treatment. Data presented as mean ± SEM. Statistical significance was determined by 2-way ANOVA with the Dunnett's test versus ALS + vehicle. **p<0.01; ***p<0.001; ****p<0.0001. N=10 per group.*

ALS, amyotrophic lateral sclerosis; CMAP, compound muscle action potential; NCV, nerve conduction velocity; WT, wild-type.

The above study results, depicted in Figure 11, were presented at the American Academy of Neurology ("AAN") in April 2023, and the ALS Drug Development Summit in May 2023.

Treatment with ATH-1105 in a mouse model of ALS extends survival and improves other disease-related measures. Figure 12 below compares ALS mice treated with oral ATH-1105 20 mg/kg once daily in blue with oral vehicle once daily treated ALS mice in red. Mice were treated from one month of age to a humane endpoint maximum of five months of age, for a total of up to four months of treatment. ATH-1105 increased time to first mortality and significantly prolonged survival compared to ALS disease control animals (p=0.0035). ATH-1105 also significantly protected against body weight reduction (p<0.01). These findings were reported at the AAN 2023 Annual Meeting in April 2023.

Figure 12. ATH-1105 Significantly Improved Survival in a Mouse Model of ALS.



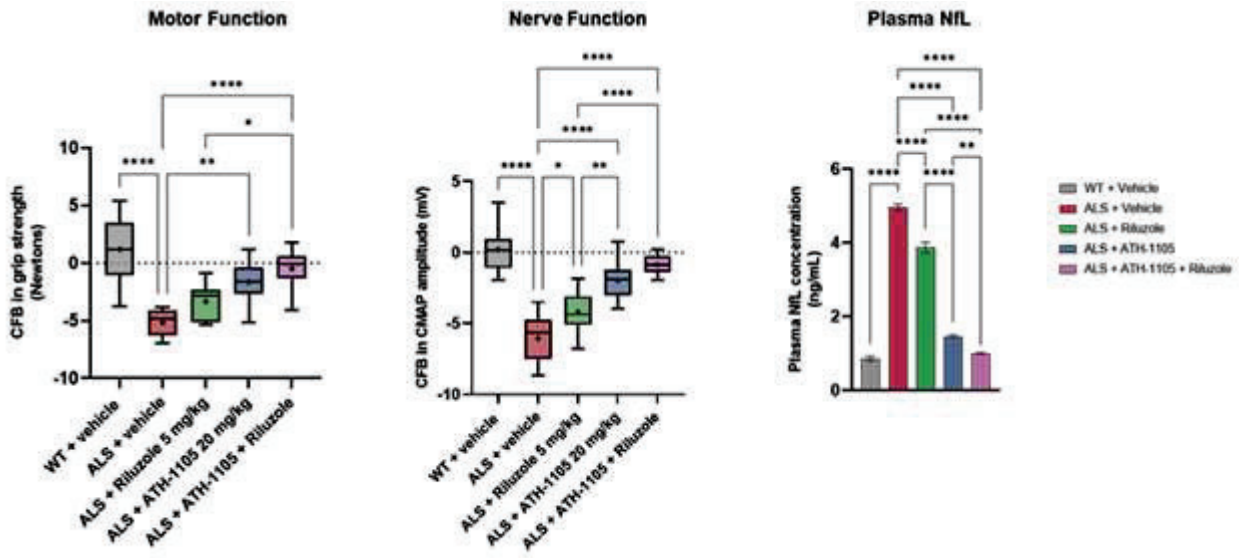
Data presented as Kaplan-Meier curve

*Statistics applied: Log-rank (Mantel-Cox) test for survival curve comparison, **p<0.01. n=20 mice per group at start*

Treatment with ATH-1105 in a mouse model of ALS outperforms treatment with riluzole under the conditions tested in assessments of motor function, nerve function, and in reducing disease-related plasma biomarkers. Figure 13 below compares performance in the grip test of WT mice (grey) and transgenic ALS mice treated daily with vehicle (red), intraperitoneal riluzole 5 mg/kg (green), oral ATH-1105 20 mg/kg (blue), or both ATH-1105 and riluzole (purple). Mice treated with ATH-1105 alone and co-administration of riluzole and ATH-1105 consistently outperformed the vehicle-treated ALS disease control group. In both the ATH-1105 and the co-administration of ATH-1105 and riluzole groups, performance on the test approached that of the WT healthy control group. ATH-1105 treatment outperformed riluzole treatment in tests of motor function including the grip, rotarod, Kondziela screen, and balance beam tests. ATH-1105 also outperformed riluzole in tests of nerve function preservation including CMAP amplitude and NCV. ATH-1105 treatment further reduced plasma levels of IL-6 and TNF-alpha compared to riluzole, which are biomarkers of inflammation. ATH-1105 treatment also more greatly reduced plasma levels of NfL compared to riluzole, which is a biomarker of neurodegeneration.

Furthermore, ATH-1105 significantly reduced levels of pTDP-43 in the sciatic nerve, whereas riluzole did not. These findings were reported at the Northeast ALS Consortium meeting in October 2023, and the Motor Neurone Disease Association conference in December 2023.

Figure 13. ATH-1105 Preserves Motor and Nerve Function and Reduces Plasma NfL Compared with Riluzole in a Mouse Model of ALS.



Graphical representation of change from baseline following two months of treatment in grip test, CMAP amplitude, and Plasma Nfl. Data presented as mean ± SEM, or box-and-whisker plots

*Statistics applied: One-way ANOVA with Dunnett's test. *p<0.05; **p<0.01; *** p<0.001; **** p<0.0001. n=10 mice per group; Abbreviations: CFB, change from baseline, CMAP, compound muscle action potential, NfL, neurofilament light chain*

Clinical Evidence for ATH-1105 in ALS

We conducted a first-in-human Phase 1 double-blind, placebo-controlled trial that enrolled 80 healthy volunteers to evaluate single and multiple oral ascending doses of ATH-1105. The trial was completed in November 2024 and evaluated the safety and tolerability of ATH-1105 and included measurements of pharmacokinetic outcomes. The results of the Phase 1 trial showed that ATH-1105 exhibited a favorable safety and tolerability profile, dose-proportional PK, and CNS penetration. In an exploratory target engagement study, analysis of neuron-derived extracellular vesicles from human plasma showed a treatment-associated increase in phospho-GSK3β(Ser9), supporting its potential use as a biomarker for future clinical development.

Clinical Development Plan for ATH-1105 in ALS

We are planning for a Phase 2, proof-of-concept trial designed to assess plasma NfL levels, safety, pharmacokinetics, and exploratory outcomes for a once daily single-dose treatment of ATH-1105 in people living with ALS. NfL is a well-established biomarker of axonal injury and is elevated in people living with ALS, reflecting rate of neurodegeneration. In our proposed Phase 2 study, demonstrating a reduction or stabilization of NfL would deliver proof-of-concept for neuroprotection, indicating that ATH-1105 may slow the biological drivers of ALS progression. Exploratory outcome measures may include ALSFRS-R, Slow Vital Capacity, and other ALS-related biomarkers. The trial population is expected to include males and females between ≥18 and 80 years of age, inclusive with sporadic or familial ALS who are diagnosed with definite ALS, probable ALS, probable ALS laboratory results supported or possible ALS according to the El Escorial revised criteria for the diagnosis of ALS. This trial is anticipated to provide clinical evidence to support the design and conduct of a larger pivotal clinical trial. The Phase 2 trial in people living with ALS has an anticipated start in 2026.

Market Opportunity

The potential target indications for ATH-1105, our small molecule positive modulator, include ALS and other neurodegenerative diseases. ALS is a progressive and fatal neurodegenerative disease with a typical life expectancy of only 2-5 years after diagnosis. Given its severity, persistent unmet medical need, and lack of any effective therapies, any treatment that demonstrates meaningful clinical benefit has the potential for rapid adoption. A recent study estimated that approximately 33,000 persons in the United States are affected with ALS. This number was projected to increase to approximately 36,000 by 2030. Prevalence at the global level varies geographically. Cases worldwide have been projected to increase from approximately 223,000 in 2015 to approximately 377,000 in 2040.

Currently, there are only four approved drugs that are specifically indicated for the treatment of ALS, of which none targets neurotrophic factor systems with a multimodal mechanism of action with the potential to offer neuroprotective, anti-inflammatory and potentially disease modifying effects.

Potential Commercialization Plan

ATH-1105 is currently in development for the potential treatment of ALS. The potential commercialization strategy is expected to consider the following key elements:

1. potential first-line therapy;
2. an add-on therapy for patients on existing therapies; and
3. a therapy for patients who have stopped existing therapies.

We aim to demonstrate the therapeutic potential of ATH-1105 in ALS as an initial indication. Given the unique potential of ATH-1105 to provide broad protection against neurodegeneration, there may be therapeutic application in additional neurodegenerative indications including but not limited to frontal temporal dementia, Parkinson’s disease, Alzheimer’s disease, primary lateral sclerosis, progressive muscle atrophy, polyneuropathies, and others. We expect our commercialization strategies, including distribution plans, will evolve as clinical development progresses.

We are actively reviewing options for partnerships or other arrangements that will allow us to realize the commercial potential of our drug candidates.

ATH-1020

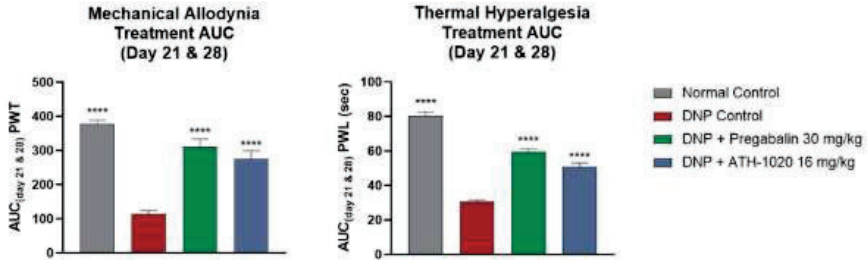
ATH-1020 Preclinical Evidence for Neuropathic Pain

ATH-1020 is a novel, orally available, next generation, small molecule drug candidate designed to positively modulate the neurotrophic HGF system. Enhancing HGF/MET signaling promotes neuroprotective, neurotrophic, and anti-inflammatory effects, and as neuropathic pain disorders, including diabetic neuropathy ("DNP"), have components of oxidative stress, nerve damage, and inflammation, positive modulation of the HGF/MET pathway may provide therapeutic benefit in these disease areas. Data below were presented at the Society for Neuroscience Annual Conference in November 2022.

This compound was originally assessed for neuropsychiatric indications in preclinical models as presented at the American Society for Experimental Therapeutics Annual Conference in February 2022.

In animal models of DNP hypersensitivity to mechanical and thermal pain are commonly experienced, which are also representative of symptoms in people with neuropathic pain. Shown in Figure 11 below, treatment with ATH-1020 significantly reduced pain behaviors over the testing period compared to DNP controls alone.

Figure 11. Reduced Mechanical and Thermal Pain-Related Behaviors Following ATH-1020 Treatment in a Rat Model of Diabetic Neuropathic Pain.

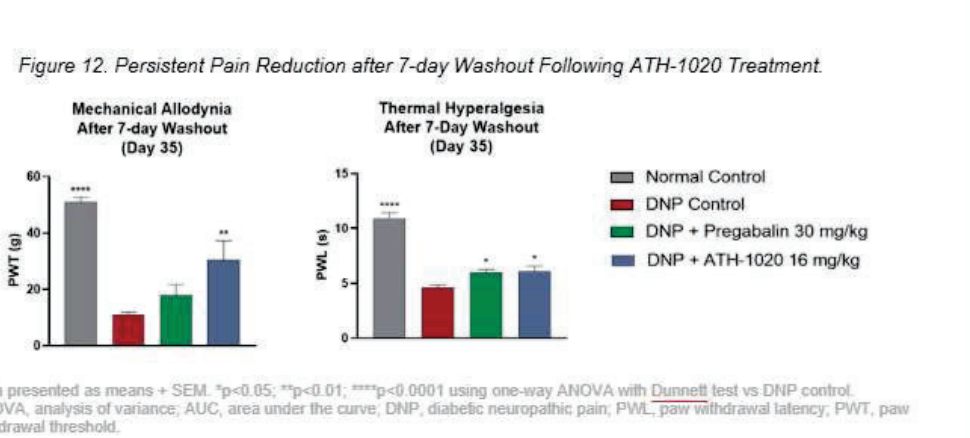


Data presented as means + SEM. ****p<0.0001 using one-way ANOVA with Dunnett test vs DNP control.
ANOVA, analysis of variance; AUC, area under the curve; DNP, diabetic neuropathic pain; PWL, paw withdrawal latency; PWT, paw withdrawal threshold.

Data presented as means + SEM. ****p<0.0001 using one-way ANOVA with Dunnett test vs DNP control.

ANOVA, analysis of variance; AUC, area under the curve; DNP, diabetic neuropathic pain; PWL, paw withdrawal latency; PWT, paw withdrawal threshold. Persistence of reduced pain behaviors following ATH-1020 treatment were assessed after a short-term (23-hour) or a long-term (7-day) washout period. Study results demonstrated that even after short- and long-term washout periods, where no drug is present, the effects of ATH-1020 reduction of pain behaviors remained persistent, suggesting a potential disease modifying effect (Figure 12).

Figure 12. Persistent Pain Reduction after 7-day Washout Following ATH-1020 Treatment.



Data presented as means + SEM. *p<0.05; **p<0.01; ****p<0.0001 using one-way ANOVA with Dunnett test vs DNP control.

ANOVA, analysis of variance; AUC, area under the curve; DNP, diabetic neuropathic pain; PWL, paw withdrawal latency; PWT, paw withdrawal threshold

ATH-1020 Clinical – Phase 1 Trial in Healthy Volunteers

We filed an IND application with the FDA for ATH-1020 at the end of 2021 and received notice of acceptance in January 2022. In September 2022, we completed the single-ascending dose escalation portion of the Phase 1 trial. ATH-1020 demonstrated a favorable safety profile and was well-tolerated in healthy volunteers.

Sermonix Transaction

On December 18, 2025, we entered into agreements with Sermonix and Ligand granting us exclusive licenses and rights to develop, manufacture and commercialize oral forms of the SERM known as lasofoxifene in all countries and territories of the world except for Asia and certain countries in the Middle East. Under the terms of our license agreement with Sermonix (the "Sermonix License"), we are assuming responsibility, in such countries and territories, for the ongoing ELAINE-3 trial and we are coordinating with Sermonix and Shanghai Henlius Biotech, Inc. ("Henlius"), Sermonix's exclusive licensee for oral lasofoxifene in Asia and certain countries in the Middle East (the "Retained Territory"), with respect to Henlius' conduct of the ELAINE-3 trial in the Retained Territory.

Sermonix License

Licenses and Exclusivity. Under the Sermonix License, we received from Sermonix an exclusive (even as to Sermonix), sublicensable license, under relevant patents and know-how owned or in-licensed by Sermonix, to develop, manufacture, commercialize and otherwise exploit products containing lasofoxifene (the "Licensed Products") in all countries outside the Retained Territory (the "Licensed Territory"). Such license does not include a sublicense under the patents and know-how that Sermonix previously in-licensed from Ligand because we entered into a direct license agreement with Ligand (the "Ligand Agreement") to replace Sermonix's license from Ligand with respect to Licensed Products in the Licensed Territory. During the term of the Sermonix License, Sermonix and its affiliates are prohibited

from developing, manufacturing, commercializing or otherwise exploiting products that selectively modulate the estrogen receptor, although Sermonix retains the ability to fulfill its obligations under its license agreement with Henlius (the "Sermonix-Henlius Agreement") with respect to the Licensed Products in the Retained Territory.

Development, Manufacture and Commercialization Activities. Under the Sermonix License, we have the sole right to conduct all development (including regulatory activities), manufacturing and commercialization of Licensed Products in the Licensed Territory, at our sole cost and expense. We are obligated to use commercially reasonable efforts to develop and obtain and maintain regulatory approval for at least one Licensed Product in the Licensed Territory and, following receipt of regulatory approval, to use commercially reasonable efforts to commercialize the applicable Licensed Product in the country of approval. We have assumed certain of Sermonix's agreements with third parties that are conducting or supporting the ELAINE-3 trial in the Licensed Territory and certain agreements with third-party contract manufacturers that have been supplying drug substance and drug product for the ELAINE-3 trial. All of Sermonix's existing inventory of Licensed Product drug substance and drug product was transferred to us at no additional cost and we will provide a portion of such inventory to Henlius for use in the ELAINE-3 trial in the Retained Territory.

Financial Terms. As consideration for the rights granted to us under the Sermonix License, we issued Sermonix a pre-funded warrant to purchase 5,502,402 shares of our common stock pursuant to a securities purchase agreement (the "Sermonix Securities Purchase Agreement"), as further described below, will make payments to certain of Sermonix's third-party service providers that total approximately $16.8 million, and will make payments to Sermonix of $75,000 per month, subject to adjustment from time to time upon mutual agreement of the parties. We may credit the amount of such monthly payments against all milestone, royalty, and net proceed payments that become due to Sermonix under the Sermonix License, and our obligation to make such monthly payments will end upon Sermonix's receipt of the first such milestone, royalty or net proceeds payment. If we or our affiliates achieve certain commercialization or annual net sales milestones with respect to the Licensed Products, we will make milestone payments to Sermonix up to a maximum aggregate total of $100.0 million, a portion of which may be payable in cash or shares of our common stock at our discretion, with final terms to be negotiated between the parties. In addition, we are obligated to make royalty payments to Sermonix based on our and our affiliates' annual net sales of the Licensed Products in the Licensed Territory, with the applicable royalty rates ranging from sub-single digit to low-single digit percentages, subject to customary reductions. Such royalty obligation will expire on a Licensed Product-by-Licensed Product and country-by-country basis on the latest of (i) the expiration of the last valid claim of a patent licensed to us by Sermonix that covers such Licensed Product in such country, (ii) the expiration of regulatory exclusivity of such Licensed Product in such country; and (iii) 10 years after the first commercial sale of such Licensed Product in such country. If we sublicense or divest our rights to the Licensed Products to a third party, then in lieu of the milestone and royalty payments described above, we will pay to Sermonix a percentage of the net proceeds from such sublicensing or divestiture transaction. The applicable percentage is based on the type of payment received by us from the third party as well as status of development of the Licensed Products at the time that the sublicense or divestiture transaction is entered into, with rates starting in the high-double digit and decreasing to low-double digit percentages. As consideration for our payment and other obligations under the Sermonix License, we will be eligible to receive from Henlius all milestone and royalty payments that Henlius owes to Sermonix under the Sermonix-Henlius Agreement other than royalties that Sermonix owes to Ligand on account of Sermonix's license agreement with Ligand, and with the further exception that, if Henlius and Sermonix later agree to share the costs of

developing the Licensed Products in Japan, then we will not be entitled to receive Sermonix's share of income arising from the sublicensing of Japanese rights to the Licensed Product.

Term and Termination. The Sermonix License will remain in effect until we, our affiliates and our sublicensees are no longer developing or commercializing any Licensed Product in the Licensed Territory. Each party may terminate the Sermonix License in its entirety for the uncured material breach by or insolvency of the other party. We may terminate the Sermonix License in its entirety for safety reasons or for convenience at any time after the topline data readout of the ELAINE-3 trial. Upon termination, all rights and licenses granted to Company will revert to Sermonix.

The Sermonix License includes certain other customary terms and conditions, including mutual representations and warranties, indemnification, and confidentiality provisions.

Ligand Agreement

Licenses and Exclusivity. Under the Ligand Agreement, we received from Ligand an exclusive (even as to Ligand), sublicensable license, under relevant know-how and certain patents owned or in-licensed by Ligand, to develop, manufacture and sell Licensed Products for oral use in the Licensed Territory. Ligand does not have any restrictions on its ability to develop, manufacture or sell products (other than the Licensed Products) that selectively modulate the estrogen receptor. We granted to Ligand a non-exclusive, sublicensable, worldwide license, under our improvements to Ligand's licensed technology, to develop, manufacture and sell products containing lasofoxifene for topical and other non-oral uses.

Development, Manufacture and Commercialization Activities. Under the Ligand Agreement, we have the sole right to conduct all development (including regulatory activities), manufacturing and commercialization of Licensed Products for oral use in the Licensed Territory, at our sole cost and expense. We are obligated to diligently develop, manufacture and sell oral Licensed Products in the Licensed Territory, to use commercially reasonable efforts to develop oral Licensed Products for treatment of metastatic breast cancer and develop markets for oral Licensed Products in the Licensed Territory, and to obtain all necessary regulatory approvals for our manufacture, use, importation and sale of oral Licensed Products in the Licensed Territory.

Financial Terms. The financial terms of the Ligand Agreement are the same, with respect to the Licensed Territory, as the financial terms of Sermonix's license agreement with Ligand that was in place immediately prior to the Sermonix transaction (the "Prior Sermonix-Ligand Agreement") As a result, the following payments, which are owed by us to Ligand, are identical to the amounts that Sermonix would have owed to Ligand if the Prior Sermonix-Ligand Agreement had not been replaced, with respect to the Licensed Territory, by the Ligand Agreement. If we or our affiliate or sublicensee achieves certain regulatory approval and commercialization milestones with respect to any oral Licensed Product in the Licensed Territory, we will make milestone payments to Ligand up to a maximum aggregate total of $4.25 million in regulatory milestones and $10.5 million in commercialization milestones per Licensed Product. In addition, we are obligated to make royalty payments to Ligand based on our and our affiliates' and sublicensees' annual net sales of oral Licensed Products in the Licensed Territory, with the applicable royalty rates ranging from mid-single digit to low-double digit percentages, subject to reductions for generic product sales and amounts paid to third parties for certain intellectual property licenses. Such royalty obligation will expire on a Licensed Product-by-Licensed Product and country-by-country basis on the latest of (i) the expiration of the last valid claim of a patent licensed to us by Ligand in such country, (ii) the expiration of regulatory exclusivity of such Licensed Product in such country, and

(iii) 15 years after the first commercial sale of such Licensed Product in such country. If we sublicense our rights to the oral Licensed Products to a third party, then in addition to the milestone and royalty payments described above, we will pay to Ligand a mid-teen percentage of certain amounts received by us from such third-party sublicensee.

Term and Termination. The Ligand Agreement will remain in effect so long as we, our affiliates and our sublicensees are developing, manufacturing, using or commercializing any Licensed Product in the Licensed Territory. Each party may terminate the Ligand Agreement in its entirety for the uncured material breach by or insolvency of the other party. We may terminate the Ligand Agreement in its entirety or on a product-by-product or country-by-country basis, prior to regulatory approval, for safety reasons or failure to achieve the primary efficacy endpoint in any clinical trial of a Licensed Product for oral use. Ligand may terminate the Ligand Agreement if we or our affiliate or sublicensee challenges the validity or enforceability of any patent licensed to us by Ligand. Upon termination, all rights and licenses granted to Company will revert to Ligand and we are obligated to transfer to Ligand our data, regulatory approvals, domain names and trademarks for the oral Licensed Products in the Licensed Territory.

The Ligand Agreement includes certain other customary terms and conditions, including mutual representations and warranties, indemnification, and confidentiality provisions.

Sermonix Securities Issuance

In connection with entry into the Sermonix License, on December 18, 2025 we entered into the Sermonix Securities Purchase Agreement, pursuant to which Sermonix was issued a pre-funded warrant (the “Sermonix Pre-Funded Warrant” or the “Sermonix Securities”) to purchase 5,502,402 shares of our common stock (the “Sermonix Pre-Funded Warrant Shares”). The Sermonix Securities were issued as partial consideration for Sermonix’s entry into the Sermonix License. The closing of the purchase and sale of the Sermonix Securities (the “Sermonix Closing”) occurred on December 23, 2025.

At the Sermonix Closing, we entered into a registration rights agreement with Sermonix pursuant to which we are required to prepare and file a registration statement with the Securities and Exchange Commission (the "SEC") to register the resale of the Sermonix Pre-Funded Warrant Shares within 30 calendar days after the date of the Sermonix Closing, and to use reasonable best efforts to have the registration statement declared effective at the earliest possible date but no later than the earlier of (a) 75 calendar days after the filing date if the SEC notifies us that it will review the registration statement and (b) the fifth business day after we are notified by the SEC that the registration statement will not be reviewed or will not be subject to further review.

The Sermonix Pre-Funded Warrant has an exercise price of $0.001 per share, subject to customary adjustments under the terms thereof. The Sermonix Pre-Funded Warrant was not exercisable until the Sermonix Stockholder Approval (as defined below) was obtained. Following receipt of the Sermonix Stockholder Approval, the Sermonix Pre-Funded Warrant became exercisable at any time, except that the Sermonix Pre-Funded Warrant cannot be exercised if, immediately prior to or following such exercise, Sermonix, together with its affiliates and any other persons whose beneficial ownership of shares of our common stock would be aggregated with Sermonix for purposes of Section 13(d) of the Exchange Act, would beneficially own more than 4.99% (the “Sermonix Maximum Percentage”) of the total number of issued and outstanding shares of our common stock following such exercise. The Sermonix Maximum Percentage may be increased or decreased by Sermonix with 61 days’ written notice to us; provided,

however, that such percentage may in no event exceed 19.99% prior to receipt of the Sermonix Stockholder Approval.

In March 2026, we held a special meeting of stockholders seeking approval of various matters, including that the Sermonix Pre-Funded Warrant can be exercised at any time without restriction or additional stockholder approval (the “Sermonix Stockholder Approval”). At the March 2026 special meeting of stockholders, the Sermonix Stockholder Approval was obtained and the Sermonix Redemption Right (described in Note 3 to our consolidated financial statements included elsewhere in this report) terminated.

Manufacturing

Our focus on small molecule therapeutics enables us to use well-established and widely available manufacturing processes and infrastructure, formulation compositions, and drug administration technologies or devices. We do not operate our own facilities for manufacturing, storing, or distributing drug candidates. We utilize third-party contract development and manufacturing organizations ("CDMOs") to manufacture and supply preclinical and clinical materials during the development of drug candidates. We and various regulatory bodies have audited the CDMOs we contract with, and such CDMOs have a proven track record of GMP-compliant manufacturing.

Lasofoxifene is purified as a stable solid and then released to other CDMOs for formulation and packaging into the final tablet drug product for use in clinical testing and commercial supply. The drug chemistry is well established, the highly potent API has been well characterized, and the impurity profile is well controlled. We believe the synthesis of lasofoxifene tablets is reliable and reproducible and the synthetic routes can be further optimized to enable large-scale production, including the manufacturing of API in a high-potent suite. Current manufactured drug product follows the original Pfizer formulation. Clinical supply manufacturing is on track to meet the projected drug supply demand of the ongoing ELAINE-3 trial. Ongoing process optimization is being conducted to establish larger clinical and commercial supplies that meet quality and manufacturing standards for the potential commercial manufacturing of lasofoxifene, with an anticipated 4-year shelf life.

ATH-1105 is purified as a stable solid and then released to other CDMOs for formulation and packaging into the final drug product for use in clinical testing. We believe the synthesis of ATH-1105 is reliable and reproducible and the synthetic routes can be further optimized to enable large-scale production that continues to avoid use of toxic materials or specialized equipment or handling during the manufacturing process.

For the potential commercialization of any drug candidates that receive regulatory approval, we expect to rely on partners’ manufacturing capabilities or use similar third-party CDMO contract resources.

Competition

The biotechnology and biopharmaceuticals industries are characterized by rapid technological advancement, significant competition, and an emphasis on intellectual property. The mBC market is active, especially in the 2L+ treatment landscape. As a clinical-stage biopharmaceutical company developing novel therapies for high unmet medical needs, with its principal drug candidate focused on the treatment of metastatic breast cancer, we face, and in the future may face, increased competitive pressures from both large and small pharmaceutical companies and from established and emerging biotechnology companies, as well as academic, government, and public and private research institutions. Any drug candidates that we successfully develop and commercialize will compete with current treatments and new treatments that may become available in the future.

While lasofoxifene is a novel SERM, several pharmaceutical and biotechnology companies are developing or marketing 1L+ therapies for patients with ESR1 mBC. These include, but are not limited to, The Menarini Group (Orsedu ®), Eli Lilly (Inluriyo ®), AstraZeneca, Arvinas, Olema, and F. Hoffman- La Roche AG. Although SERDs and SERMs may have utility in the 1L setting, we believe that demonstrating a substantial and clinically meaningful improvement from the current standard of care remains challenging. Existing AI and CDK4/6i combinations have demonstrated efficacy, safety, tolerability, and cost effectiveness that are difficult to displace. SERDs have been found to be most effective in patients with low ESR1 mutation rates. Given common clinical practice to sequence therapies with different mechanisms of action upon disease progression, a SERM such as lasofoxifene may be positioned for use following prior exposure to SERDs, AIs, CDK4/6i, or other therapies utilized in earlier stages of the disease.

In addition, with the advancement of ATH-1105 as a novel small molecule therapeutic that positively modulates the neurotrophic HGF system, we must consider companies as competitors who are developing other novel approaches, including those that target other neurotrophic systems to address ALS and other neurological diseases. Additionally, because ATH-1105 is orally administered, we must also consider as competitors companies developing ALS therapies as oral therapeutics or with other routes of administration.

Because of the range of potential competitors, many of our competitors, alone or with strategic partners, have greater access to financial, technical, and human resources, market presence, and deeper experience in drug discovery and development, preclinical and clinical testing, manufacturing, commercialization, the regulatory approval process in the U.S. and abroad, or marketing and sales than we do. They also have extensive expertise in commercializing approved therapies and leveraging established market channels. In addition, these same competitors, who may be in a clinical development stage, could also be competing with us for patient recruitment, clinical research organization, and operational resources. These entities also compete with us in the recruitment and retaining of qualified scientific and management personnel, as well as the acquisition of enabling or complementary technologies for advancing our product candidates across all competitors. Merger and acquisition activity in the biotechnology and biopharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. As a result, competitors may achieve regulatory approval more rapidly, secure broad market penetration, and gain stronger physician and payer adoption. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. Our commercial opportunity could be substantially limited if our competitors develop and commercialize products that are more effective, safer, more convenient or less expensive than our comparable drug products. In geographies that are critical to our commercial success, competitors may also obtain regulatory approvals first, resulting in these competitors building a strong market position in advance of the entry of our drug candidates. In addition, our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of other treatments.

We believe that the key competitive factors affecting the success of lasofoxifene will include efficacy, quality of life improvement, safety profile, convenience, cost, and intellectual property protection compared to existing treatments. Compared to its competitors, we believe lasofoxifene has the potential to deliver durable efficacy (13 months PFS in CDK4/6i combo setting), superior tolerability (grade 1/2 AEs such as diarrhea), and combination potential (minimal drug-drug interactions). In addition, we believe that lasofoxifene is the only targeted novel endocrine treatment with potential quality of life benefits (improved urogenital symptoms and potential bone density and lipid health benefits) in precision oncology medicine. Thus, we believe that these advantages may position lasofoxifene as a potential standard in endocrine

therapy. Newly approved treatments for ESR1+ mBC will have to compete with existing therapies as well as therapies currently in development and that may be developed in the future.

Specific to targeting HGF/MET, however, we are not aware of any direct competitors currently developing small molecules targeting the neurotrophic HGF system for ALS. We are aware of two companies developing HGF/MET-directed therapies for ALS, including VM202, a plasmid DNA encoding human HGF developed by Helixmith, and KP-100, a recombinant HGF protein developed by Kringle Pharma. Both assets have been investigated in Phase 2 clinical trials for ALS, where they were shown to be safe and well-tolerated but failed to reach statistical significance on efficacy endpoints.

ATH-1105 has the potential to offer neuroprotective and anti-inflammatory effects by targeting the HGF/MET system. Our preclinical data have demonstrated this multimodal approach may have positive benefits across several biological and clinical measures. While several potential direct competitors may exist, we have not yet identified any competitive asset that has demonstrated consistent and congruent positive effects offering neuroprotection, anti-inflammation, reduction in disease-specific protein pathologies, and neurotrophic and functional benefits.

Intellectual Property

We own or have in-licensed numerous patents and patent applications and possesses substantial know-how and trade secrets relating to the development and commercialization of our drug candidates, including related manufacturing processes and technologies, as well as their use in the treatment of various diseases. For drug candidates, we generally pursue multilayered patent protection covering compositions of matter, methods of use, and methods of manufacture. We intend to strengthen the patent protection of drug candidates and technologies through additional patent application filings. Patent terms for our owned or in-licensed patents discussed herein may exclude any patent term extension or patent term adjustment that may be awarded. Further, data and/or market exclusivity based on new chemical entity or other categories may be available in various jurisdictions.

We have in-licensed a portfolio of patents and patent applications from Sermonix and Ligand, centered around lasofoxifene and its method of use in several settings including in (1) combination with abemaciclib for the treatment of ER+/HER2-, locally advanced or metastatic breast cancer with an ESR1 mutation after progression on an AI in combination with the CDK4/6 inhibitor palbociclib or ribociclib, (2) ER+ breast cancer with no ESR1 mutations after progression on an AI, (3) ER+ breast cancer in neoadjuvant setting, and (4) ER+ breast cancer in adjuvant setting. Our in-licensed patent portfolio includes issued patents and pending patent applications in the United States, issued patents and pending patent applications in jurisdictions outside of the United States, and pending international patent applications filed under the Patent Cooperation Treaty. Our in-licensed issued patents expire on dates ranging from 2037 to 2042, absent any patent term adjustment and/or extension. Our in-licensed pending patent applications, if issued, or pending international patent applications, if nationalized and issued, would be expected to expire on dates ranging from 2037 to 2045.

We own patents and patent applications directed to ATH-1105 and our other small molecule drug candidates, including ATH-1020, directed to compositions of matter and methods of use thereof. Our owned patent portfolio includes pending patent applications in the United States, issued patents and pending patent applications in jurisdictions outside of the United States, and pending international patent applications filed under the Patent Cooperation Treaty. Our owned and issued patents directed to ATH-1105 and our other small molecule drug candidates, including ATH-1020, directed to compositions of matter and methods of use thereof, will expire on dates ranging from 2041 to 2042. If patents are issued

on our owned pending patent applications directed to ATH-1105 and our other small molecule drug candidates, including ATH-1020, directed to compositions of matter and methods of use thereof, the resulting patents are projected to expire on dates ranging from 2041 to 2045, absent any patent term adjustment and/or extension.

Individual patents are in force for varying periods of time, depending upon the date of filing of the patent application, the date of patent issuance, and the legal term of patents in the countries in which they are obtained. Generally, patents issued for applications filed in the United States are in force for 20 years from the earliest nonprovisional filing date. In addition, in certain instances, a patent term can be adjusted or extended to recapture a portion of the term effectively lost as a result of the United States Patent and Trademark Office ("USPTO") delay or the FDA regulatory review period (a patent term adjustment or patent term extension, respectively). The restoration period for FDA delay cannot be longer than five years and the total patent term, including the restoration period, must not exceed 14 years following FDA approval. The duration of patents outside of the United States varies in accordance with provisions of applicable local law, but typically is also 20 years from the earliest nonprovisional filing date. However, the actual protection afforded by a patent varies on a product-by-product basis, from country-to-country, and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory-related extensions, the availability of legal remedies in a particular country, and the validity and enforceability of the patent.

When appropriate, we seek to protect aspects of our technology and business not amenable to, or that we do not consider appropriate for, patent protection as trade secrets. we seek to protect this intellectual property, in part, as trade secrets, by entering into confidentiality agreements with those who have access to our confidential information, including employees, contractors, consultants, collaborators, and advisors. We also seek to preserve the integrity and confidentiality of our proprietary technology and processes by maintaining physical security of our premises and physical and electronic security of our information technology systems.

We seek trademark protection in the United States and in certain other jurisdictions where available and when we deem appropriate. Our trademark portfolio includes pending trademark applications for LeonaBio and related trademarks in the United States and internationally.

Government Regulation

Government authorities in the United States, at the federal, state, and local level, and other countries extensively regulate, among other things, the research, development, nonclinical and clinical testing, manufacture, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing, and export and import of products such as those we are developing. Generally, before a new drug can be marketed, considerable data must be generated, which demonstrate the drug's quality, safety, and efficacy. Such data must then be organized into a format specific for each regulatory authority, submitted for review and approved by the regulatory authority.

U.S. Drug Development Process

In the United States, the FDA regulates drugs under the federal Food, Drug, and Cosmetic Act ("FDCA") and its implementing regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S.

requirements at any time during the product development process, the approval process or after approval may subject an applicant to administrative or judicial sanctions. These sanctions could include the FDA's refusal to approve pending applications, withdrawal of an approval, a clinical hold, warning 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 penalties. Any agency or judicial enforcement action could have a material adverse effect on us.

The process required by the FDA before a drug may be marketed in the United States generally involves the following:

- completion of nonclinical laboratory tests, animal studies, and formulation studies in accordance with FDA's good laboratory practice, requirements and other applicable regulations;
- submission to the FDA of an IND, which must become effective before human clinical trials may begin;
- approval by an independent institutional review board ("IRB") ethics committee, either centralized or with respect to each clinical site, before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with good clinical practice ("GCP") requirements to establish the safety and efficacy of the proposed drug for its intended use;
- submission to the FDA of a New Drug Application ("NDA") after completion of all pivotal trials;
- determination by the FDA within 60 days of its receipt of an NDA to accept the filing for substantive review;
- satisfactory completion of an FDA advisory committee review, if applicable;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the drug is produced to assess compliance with current good manufacturing practice ("cGMP") requirements to ensure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality, and purity, and of selected clinical investigation sites to assess compliance with GCPs; and
- FDA review and approval of the NDA to permit commercial marketing of the product for particular indications for use in the United States.

Prior to beginning the first clinical trial with a product candidate in the United States, we must submit an IND to the FDA. An IND is a request for authorization from the FDA to administer an investigational new drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol(s) for clinical studies. The IND also includes results of animal and *in vitro* studies assessing the toxicology, pharmacology, and PK/PD characteristics of the product; chemistry, manufacturing, and controls information; and any available human data or literature to support the use of the investigational product. An IND must become effective before human clinical trials may begin. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, raises safety concerns or questions about the proposed clinical trial. In such a case, the IND may be placed on clinical hold and the IND sponsor and the FDA must resolve any outstanding concerns or questions before the clinical trial can begin. Submission of an IND therefore may or may not result in FDA authorization to begin a clinical trial.

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators in accordance with GCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical study. Clinical trials are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A separate submission to the existing IND must be made for each successive clinical trial conducted during product development and for any subsequent protocol amendments. Furthermore, an independent IRB for each site proposing to conduct the clinical trial must review and approve the plan for any clinical trial and its informed consent form before the clinical trial begins at that site and must monitor the study until completed. 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 clinical trial is unlikely to meet its stated objectives. Some studies also include oversight by an independent group of qualified experts organized by the clinical study sponsor, known as a data safety monitoring board, which may review data and endpoints at designated check points, make recommendations or halt the clinical trial if it determines that there is an unacceptable safety risk for subjects or other grounds, such as no demonstration of efficacy. There are also requirements governing the reporting of ongoing clinical studies and clinical study results to public registries.

Human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

- *Phase 1*: The product candidate is initially introduced into healthy human subjects or patients with the target disease or condition. These studies are designed to test the safety, dosage tolerance, absorption, metabolism, and distribution of the investigational product in humans, the side effects associated with increasing doses, and, if possible, to gain early evidence on effectiveness. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.
- *Phase 2*: The product candidate is administered to a limited patient population with a specified disease or condition to evaluate the preliminary efficacy, optimal dosages, and dosing schedule and to identify possible adverse side effects and safety risks. Multiple Phase 2 clinical trials may be conducted to obtain information prior to beginning larger and more expensive Phase 3 clinical trials.
- *Phase 3*: The product candidate is administered to an expanded patient population to further evaluate dosage, to provide statistically significant evidence of clinical efficacy and to further test for safety, generally at multiple geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the investigational product and to provide an adequate basis for product approval.

Post-approval clinical trials, sometimes referred to as Phase 4 studies, may be conducted after initial marketing approval. These clinical trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval of an NDA.

The FDA or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients. In addition, some clinical trials are overseen by an independent group of qualified experts organized by the sponsor, known as a data safety monitoring board or committee.

Depending on its charter, this group may determine whether a clinical trial may move forward at designated check points based on access to certain data from the clinical trial.

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, for the FDA to provide advice, and for the sponsor and the FDA to reach agreement on the next phase of development. Sponsors typically use the meetings at the end of the Phase 2 clinical trial to discuss Phase 2 clinical results and present plans for the pivotal Phase 3 clinical trials that they believe will support approval of the new drug.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the drug and finalize a process for manufacturing the 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, among other things, the manufacturer must develop methods for testing the identity, strength, quality, and purity of the final drug. In addition, 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.

While the IND is active and before approval, progress reports summarizing the results of the clinical trials and nonclinical studies performed since the last progress report must be submitted at least annually to the FDA, and written IND safety reports must be submitted to the FDA and investigators for serious and unexpected suspected adverse events, findings from other studies suggesting a significant risk to humans exposed to the same or similar drugs, findings from animal or *in vitro* testing suggesting a significant risk to humans, and any clinically important increased incidence of a serious suspected adverse reaction compared to that listed in the protocol or investigator brochure.

NDA Review and Approval Process

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, the results of product development nonclinical and clinical trials, along with descriptions of the manufacturing process, analytical tests conducted on the chemistry of the drug, proposed labeling and other relevant information are submitted to the FDA as part of an NDA requesting approval to market the product. The submission of an NDA is subject to the payment of substantial user fees; a waiver of such fees may be obtained under certain limited circumstances.

The results of drug development, preclinical studies and clinical trials are submitted to the FDA as part of an NDA. NDAs also must contain extensive chemistry, manufacturing and control information. An NDA must be accompanied by a significant user fee, which may be waived in certain circumstances. The FDA conducts a preliminary review of an NDA within the first 60 days after submission, before accepting the application for filing, to determine whether it is sufficiently complete to permit substantive review. The FDA may refuse to file any NDA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In this event, the NDA must be resubmitted with the additional information before FDA will review the application. Once the submission has been accepted for filing, the FDA's goal is to review applications within ten months of the filing date, or if the submission qualifies for priority review, six months from the filing. The review process may also be extended for a three-month period for FDA to review additional information deemed a "major amendment" to the application. The FDA may refer an application for a novel drug 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.

Before approving an NDA, the FDA will typically inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCPs. If the FDA determines that the application, manufacturing process, or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

After the FDA evaluates an NDA, it will issue an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the drug with prescribing information for specific indications. 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 usually describes the specific deficiencies in the NDA identified by the FDA and may require additional clinical data, such as an additional pivotal Phase 3 clinical trial or other significant and time-consuming requirements related to clinical trials, nonclinical studies, or manufacturing. If a Complete Response Letter is issued, the sponsor must resubmit the NDA, addressing all of the deficiencies identified in the letter, or withdraw the application. Even if such data and information are submitted, the FDA may decide that the NDA does not satisfy the criteria for approval. Further, FDA's "real time" release of newly issued Complete Response Letters associated with withdrawn or abandoned applications, if applicable to any of our product candidates, can materially impact our business and competitive advantage.

If regulatory approval of a product is granted, such approval will be granted for particular indications and may entail limitations on the indicated uses for which such product may be marketed. For example, the FDA may approve the NDA with a risk evaluation and mitigation strategy ("REMS") to ensure the benefits of the product outweigh its risks. A REMS is a safety strategy to manage a known or potential serious risk associated with a medicine and to enable patients to have continued access to such medicines by managing their safe use. It could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries, and other risk minimization tools. The FDA also may offer conditional approval subject to, among other things, changes to proposed labeling or the development of adequate controls and specifications. Once approved, the FDA may withdraw the product approval if compliance with pre- and post-marketing requirements is not maintained or if problems occur after the product reaches the marketplace. The FDA may also require one or more Phase 4 post-market studies and surveillance to further assess and monitor the product's safety and effectiveness after commercialization, and may limit further marketing of the product based on the results of these post-marketing studies. In addition, new government requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could impact the timeline for regulatory approval or otherwise impact ongoing development programs.

In addition, the FDA and other regulatory authorities may change their policies, issue additional regulations or revise existing regulations, any of which could delay our ability to obtain approvals, increase the costs of compliance or restrict our ability to maintain any regulatory approvals we may have obtained. In June 2024, the U.S. Supreme Court overruled the Chevron doctrine, which gives deference to federal agencies' statutory interpretations in litigation against the agencies where the law is ambiguous.

This Supreme Court decision may invite various stakeholders to bring lawsuits against the FDA or other federal agencies to challenge longstanding decisions and policies, including FDA's statutory interpretations of market exclusivities and the "substantial evidence" requirements for drug approvals, which could undermine the FDA's authority, lead to uncertainties in the industry, and disrupt the agency's normal operations. We cannot predict the full impact of this decision on us or the pharmaceutical industry in general. Further, changes in the leadership of the FDA and other federal agencies under the new Trump administration can result in further changes in the funding, operations, and policies of the federal agencies, which may impact our clinical development plans and timelines.

Expedited Development and Review Programs

The FDA has a fast track designation program that is intended to expedite or facilitate the process for reviewing new drug products that meet certain criteria. Specifically, new drugs are eligible for fast track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. With regard to a fast track product, the FDA may consider for review sections of the NDA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA, the FDA agrees to accept sections of the NDA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the NDA.

Any product submitted to the FDA for approval, including a product with a fast track designation, may also be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. A product is eligible for priority review if it has the potential to provide safe and effective therapy where no satisfactory alternative therapy exists or a significant improvement in the treatment, diagnosis, or prevention of a disease compared to marketed products. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug designated for priority review in an effort to facilitate the review. The FDA endeavors to review applications with priority review designations within six months of the filing date as compared to ten months for review of new molecular entity NDAs under its current PDUFA review goals.

In addition, a product may be eligible for accelerated approval. Drug products intended to treat serious or life-threatening diseases or conditions may be eligible for accelerated approval upon a determination that the product has 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, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a drug receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. In addition, the FDA currently requires pre-approval of promotional materials as a condition for accelerated approval, which could adversely impact the timing of the commercial launch of the product.

The Food and Drug Administration Safety and Innovation Act established a category of drugs referred to as "breakthrough therapies" that may be eligible to receive breakthrough therapy designation. A sponsor may seek FDA designation of a product candidate 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 disease or condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The designation includes all of the fast track program features, as well as more intensive FDA interaction and guidance. The breakthrough

therapy designation is a distinct status from both accelerated approval and priority review, which can also be granted to the same drug if relevant criteria are met. If a product is designated as breakthrough therapy, the FDA will work to expedite the development and review of such drug.

Fast track designation, priority review, accelerated approval, and breakthrough therapy designation do not change the standards for approval, but may expedite the development or approval process. 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. We may explore some of these opportunities for our drug product candidates as appropriate. On December 29, 2022, the Consolidated Appropriations Act, 2023, including the Food and Drug Omnibus Reform Act ("FDORA"), was signed into law. FDORA made several changes to the FDA's authorities and its regulatory framework, including, among other changes, reforms to the accelerated approval pathway, such as requiring the FDA to specify conditions for post-approval study requirements and setting forth procedures for the FDA to withdraw a product on an expedited basis for non-compliance with post-approval requirements.

Post-Approval Requirements

Any products manufactured or distributed by us pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to record-keeping, reporting of adverse experiences, periodic reporting, product sampling and distribution, and advertising and promotion of 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 continuing, annual program fees for any marketed products. Drug manufacturers and their subcontractors are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP, which impose certain procedural and documentation requirements upon us and our third-party manufacturers. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

The FDA may withdraw 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 revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical studies to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. 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 untitled letters;
- clinical holds on post-approval or Phase IV clinical studies, if applicable;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of product license approvals;

- drug 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;
- the issuance of safety alerts, Dear Healthcare Provider letters, press releases, and other communications containing warnings or other safety information about the product; or
- injunctions or the imposition of civil or criminal penalties.

The FDA closely regulates the marketing, labeling, advertising, and promotion of drug products. A company can make only those claims relating to safety and efficacy that are approved by the FDA and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. Failure to comply with 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 tested 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 or permanent injunctions under which specified promotional conduct is changed or curtailed. However, companies may share truthful and not misleading information that is otherwise consistent with a product's FDA-approved labelling.

Orange Book Listing

In seeking approval of an NDA or a supplement thereto, NDA applicants 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 FDA approval, each of the patents listed by the NDA applicant is published in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Upon submission of an abbreviated new drug application ("ANDA") or an NDA submitted under Section 505(b)(2) ("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.

Marketing Exclusivity

Market exclusivity provisions authorized under the FDCA can delay the submission and approval of certain marketing applications for products containing the same active ingredient. The FDCA permits patent term restoration of up to five years as compensation for a patent term lost during product development and FDA regulatory review process to the first applicant to obtain approval of an NDA for a new chemical entity in the United States. Patent-term restoration, however, cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. A drug is a new chemical entity 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 action of the drug substance. During the exclusivity period, the FDA may not approve or even accept for review an ANDA or 505(b)(2) NDA submitted by another company for another drug based on the same active moiety, regardless of whether the drug is intended for the same indication as the original innovative drug or for another indication, where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement to one of the patents listed with the FDA by the innovator NDA holder.

The FDCA alternatively provides three years of marketing exclusivity for an 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 covers only the modification for which the drug received approval on the basis of the new clinical investigations and does not prohibit the FDA from approving ANDAs or 505(b)(2) NDAs for drugs containing the active agent for the original indication or condition of use. 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 any nonclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Pediatric exclusivity is another type of marketing exclusivity available in the United States. Pediatric exclusivity provides for an additional six months of marketing exclusivity attached to another period of exclusivity if a sponsor conducts clinical trials in children in response to a written request from the FDA. The issuance of a written request does not require the sponsor to undertake the described clinical trials.

Other Healthcare Laws

Pharmaceutical manufacturers are subject to additional healthcare laws, regulation, and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which they conduct their business. Such laws include, without limitation, U.S. federal anti-kickback, anti-self-referral, false claims, transparency, including the federal Physician Payments Sunshine Act, consumer fraud, pricing reporting, data privacy, data protection, and security laws and regulations as well as similar foreign laws in the jurisdictions outside the U.S. Similar state and local laws and regulations may also restrict business practices in the pharmaceutical industry, such as state anti-kickback and false claims laws, which may apply to business practices, including but not limited to, research, distribution, sales, and marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, or by patients themselves; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws and regulations that require drug manufacturers to file reports relating to pricing and marketing information; state and local laws which require the tracking of gifts and other remuneration and any transfer of value provided to physicians, other healthcare providers and entities; and state and local laws that require the registration of pharmaceutical sales representatives; and state and local laws governing 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 the Health Insurance Portability and Accountability Act of 1996, thus complicating compliance efforts.

The risk of us being found in violation of these or other laws and regulations is increased by the fact that many have not been fully interpreted by the regulatory authorities or the courts and their provisions are open to various interpretations. These laws and regulations are subject to change, which can increase the resources needed for compliance and delay drug approval or commercialization. Any action brought against us for violations of these laws or regulations, even successfully defended, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Also, we may be subject to private "qui tam" actions brought by individual whistleblowers on behalf of the federal or state governments. Actual or alleged violation of any such laws or regulations may lead to investigations and other claims and proceedings by regulatory authorities and in certain cases, private actors, and violation of any of such laws or any other governmental regulations that apply may result in penalties, including, without limitation, significant administrative, civil and criminal penalties, damages, fines, additional reporting obligations, and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, the curtailment or restructuring of operations, exclusion from participation in government healthcare programs and imprisonment.

Coverage and Reimbursement

Sales of any pharmaceutical product depend, in part, on the extent to which such product will be covered by third-party payors, such as federal, state, and foreign government healthcare programs, commercial insurance, and managed healthcare organizations, and the level of reimbursement for such product by third-party payors. Significant uncertainty exists as to the coverage and reimbursement status of any newly approved product. Decisions regarding the extent of coverage and amount of reimbursement to be provided are made on a plan-by-plan basis. One third-party payor's decision to cover a particular product does not ensure that other payors will also provide coverage for the product. As a result, the coverage determination process can require manufacturers to provide scientific details,

information on cost-effectiveness, and clinical support for the use of a product to each payor separately. This can be a time-consuming process, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

In addition, third-party payors are increasingly reducing reimbursements for pharmaceutical products and related services. The U.S. government and state legislatures have continued implementing cost-containment programs, including price controls, restrictions on coverage and reimbursement and requirements for substitution of generic products. Third-party payors are increasingly challenging the prices charged, examining the medical necessity and reviewing the cost effectiveness of pharmaceutical products, in addition to questioning their safety and efficacy. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit sales of any product. Decreases in third-party reimbursement for any product or a decision by a third-party payor not to cover a product could reduce physician usage and patient demand for the product.

In international markets, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies. 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. 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. Pharmaceutical products may face competition from lower-priced products in foreign countries that have placed price controls on pharmaceutical products and may also compete with imported foreign products. Furthermore, there is no assurance that a product will be considered medically reasonable and necessary for a specific indication, that it will be considered cost-effective by third-party payors, that an adequate level of reimbursement will be established even if coverage is available, or that the third-party payors' reimbursement policies will not adversely affect the ability for manufacturers to sell products profitably.

U.S. Healthcare Reform

In the United States and certain foreign jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes to the healthcare system. In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the "ACA") was signed into law, which substantially changed the way healthcare is financed by both governmental and private insurers in the United States and was intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms.

Since its enactment, there have been executive, judicial and Congressional challenges to certain aspects of the ACA. For example, in June 2021 the U.S. Supreme Court held that Texas and other challengers had no legal standing to challenge the ACA, dismissing the case on procedural grounds without specifically ruling on the constitutionality of the ACA. Thus, the ACA will remain in effect in its current form. It is possible that the ACA will be subject to judicial or Congressional challenges in the future. It is unclear how any such challenges and healthcare measures promulgated by the new Trump administration will impact the ACA, our business, financial condition and results of operations.

Other legislative changes have been proposed and adopted since the ACA was enacted. These changes include aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, effective April 1, 2013, which, due to subsequent legislative amendments, will stay in effect through 2032 with the exception of a temporary suspension implemented under various novel coronavirus disease, relief legislation. Moreover, there has recently been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted legislation designed, among other things, to bring more transparency to product pricing, to review the relationship between pricing and manufacturer patient programs, and to reform government program reimbursement methodologies for pharmaceutical products. For example, effective January 1, 2024, the American Rescue Plan Act of 2021 eliminated the statutory cap on Medicaid Drug Rebate Program rebates that manufacturers pay to state Medicaid programs. Elimination of this cap may require pharmaceutical manufacturers to pay more in rebates than it receives on the sale of products, which could have a material impact on our business.

In August 2022, the Congress passed the Inflation Reduction Act of 2022, which includes prescription drug provisions that have significant implications for the pharmaceutical industry and Medicare beneficiaries, including allowing the federal government to negotiate a maximum fair price for certain high-priced single source Medicare drugs, imposing penalties and excise tax for manufacturers that fail to comply with the drug price negotiation requirements, requiring inflation rebates for all Medicare Part B and Part D drugs, with limited exceptions, if their drug prices increase faster than inflation, and redesigning Medicare Part D to reduce out-of-pocket prescription drug costs for beneficiaries, among other changes. Only high-expenditure single-source drugs that have been approved for at least 7 years (11 years for single-source biologics) can qualify for negotiation, with the negotiated price taking effect two years after the selection year. For 2026, Centers for Medicare & Medicaid Services (“CMS”) selected 10 high-cost Medicare Part D drugs in 2023 and the negotiated maximum fair price for each drug has been announced. CMS has selected 15 additional Medicare Part D drugs for negotiated maximum fair pricing in 2027. For 2028, up to an additional 15 drugs, which may be covered under either Medicare Part B or Part D, will be selected, and for 2029 and subsequent years, up to 20 additional Part B or Part D drugs will be selected. Various industry stakeholders, including pharmaceutical companies and the Pharmaceutical Research and Manufacturers of America, have initiated lawsuits against the federal government asserting that the price negotiation provisions of the Inflation Reduction Act are unconstitutional.

Additionally, the One Big Beautiful Bill Act (OBBBA), which was signed into law in July 2025, includes provisions that will impact the United States healthcare system in various ways, including by cuts to Medicaid and introducing new participant work and eligibility requirements for Medicaid coverage, which are expected to significantly change the administration and applicability of Medicaid coverage. In November 2025, CMS announced a voluntary initiative called the GENEROUS Model (Generating cost Reductions for U.S. Medicaid Model) to introduce the option of most-favored-nation pricing to the Medicaid program, whereby a drug manufacturer may voluntarily offer supplemental rebates to participating state Medicaid programs for a manufacturer’s covered outpatient drugs. We cannot predict the full impact of the OBBBA, executive orders, and new laws focused on reducing prescription drug prices or increasing domestic drug manufacturing capacity, or other measures that may be implemented by the current administration related to drug pricing, drug supply chain and manufacturing in the United States.

Further, the current administration has issued executive orders focused on decreasing prescription drug prices, including directing the Secretary of Health and Human Services to establish a mechanism through which American patients can buy drugs directly from manufacturers who sell at a most-favored-

nation price and directing the U.S. Trade Representative and Secretary of Commerce to take action to ensure foreign countries are not engaged in practices that purposefully and unfairly undercut market prices and drive price hikes in the United States. Government agreements with pharmaceutical companies and any other government measures that use most-favored-nation pricing targets for prescription drugs, including the use of international pricing reference to set drug prices in the United States, or that increase generic drug and biosimilar entry sooner than expected, could materially harm our business, including with respect to our ability to set adequate pricing for new drugs to recover our research and development costs.

In addition, individual states in the United States 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, mechanisms to encourage importation from other countries and bulk purchasing. A number of states are considering or have recently enacted state drug price transparency and reporting laws that could substantially increase our compliance burdens and expose us to greater liability under such state laws once we begin commercialization after obtaining regulatory approval for any of our drug products. FDA has authorized the state of Florida to develop Section 804 Importation Programs to import certain prescription drugs from Canada for a limited period of time to help reduce drug costs, provided that Florida's Agency for Health Care Administration meets the requirements set forth by the FDA. Other states may follow Florida.

We expect that additional state and federal healthcare reform measures will be adopted in the future. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our drug candidates. Complying with any new legislation and regulatory changes could be time-intensive and expensive, resulting in a material adverse effect on our business.

Employees and Human Capital Resources

As of December 31, 2025, we had 19 employees, all of whom were full-time and ten of whom were engaged in research and development activities. Six of our employees hold Ph.D. or M.D. degrees. None of our employees is represented by a labor union or covered under a collective bargaining agreement. 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 and cash incentive plans are to attract, retain and reward personnel through the granting of stock-based and cash-based compensation awards, in order to increase stockholder value and the success of our company by motivating such individuals to perform to the best of their abilities and achieve our objectives.

Corporate Information

We were incorporated in Washington as a corporation in March 2011 under the name M3 Biotechnology, Inc. In October 2015, we converted to a Delaware corporation and subsequently changed our name to "Athira Pharma, Inc." In January 2026 we changed our name to "LeonaBio, Inc." Our principal executive office is located at 18706 North Creek Parkway, Suite 104, Bothell Washington 98011. Our telephone number is (425) 620-8501. Our website is www.leonabio.com. Information contained in, or that can be accessed through, our website is not a part of, and is not incorporated into, this report, and the inclusion of our website address in this report is an inactive textual reference only.

We make available on or through our website certain reports and amendments to those reports that we file with or furnish to the SEC, in accordance with the Exchange Act. These include our annual reports on Form 10-K, our quarterly reports on Form 10-Q, and our current reports on Form 8-K, and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act. We make this information available on or through our website free of charge as soon as reasonably practicable after we electronically file the information with, or furnish it to, the SEC.

Item 1A. Risk Factors

You should carefully consider the following risk factors, in addition to the other information contained in this report, including the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section and our consolidated financial statements and related notes. If any of the events described in the following risk factors and the risks described elsewhere in this report occurs, our business, operating results and financial condition could be seriously harmed. This report also contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those anticipated in the forward-looking statements as a result of factors that are described below and elsewhere in this report. Our Risk Factors are not guarantees that no such conditions exist as of the date of this report and should not be interpreted as an affirmative statement that such risks or conditions have not materialized, in whole or in part.

Risks Related to Our Business and the Development of Our Drug Candidates

We are a clinical-stage biopharmaceutical company with a limited operating history.

We are a clinical-stage biopharmaceutical company dedicated to the development of novel therapeutics for high unmet medical needs. Our limited operating history may make it difficult to evaluate the success of our business. Drug development is a highly uncertain undertaking and involves a substantial degree of risk. To date, we have not completed a pivotal clinical trial, obtained marketing approval for any drug candidate, manufactured a commercial scale drug candidate, or conducted sales and marketing activities necessary for successful drug candidate commercialization. Our history as a company makes any assessment of our future success and viability subject to significant uncertainty. We will encounter risks and difficulties frequently experienced by clinical-stage biopharmaceutical companies in rapidly evolving fields, and we have not yet demonstrated an ability to overcome such risks and difficulties successfully. If we do not address these risks and difficulties successfully, our business will suffer.

We may fail to or be unable to design and execute clinical trials to support marketing approval of lasofoxifene, ATH-1105, or any other drug candidates we seek to develop. We cannot be certain that our current or planned clinical trials or any other future clinical trials will be completed on time or be successful. We cannot guarantee that the FDA or foreign regulatory authorities will agree with our study design, protocol or protocol amendments, or statistical plan, or that they will interpret clinical trial results as we do, and more clinical trials could be required before we are able to submit applications seeking approval of our drug candidates. To the extent that the results of the clinical trials are not satisfactory to the FDA or foreign regulatory authorities for support of a marketing application, we may be required to expend significant resources, which may not be available to us, to conduct additional clinical trials in support of potential approval of our drug candidates. Even if regulatory approval is secured for any of our drug candidates, the terms of such approval may limit the scope and use of our drug candidates, which may also limit their commercial potential.

Our approach to inhibiting estrogen receptor signaling in both wild-type and ESR1-mutated breast cancer through treatment by lasofoxifene exposes us to unforeseen risks. We have limited data from preclinical studies and clinical trials to date, and we cannot be certain that future trials will yield data in support of the safety, efficacy and tolerability of our drug candidates.

We are developing lasofoxifene to treat breast cancer. Lasofoxifene is a next-generation SERM that inhibits estrogen receptor signaling in both wild-type and ESR1-mutated breast cancer. Unlike other endocrine agents that lose potency in the presence of ESR1 mutations such as Y537S and D538G, lasofoxifene maintains full inhibitory activity by stabilizing the receptor in an inactive conformation. Structural and preclinical data show that lasofoxifene fully disrupts the activation helix within the ER ligand-binding domain, uniquely preventing downstream estrogen signaling. This mechanism effectively shuts down the constitutive signaling that drives proliferation and metastasis in ESR1-mutated tumors. Given this unique approach, we cannot be certain of the safety and efficacy of lasofoxifene in applicable patients or that our clinical trials will provide sufficient evidence that our design approach results in the intended therapeutic effect.

We have limited evidence regarding the efficacy, safety and tolerability of lasofoxifene. We or a future partner may ultimately determine that lasofoxifene does not possess certain properties required for therapeutic effectiveness. We or a future partner may spend substantial funds attempting to develop lasofoxifene and never succeed in doing so.

Our approach to targeting neurotrophic factors through the use of small molecules like ATH-1105 is based on a novel therapeutic approach, which exposes us to unforeseen risks. We have limited data from preclinical studies and clinical trials to date, including for ATH-1105, and we cannot be certain that future trials will yield data in support of the safety, efficacy and tolerability of our drug candidates.

We have discovered and are developing small molecule drug candidates to treat neurodegenerative diseases, including ALS. Our drug candidates target an endogenous neurotrophic factor which is expected to protect and repair neuronal networks, which we believe could ultimately result in improvements in clinical outcomes and disease-relevant biomarkers. The therapeutic promise of neurotrophic factors in neurodegenerative diseases had been hampered in earlier therapies by the lack of efficient and non-invasive delivery to the CNS. Our small molecule drug candidates are designed to penetrate the BBB and enhance the activity of a neurotrophic factor, but we cannot be certain of the safety and efficacy of our drug candidates in applicable patients or that our clinical trials will provide sufficient evidence that our design approach results in the intended therapeutic effect.

For example, fosgonimeton, a first-generation small molecule designed to positively modulate the neurotrophic HGF system for potential treatment of CNS disorders, was tested in Phase 1 to 2/3 clinical trials. The primary and all secondary endpoints of our Phase 2 ACT-AD clinical trial of fosgonimeton in AD were not met by protocoled analysis. A subsequent post hoc analysis of the data in a pre-specified subgroup from patients on fosgonimeton without background AChEIs showed a meaningful, but not statistically significant, improvement in both ERP P300 latency and cognitive performance compared to placebo at 26 weeks. Although post hoc analyses cannot be used to establish efficacy, these analyses can be helpful in informing the design of current and future clinical studies. The topline data from our Phase 2/3 LIFT-AD clinical trial of fosgonimeton in AD showed that neither the trial's primary endpoint (the Global Statistical Test ("GST"), a combination of results from measures of cognition (ADAS-Cog11) and function (ADCS-ADL23)) nor its key secondary endpoints of ADAS-Cog11 and ADCS-ADL23 reached statistical significance compared with placebo at 26 weeks. However, both components of GST, cognition (ADAS-Cog11) and function (ADCS-ADL23), directionally favored fosgonimeton treatment, and in pre-specified subgroups characterized by more rapid disease progression (moderate AD and APOE4 carriers), cognition and function improved or stabilized in the fosgonimeton treated group. In addition, data across biomarkers of protein pathology (Aβ42/40, p-Tau181, and p-Tau217), inflammation (GFAP) and neurodegeneration (NfL) showed directional improvements with fosgonimeton treatment that are consistent with the broad neuroprotective mechanism of HGF modulation. Based on these results, we decided to pause further development of fosgonimeton.

Since the readout of topline data from LIFT-AD, in September 2024 we have been focused on advancing the clinical development program for ATH-1105, a next generation small molecule designed for the potential treatment of ALS. We completed our first-in-human Phase 1 clinical trial in healthy volunteers evaluating the safety and tolerability of ATH-1105, in November 2024. The results of the Phase 1 clinical trial showed that ATH-1105 demonstrated a favorable safety profile and was well-tolerated in healthy volunteers, supporting continued clinical development. We have substantially completed preparation activities to enable initiation of a future clinical trial in people living with ALS, either by us or in conjunction with a partner subject to our continued exploration of strategic alternatives focused on maximizing stockholder value; however, our plans, including trial design, timing and other factors have not yet been finalized.

We have limited evidence regarding the efficacy, safety and tolerability of ATH-1105 and other small molecules in our drug product pipeline. We or a future partner may ultimately determine that ATH-1105, or any of our other small molecules, does not possess certain properties required for therapeutic effectiveness. We or a future partner may spend substantial funds attempting to develop these drug candidates and never succeed in doing so.

Our development of lasofoxifene or ATH-1105 may never lead to marketable products.

We are developing lasofoxifene and ATH-1105 to address devastating diseases where current treatment options are limited or ineffective. We have not received regulatory approval for any of our product candidates and cannot be certain that our approach will lead to the development of an approvable or marketable product, alone or in combination with other therapies.

Advancing our product candidates creates significant challenges for us or a future partner, including:

- obtaining marketing approval;
- if lasofoxifene or ATH-1105 is approved, educating medical personnel regarding the potential efficacy and safety benefits, as well as the challenges, of incorporating lasofoxifene or ATH-1105 into existing treatment regimens, including in combination with other treatments or as a monotherapy; and
- establishing the sales and marketing capabilities upon obtaining any marketing approvals to gain market acceptance.

Our prospects as a standalone business are highly dependent on the successful development of lasofoxifene and ATH-1105. Following the September 2024 announcement of topline results from the Phase 2/3 LIFT-AD clinical trial of fosgonimeton showing that neither the trial's primary endpoint nor its key secondary endpoints were met, we announced our intention to focus on advancing the clinical development program for ATH-1105 as a potential treatment for ALS and other neurodegenerative diseases. Like fosgonimeton, ATH-1105 is a small molecule designed to positively modulate the HGF system. Although we are encouraged about the potential for ATH-1105 based on preclinical data, and about the potential of lasofoxifene based on clinical and preclinical data, we or a future partner may decide to discontinue development of some or all of our product candidates, including if we or a future partner do not demonstrate the safety and efficacy of our product candidates in current and future clinical trials.

Our ability to generate revenue and achieve profitability depends significantly on our ability to achieve a number of objectives.

Our business depends entirely on the successful discovery, development and commercialization of our drug candidates. We have no drug products approved for commercial sale and do not anticipate generating any revenue from drug product sales for the next several years, if ever. Our ability to generate drug product revenue will depend heavily on the successful clinical development and eventual commercialization of lasofoxifene, ATH-1105 or any other drug candidates we may seek to develop. Our ability to generate revenue and achieve profitability depends significantly on our ability to achieve a number of objectives, including:

- successful and timely completion of nonclinical and clinical development of our drug candidates and any future drug candidates, as well as the associated costs, including any unforeseen costs;
- establishing and maintaining relationships with contract research organizations ("CROs") and clinical sites for the clinical development, both in the United States and internationally, of our drug candidates and any future drug candidates;

- timely submission of application for and receipt of marketing approvals from applicable regulatory authorities for any drug candidates for which we successfully complete clinical development;
- making any required post-marketing approval commitments to applicable regulatory authorities;
- developing an efficient and scalable manufacturing process for our drug candidates, including obtaining finished products that are appropriately packaged for sale;
- establishing and maintaining commercially viable supply and manufacturing relationships with third parties that can provide adequate, in both amount and quality, products and services to support clinical development and meet the market demand for drug candidates that we develop, if approved;
- successful commercial launch following any marketing approval, including the development of a commercial infrastructure, whether inhouse or with one or more collaborators;
- a continued acceptable safety profile following any marketing approval of our drug candidates;
- commercial acceptance of our drug candidates by patients, the medical community and third-party payors;
- identifying, assessing and developing new drug candidates;
- obtaining, maintaining and expanding patent protection, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- protecting our rights in our intellectual property portfolio;
- defending against third-party patent challenges, derivation proceedings, or interference or infringement claims, if any;
- negotiating favorable terms in any collaboration, licensing or other arrangement that may be necessary or desirable to develop, manufacture or commercialize our drug candidates;
- obtaining coverage and adequate reimbursement by hospitals, government and third-party payors for drug candidates that we develop;
- addressing any competing therapies and technological and market developments; and
- attracting, hiring and retaining qualified personnel.

We may never be successful in achieving our objectives and, even if we are, may never generate revenue that is significant or large enough to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable may decrease the value of our company and could impair our ability to maintain or further our research and development efforts, raise additional necessary capital, grow our business and continue our operations.

We may also experience delays in developing a sustainable, reproducible and scalable manufacturing process or transferring that process to commercial partners, which may prevent us from completing our clinical trials or commercializing our drug candidates on a timely or profitable basis, if at all. Changes in the manufacturing process or facilities will require further comparability analysis and approval by the FDA before implementation, which could delay our clinical trials and drug candidate development, and could require additional clinical trials, including bridging studies and potential confirmatory or Phase 3 registrational trials, to demonstrate consistent and continued safety and efficacy.

We have not previously submitted an NDA to the FDA or similar approval filings to a comparable foreign regulatory authority, for any drug candidate. An NDA or other relevant regulatory filing must include extensive nonclinical and clinical data and supporting information to establish that the drug candidate is safe and effective for each desired indication. The NDA or other relevant regulatory filing must also include significant information regarding the chemistry, manufacturing and controls for the drug product. We cannot be certain that our current or future drug candidates will be successful in clinical trials. Further, even if they are successful in clinical trials, our drug candidates or any future drug candidates may not receive regulatory approval. If we do not receive regulatory approvals for current or future drug candidates, we may not be able to continue our operations. Even if we successfully obtain regulatory approval to market a drug candidate, our revenue will depend, in part, upon the size of the markets in the territories for which we gain regulatory approval and have commercial rights, as well as the availability of competitive products, whether there is sufficient third-party reimbursement and adoption by physicians.

Treatment of central and peripheral nervous system degenerative disorders is a field that has seen very limited success in product development and our research and development efforts may be unsuccessful.

Prior to our in-licensing of lasofoxifene, we have focused our research and development efforts on addressing CNS and PNS degenerative disorders. Collectively, efforts by pharmaceutical companies in the field of CNS and PNS degenerative disorders have seen very limited successes in product development. The development of CNS therapies presents unique challenges, including an imperfect understanding of the biology, the presence of the BBB that can restrict the flow of drugs to the brain, a frequent lack of translatability of preclinical study results in subsequent clinical trials and dose selection, and the product candidate having an effect that may be too small to be detected using the outcome measures selected in clinical trials or if the outcomes measured do not reach statistical significance. There are limited effective therapeutic options available for patients with AD, ALS, and other CNS or PNS disorders. Our future success is highly dependent on the successful development of our technology and our drug candidates for treating CNS and PNS disorders. Developing and, if approved, commercializing our drug candidates for treatment of CNS and PNS disorders subjects us to a number of challenges, including ensuring that we have selected the optimal doses, executing an appropriate clinical trial to test for efficacy and obtaining regulatory approval from the FDA and other regulatory authorities.

We have no marketed proprietary products and have not yet completed any Phase 3 clinical trials, which makes it difficult to assess our ability to develop our future product candidates and commercialize any resulting products independently.

While we have completed Phase 1 and 2/3 clinical trials for product candidates focused on addressing CNS and PNS disorders, we have no previous experience in completing a Phase 3 clinical trial or in completing clinical trials in oncology, and the related regulatory requirements, or the commercialization of any products. We have not yet demonstrated our ability to independently and repeatedly conduct clinical development after Phase 2, obtain regulatory approval, manufacture drug product on a registrational and commercial scale or arrange for a third-party to do so on our behalf, and commercialize therapeutic products. If we do not find a partner to assist with the later stage clinical development and commercialization of our product candidates, we will need to develop such abilities in order to successfully develop and commercialize our product candidates. To develop and commercialize our product candidates independently, we will need to successfully:

- reach agreement with multiple regulatory agencies on clinical and pre-clinical studies required for registration;

- execute our clinical development and manufacturing plans for later-stage product candidates;
- obtain required regulatory approvals in each jurisdiction in which we will seek to commercialize products;
- build and maintain appropriate pre-commercialization capabilities as well as commercial sales, distribution and marketing capabilities;
- build and implement effective market access strategy and gain market acceptance for our future products, if any; and
- manage our spending as costs and expenses increase due to clinical trials, regulatory approvals and commercialization activities.

If we are unsuccessful in accomplishing these objectives, we will not be able to develop and commercialize any future product candidates independently and could fail to realize the potential advantages of doing so.

Clinical development involves a lengthy and expensive process with an uncertain outcome, and results of preclinical studies and earlier clinical trials with a single or few clinical trial sites may not be predictive of eventual safety or effectiveness in large-scale potentially pivotal clinical trials across multiple clinical trial sites. We may encounter substantial delays in clinical trials, or may not be able to conduct or complete clinical trials on the expected timelines, if at all.

We currently have two clinical-stage drug candidates, each for the treatment of different indications. It is impossible to predict when or if any of our drug candidates will prove to be effective and safe in humans or will receive regulatory approval.

Before obtaining regulatory approvals for the commercial sale of our drug candidates, we or a future partner must demonstrate through lengthy, complex and expensive nonclinical studies and clinical trials that our drug candidates are both safe and effective for each target indication. Nonclinical and clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Clinical trials can also be lengthy due to the challenge of identifying patients. Even if patients are successfully identified, they may fail screening criteria and, as a result, not be enrolled in the trial. These uncertainties are enhanced where the diseases or disorders under study lack established clinical endpoints, validated measures of efficacy, as is often the case with disorders for which no drugs have been developed previously and where the product candidates target novel mechanisms. In oncology clinical trials, there is often a need to enroll large numbers of patients, which can be expensive and time-consuming. Failure can occur at any time during the nonclinical study and clinical trial processes, and there is a high risk of failure and we may never succeed in developing marketable products. The results of nonclinical studies and early clinical trials of our drug candidates may not be predictive of the results of later-stage clinical trials. Although product candidates may demonstrate promising results in nonclinical studies and early clinical trials, they may not prove to be safe or effective in subsequent clinical trials. For example, testing on animals occurs under different conditions than testing in humans and therefore, the results of animal studies may not accurately predict safety and effectiveness in humans. There is typically an extremely high rate of attrition from the failure of product candidates proceeding through nonclinical studies and clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy profile despite having progressed through nonclinical studies and initial clinical trials. For example, we paused further development of our fosgonimeton product candidate after our Phase 2 and Phase 2/3 clinical trials failed to meet their primary endpoints and secondary endpoints.

Early, smaller-scale studies, biomarker analyses, and clinical trials with a single or relatively few clinical trial sites may not be predictive of eventual safety and effectiveness in large-scale pivotal clinical trials across multiple clinical trial sites. Even if data from a pivotal clinical trial are positive, regulators may not agree that such data are sufficient for approval and may require that we conduct additional clinical trials, which could materially delay our anticipated development timelines, require additional funding for such additional clinical trials, and adversely impact our business. A number of companies in the

biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or unacceptable safety issues, notwithstanding promising results in earlier trials. Most product candidates that commence nonclinical studies and clinical trials are never approved as products.

In addition, in some instances, there can be significant variability in safety or efficacy results between different nonclinical studies and clinical trials of the same product candidate due to numerous factors, including changes in clinical trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the clinical trial protocols and the rate of dropout among clinical trial participants.

In the future, we may initiate an open-label trial for one or more of our product candidates. An "open-label" clinical trial is one where both the patient and investigator know whether the patient is receiving the investigational product candidate or either an existing approved drug or placebo. Most typically, open-label clinical trials test only the investigational product candidate and sometimes may do so at different dose levels. Open-label clinical trials are subject to various limitations that may exaggerate any therapeutic effect as patients in open-label clinical trials are aware when they are receiving treatment. Open-label clinical trials may be subject to a "patient bias" where patients perceive their symptoms to have improved merely due to their awareness of receiving an experimental treatment. In addition, open-label clinical trials may be subject to an "investigator bias" where those assessing and reviewing the physiological outcomes of the clinical trials are aware of which patients have received treatment and may interpret the information of the treated group more favorably given this knowledge.

We could also encounter delays if a clinical trial is suspended or terminated by us, by the institutional review boards ("IRBs") of the institutions in which such clinical trials are being conducted, by a data safety monitoring board for such clinical trial, or by the FDA or comparable foreign regulatory authorities. Clinical trials can be delayed or terminated or fail to meet endpoints for a variety of reasons, including delays or failures related to:

- the FDA or comparable foreign regulatory authorities disagreeing as to the design or implementation of our clinical trials;
- the FDA or comparable foreign regulatory authorities disagreeing with our clinical development strategy or statistical plan;
- changes in governmental regulations or administrative actions;
- delays in our ability to commence a clinical trial;
- reaching agreement on acceptable terms with prospective CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;
- obtaining IRB approval at each clinical trial site;
- recruiting an adequate number of suitable patients to participate in a clinical trial on a timely basis;
- the number of patients required for clinical trials of our drug candidates may be larger than we anticipate;
- having subjects complete a clinical trial or return for post-treatment follow-up;
- clinical trial sites deviating from clinical trial protocol or dropping out of a clinical trial;
- protocol deviations or non-compliance with GCP requirements, or other data integrity reasons, that cause us or the FDA or other regulatory authorities to exclude data from non-compliant sites or investigators, which may cause the trial to be underpowered to meet the endpoints;
- delays by us or our CROs in qualifying or analyzing patient data at the completion of clinical trials;

- failure to demonstrate a benefit from using a drug candidate;
- addressing subject safety concerns that arise during the course of a clinical trial;
- adding a sufficient number of clinical trial sites; or
- obtaining sufficient supply of drug candidates for use in nonclinical studies or clinical trials from third-party suppliers.

Further, conducting clinical trials in foreign countries, as we may do for our drug 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, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries.

Moreover, principal investigators for our 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 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 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 comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of one or more of our drug candidates. If we experience delays in the completion of, or termination of, any clinical trial of our drug candidates, the commercial prospects of our drug candidates will be harmed, and our ability to generate product revenues from any of these drug candidates will be delayed. Moreover, any delays in completing our clinical trials will increase our costs, slow down our drug candidate development and approval process and jeopardize our ability to commence product sales and generate revenues.

If the results of our current and future clinical trials are inconclusive with respect to the efficacy of our drug candidates, if we or a future partner do not meet the clinical endpoints with statistical and clinically meaningful significance, or if there are safety concerns associated with our drug candidates, we may:

- incur unplanned costs;
- determine to limit or terminate clinical trials, including our open-label extension trial;
- be delayed in or prevented from obtaining marketing approval for our drug candidates;
- 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 including boxed warnings;
- be subject to changes in the way our drug candidates are administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw their approval of the drug product or impose restrictions on its distribution in the form of a modified REMS;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

We may not be successful in integrating lasofoxifene into our pipeline of product candidates.

In December 2025, we entered into agreements with Sermonix Pharmaceuticals, Inc. and Ligand Pharmaceuticals Incorporated granting us exclusive licenses and rights to develop, manufacture and commercialize oral forms of the SERM known as lasofoxifene in all countries and territories of the world except for Asia and certain countries in the Middle East. The development and any potential partnering or independent commercialization of this product candidate will require substantial additional cash to fund the costs associated with conducting the necessary preclinical and clinical testing and obtaining regulatory approval. In addition, the integration of lasofoxifene may also require more time or greater cash resources than we anticipate and expose us to other operational and financial risks, including:

- increased near-term and long-term expenditures;
- exposure to unknown liabilities;
- higher than expected acquisition, disposition or integration costs;
- incurrence of substantial debt or dilutive issuances of equity securities to fund future operations;
- write-downs of assets or incurrence of non-recurring, impairment or other charges;
- difficulty and cost in integrating the development of lasofoxifene into our operations;
- impairment of relationships with key suppliers or service providers involved in the historical development of lasofoxifene;
- inability to hire for key areas of need or retain our employees that are necessary for the development of our product candidates and management of our business; and
- possibility of future litigation.

If any of the foregoing risks materialize it could have a material adverse effect on our business, financial condition and prospects.

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

Because we have limited financial and managerial resources, we focus on research programs and drug candidates that we identify for specific indications. As a result, we may forgo or delay pursuit of opportunities with other drug candidates or for other indications that later prove to have greater commercial potential or a greater likelihood of success. In addition, pursuant to the terms of our December 2025 private placement we are obligated to use the proceeds from the financing for working capital and not for certain other uses, including (1) the repayment of borrowed debt, (2) to redeem any common stock or any of our securities that would entitle the holder thereof to acquire at any time common stock, subject to certain exceptions, (3) for the settlement of any litigation that is outstanding as of the date of the closing of the private placement, and (4) to in-license or develop a drug candidate other than lasofoxifene or any drug or drug candidate in our pipeline as of the date of the closing of the private placement, until the earlier of such time as the topline results of ELAINE-3 become available and the second anniversary of the effective date of the PIPE Securities Purchase Agreement (as defined below). As a result, we may be unable to acquire or in-license other promising drug candidates and technologies or engage in other strategic transactions that may be advantageous.

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 research and development programs and drug 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 drug candidate, we may relinquish valuable rights to that drug 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.

Our long-term prospects depend in part upon discovering, developing and commercializing additional drug candidates, which may fail in development or suffer delays that adversely affect their commercial viability.

Our future operating results are dependent on our ability to successfully discover, develop, obtain regulatory approval for and commercialize drug candidates beyond those we currently have in clinical and nonclinical development. A drug candidate can unexpectedly fail at any stage of nonclinical and clinical development. The historical failure rate for drug candidates is high due to risks relating to safety, efficacy, clinical execution, changing standards of medical care and other unpredictable variables. The results from nonclinical testing or early clinical trials of a drug candidate may not be predictive of the results that will be obtained in later stage clinical trials of the drug candidate.

The success of other future drug candidates we may develop will depend on many factors, including the following:

- generating sufficient data to support the initiation or continuation of clinical trials;
- obtaining regulatory permission to initiate clinical trials;
- contracting with the necessary parties to conduct clinical trials;
- successful enrollment of patients in, and the completion of, clinical trials on a timely basis;
- the timely manufacture of sufficient quantities of the drug candidate for use in clinical trials; and
- adverse events in the clinical trials.

Even if we successfully advance any other future drug candidates into clinical development, their success will be subject to all of the clinical, regulatory and commercial risks described elsewhere in this "Risk Factors" section. Accordingly, we cannot assure you that we will ever be able to discover, develop, obtain regulatory approval of, commercialize or generate significant revenue from our other future drug candidates.

We have been and may in the future be subject to claims, lawsuits, arbitration proceedings, government investigations, securities class action litigation and other legal, regulatory and administrative proceedings and face potential liability and expenses related thereto, which could divert management's attention, and insurance coverage may not be sufficient to cover all costs and damages. This could have a material adverse effect on our business, operating results and financial condition.

In the ordinary course of business, we have been and may in the future be the subject of various legal claims, lawsuits, arbitration proceedings, government investigations, securities class action litigation and other legal, regulatory and administrative proceedings. Any such claims, investigations or proceedings against us, whether meritorious or not, could be time-consuming, result in costly litigation, be harmful to our reputation, require significant management attention and divert significant resources, and the resolution of any such claims, investigations or proceedings could result in substantial damages, settlement costs, fines or penalties that could adversely affect our business, financial condition or operating results or result in harm to our reputation and brand, sanctions, consent decrees, injunctions or other remedies requiring a change in our business practices.

In the past, securities class action litigation has often followed announcement of significant business transactions, such as the sale of a company or other strategic transaction, or of negative events, such as negative results from clinical trials. We may be exposed to such litigation or government or regulatory investigations even if no wrongdoing occurred. Litigation and investigations are usually expensive and divert management's attention and resources, which could adversely affect our business and cash resources and our ability to consummate a potential strategic transaction or the ultimate value our stockholders receive in any such transaction.

Further, under certain circumstances we may have contractual or other legal obligations to indemnify and to incur legal expenses on behalf of investors, directors, officers, employees, customers, vendors or other third-parties. For example, our amended and restated bylaws provide that we will indemnify our directors and officers, and may indemnify our employees, agents and other persons, to the fullest extent permitted by the Delaware General Corporation Law. We have also entered into indemnification agreements with directors and officers that require us, among other things, to indemnify them against claims that may arise due to their service in those capacities. These indemnification agreements also require us to advance expenses reasonably and actually incurred by them in investigating or defending any such claims, and it may be difficult or impossible to recover any advanced expenses if it turns out the person was not entitled to indemnification. If we are required or agree to defend or indemnify, or advance expenses to, any of our investors, directors, officers, employees, customers, vendors or other third-parties, we could incur material costs and expenses that could adversely affect our business, results of operations or financial condition.

Washington State University ("WSU") previously announced that it had undertaken a review of prior claims of potential research misconduct involving our former chief executive officer's doctoral research at WSU. We cannot predict what conclusions WSU has reached or may reach in the future, whether or when WSU would share its conclusions with us or the public, and what effect, if any, this review could have on our business and reputation.

In connection with 2021 claims of potential research misconduct involving our former chief executive officer's research at WSU, WSU previously announced that it would undertake a review of these claims. These claims precipitated an investigation by our board of directors, securities class action and shareholder derivative litigation, and a Department of Justice civil investigation that have all since been resolved. We cannot predict what, if any, effect this review may have on our business and reputation, including with regards to our in-licensed patents and our relationship with WSU, from which we in-license patents underlying our previous lead drug candidate, fosgonimeton.

Any "topline", interim, initial, or preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose preliminary or topline data from our nonclinical studies and clinical trials, which is based on a preliminary analysis of then-available data, and 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 clinical trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the topline or preliminary results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Topline data 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, topline data should be viewed with caution until the final data are available.

From time to time, we may also disclose interim data from our nonclinical studies and clinical trials. Interim data 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 or as patients from our clinical trials continue other treatments for their disease. Adverse differences between preliminary or interim data and final data could significantly harm our business prospects. Further, disclosure of interim data by us or by our competitors could result in volatility in the price of our common stock.

Additionally, we rely on data received from clinical trials, whether preliminary or final, to inform decisions on future clinical trials, including trial design, trial size, and whether or not to initiate additional clinical trials. However, this does not guarantee that our expectations based on earlier data will be realized in future clinical trials. For example, in November 2020, we initiated ACT-AD, an exploratory Phase 2 clinical trial, to better understand the overall effects of fosgonimeton on working memory processing speed and cognitive measures. Topline results of ACT-AD were announced in June 2022. We used these data to help inform strategic decisions around LIFT-AD. In September 2024, we announced the topline results for LIFT-AD, showing that the trial did not meet its primary or key secondary endpoints. Moreover, preliminary or topline results are based on a preliminary analysis of then-available data, and a more comprehensive and full review of the data may result in different conclusions, which could have a negative impact on our decisions regarding any additional 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 drug candidate or drug and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you 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 drug candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

Our reporting of topline or final data for our clinical trials may be delayed and our regulatory submissions or receipt of necessary marketing approvals could be delayed or prevented.

Our projected timeline for announcing our topline data for lasofoxifene, ATH-1105 or any other drug candidates we may seek to develop may be delayed, including, among other reasons, due to possible delays in data cleaning, processing or analysis, either on our part and/or on the part of any of our third-party vendors, which could harm our business, operating results, prospects or financial condition.

We also may not be able to initiate or continue clinical trials for our drug candidates if we are unable to recruit and enroll a sufficient number of eligible patients to participate in these clinical trials through completion of such trials as required by the FDA or other comparable foreign regulatory authorities. Patient enrollment is a significant factor in the timing of clinical trials. Our ability to enroll eligible patients may be limited or may result in slower enrollment than we anticipate. Patient enrollment may also be affected if our competitors have ongoing clinical trials for programs that are under development for the same indications as our drug candidates, and patients who would otherwise be eligible for our clinical trials instead enroll in clinical trials of our competitors' programs. Additionally, publicly reported results of our clinical trials or third-party clinical trials for similar indications may impact enrollment of our trials in progress. If we are unable to locate a sufficient number of such patients, our clinical trial and development plans could be delayed.

In addition, the timely completion of our clinical trials in accordance with their protocols and applicable requirements depends, among other things, on our ability to enroll a sufficient number of participants who remain in the study until its conclusion. If we are delayed or unsuccessful in enrolling the desired number of subjects in our trials, whether as a result of the outcomes of prior trials conducted by us, competing clinical trials, overly stringent eligibility requirements, or other factors, our clinical trial

results could be delayed, the costs of our clinical trials could materially increase, and the overall development timelines for lasofoxifene, ATH-1105, or any other drug candidates we may seek to develop could be negatively impacted. Even if we are successful in enrolling the targeted number of subjects in our trials, the FDA and other regulators may request additional clinical trials with larger numbers of subjects as a condition to any regulatory approval.

Our inability to enroll a sufficient number of patients for our clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether. Further, to the extent any of our clinical trial sites fail to comply with the approved study protocol, good clinical practices, or FDA regulations, we may be required to exclude such sites, participants such sites may have enrolled, as well as the data collected by such sites. If any of these events were to occur, or if we are required to exclude any data for any reason, we may be required to recruit more sites or more participants than we initially thought. Enrollment delays or other delays in our clinical trials may result in increased development costs for our drug candidates and jeopardize our ability to obtain marketing approval for the sale of our drug candidates. Furthermore, even if we are able to enroll a sufficient number of patients for our clinical trials, we may have difficulty maintaining participation in our clinical trials through the treatment and any follow-up periods.

We face significant competition, and if our competitors develop and market technologies or products more rapidly than we do or that are more effective, safer, or less expensive than the drug candidates we develop, our commercial opportunities will be negatively impacted.

The biotechnology and pharmaceutical industries utilize rapidly advancing technologies and are characterized by intense competition. While we believe that our scientific knowledge, technology and development expertise provide us with competitive advantages, we face competitive pressures from both large and small pharmaceutical companies, emerging biotechnology companies, as well as academic, government and private research institutions. Many of our competitors have access to greater financial resources, market presence, expertise in development, preclinical and clinical testing, manufacturing, commercialization, regulatory approval process, or marketing and sales than we do. Specifically, there are a large number of companies developing or marketing treatments for cancer, including many major pharmaceutical and biotechnology companies. Our competitors may compete with us in patient recruitment, use of clinical research organizations, and procurement of operational resources. As a result, our competitors may discover, develop, license or commercialize products before or more successfully than we do.

Drug candidates that we may successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future. Key product features that would affect our ability to effectively compete with other therapeutics include the efficacy, safety and convenience of our drug products. Our competitors may obtain patent protection or other intellectual property rights that limit our ability to develop or commercialize our drug candidates. The availability of reimbursement from government and other third-party payors will also significantly affect the pricing and competitiveness of our drug products. Current and future CMS coverage restrictions on classes of drugs that encompass our drug candidates could have a material adverse impact on our ability to commercialize our drug candidates, if approved, generate revenue and attain profitability. It is unclear how future CMS coverage decisions and policies will impact our business. Our competitors may also obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. For additional information regarding our competition, see the subsections of this Annual Report on Form 10-K titled “Competition” under the section titled “Business”.

We may develop drug candidates in combination with other therapies, which exposes us to additional risks.

We may develop drug candidates in combination with one or more other approved or unapproved therapies. Even if any drug candidate we develop were to receive marketing approval or be commercialized for use in combination with other existing therapies, we would continue to be subject to the risks that the FDA or comparable foreign regulatory authorities outside of the United States could revoke approval of the therapy used in combination with our drug product or that safety, efficacy, manufacturing or supply issues could arise with any of those existing therapies. If the therapies we use in combination with our drug candidates are replaced as the standard of care for the indications we choose for any of our drug candidates, the FDA or comparable foreign regulatory authorities may require us to conduct additional clinical trials. The occurrence of any of these risks could result in our own drug products, if approved, being removed from the market or being less successful commercially.

We also may choose to evaluate drug candidates in combination with one or more therapies that have not yet been approved for marketing by the FDA or comparable foreign regulatory authorities. We will not be able to market and sell any drug candidate we develop in combination with an unapproved therapy for a combination indication if that unapproved therapy does not ultimately obtain marketing approval either alone or in combination with our drug product. In addition, unapproved therapies face the same risks described with respect to our drug candidates currently in development and clinical trials, including the potential for serious adverse effects, delay in their clinical trials and lack of FDA approval. If the FDA or comparable foreign regulatory authorities do not approve these other drugs or revoke their approval of, or if safety, efficacy, quality, manufacturing or supply issues arise with, the drugs we choose to evaluate in combination with our drug candidates we develop, we may be unable to obtain approval of or market such combination therapy.

We may not realize all the anticipated benefits of our corporate rebranding and it may result in unanticipated disruptions to our on-going business.

In order to reflect our strategic shift to focus on developing lasofoxifene, in addition to ATH-1105, in January 2026 we changed our name and ticker symbol on The Nasdaq Stock Market and re-branded our business (the “Corporate Rebrand”). We may face unanticipated disruptions to our business arising from the Corporate Rebrand, and it may expose us to additional risks, including:

- Disruptions or unanticipated delays accessing certain markets or segments due to delays or other issues with regulatory approvals, clinical trials, or other updates arising from or related to the Corporate Rebrand;
- Intellectual property risks associated with the adoption of a new corporate identity and trade dress; and
- Loss of goodwill and brand equity associated with our legacy brand that will become less prominent over time.

The Corporate Rebrand has involved and is expected to continue to involve a significant financial and resource investment as we complete this transition. The anticipated benefits of the Corporate Rebrand may not be achieved within the anticipated timeframe, without additional near or long-term investment, or at all. Any of these factors could negatively impact our brand and reputation, decrease or delay the expected accretive effect of the Corporate Rebrand, and negatively impact the price of our common stock.

Risks Related to Our Financial Position and Capital Needs

We will require substantial additional funding to finance our operations, complete the development and commercialization of our product candidates, and develop and commercialize other current and potential drug candidates. If we are unable to raise this funding and access capital when needed, we may be forced to delay, reduce, or eliminate our drug product development programs, commercialization efforts or other operations.

Since our inception, we have used substantial amounts of cash to fund our operations, and we will continue to incur expenses for the foreseeable future in connection with our ongoing activities, particularly as we continue the research and development of, initiate clinical trials of, and seek marketing approval for, lasofoxifene and ATH-1105. Developing lasofoxifene and ATH-1105 and conducting clinical trials for the potential treatment of ALS and breast cancer, respectively, and any other indications that we may pursue in the future will require substantial amounts of capital. In addition, if we or a future partner obtain marketing approval for our current or any future product candidates, we expect this would result in significant commercialization expenses related to sales, marketing, manufacturing, and distribution. Furthermore, we expect to continue to incur additional costs associated with operating as a public company.

Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. As of December 31, 2025, we had cash, cash equivalents and investments of $88.3 million. Based upon our current operating plan, we estimate that our existing cash, cash equivalents and investments will be sufficient to fund our operating expenses and capital expenditure requirements through at least the next 12 months. On December 18, 2025, we entered into a securities purchase agreement with certain institutional investors pursuant to which we issued and sold, by way of a private placement, shares of common stock and pre-funded warrants to purchase common stock, which, provided us with approximately $90 million in gross proceeds, excluding placement agent fees and offering expenses. We are obligated to use such proceeds for working capital and not for (1) the repayment of borrowed debt, (2) to redeem any common stock or any of our securities that would entitle the holder thereof to acquire at any time common stock, subject to certain exceptions, (3) for the settlement of any litigation that is outstanding as of the date of the closing of the private placement, and (4) to in-license or develop a drug candidate other than lasofoxifene or any drug or drug candidate in our pipeline as of the date of the closing of the private placement, until the earlier of such time as the topline results of ELAINE-3 become available and the second anniversary of the effective date of the PIPE Securities Purchase Agreement. As a result, we may be unable to acquire or in-license other promising drug candidates and technologies or engage in other strategic transactions that may be advantageous.

While we expect to use the proceeds from our December 2025 private placement to fund the clinical development of our product candidates, we will need to raise substantial additional capital to advance these product candidates through later-stage clinical development, partnering or independent commercialization. If we raise additional funds by issuing equity securities, our stockholders will suffer additional dilution, and the terms of any financing may adversely affect the rights of our stockholders.

The amount and timing of our future funding requirements depends on many factors, some of which are outside of our control, including but not limited to:

- the progress, costs, clinical trial design, results of and timing of our clinical trials, including for potential additional indications that we may pursue;
- the willingness of the FDA, EMA and any other regulatory agencies to accept lasofoxifene or ATH-1105 or clinical trial data, as well as data from any completed and planned clinical and nonclinical studies and other work, as the basis for review and approval of lasofoxifene for breast cancer and ATH-1105 for ALS and the potential need for additional clinical trials;
- the outcome, costs and timing of seeking and obtaining FDA, EMA and any other regulatory approvals, including ongoing management of regulatory activities;

- the number and characteristics of drug candidates that we pursue;
- our ability to manufacture sufficient quantities of our drug candidates;
- our need to expand our research and development activities and resources;
- the costs associated with securing and establishing commercialization and manufacturing capabilities;
- the costs of acquiring, licensing or investing in businesses, drug candidates and technologies;
- the cost, timing and outcomes of any litigation involving our company, including securities class actions and government investigations which we may be or may in the future become involved in;
- our ability to maintain, expand and defend the scope of our intellectual property portfolio, including the amount and timing of any payments we may be required to make, or that we may receive, in connection with the licensing, filing, prosecution, defense and enforcement of any patents or other intellectual property rights;
- our need and ability to retain management and hire scientific, clinical and other personnel;
- the effect of competing drugs and drug candidates and other market developments;
- our need to implement additional internal systems and infrastructure, including financial, quality, and reporting systems; and
- the economic and other terms, timing of and success of any collaboration, licensing or other arrangements into which we may enter in the future.

Because the time and efforts required for successful research and development of our product candidates is highly uncertain, we are unable to estimate the actual funds we will require for development and any marketing and commercialization activities for any product candidates that ultimately may be approved for sale.

In addition, in January 2023, we entered into a sales agreement with Cantor Fitzgerald and Co. ("Cantor Fitzgerald") and BTIG, LLC ("BTIG") to sell shares of our common stock having aggregate sales proceeds of up to $75.0 million, from time to time, subject to any applicable limitations on sales pursuant to SEC rules and regulations, through an at-the-market ("ATM") equity offering program under which Cantor Fitzgerald and BTIG are acting as sales agents. We have not sold any securities pursuant to this ATM offering. However, additional funding may not be available to us on acceptable terms or at all. Any such funding may result in dilution to stockholders, imposition of debt covenants and repayment obligations, or other restrictions that may affect our business. If we are unable to obtain funding on a timely basis, we may be required to significantly curtail or abandon one or more of our research or development programs. We also could be required to seek funds through arrangements with collaborative partners or otherwise that may require us to relinquish rights to some of our technologies or drug candidates or otherwise agree to terms unfavorable to us.

We expect to finance our cash needs through a combination of public or private equity offerings, debt financings, collaborations, strategic alliances, licensing arrangements and other marketing or distribution arrangements. In addition, we may seek additional capital to take advantage of favorable market conditions or strategic opportunities even if we believe we have sufficient funds for our current or future operating plans.

Our ability to raise additional funds will depend on financial, economic and other factors, many of which are beyond our control. For example, the recent inflationary economic environment, health epidemics and global conflicts have resulted in a disruption of global financial markets. If the disruption persists or deepens, we could be unable to access additional capital, which could negatively affect our ability to consummate certain corporate development transactions or other important, beneficial or opportunistic investments. If additional funds are not available to us when we need them, on terms that

are acceptable to us, or at all, we may be required to take steps that could adversely impact our business, including delaying, limiting, reducing or terminating nonclinical studies, clinical trials or other research and development activities or eliminating one or more of our development programs altogether, or delaying, limiting or reducing or terminating efforts to prepare for commercialization of any future approved drug products. We have an existing ATM equity offering program, however, as described below, we are currently unable to raise capital through our ATM equity offering program due to our loss of Form S-3 eligibility.

As a result of our failure to timely file a Current Report on Form 8-K, we are currently ineligible to file registration statements on Form S-3, which may impair our ability to raise capital on terms favorable to us, in a timely manner or at all.

Form S-3 permits eligible issuers to conduct registered offerings using a short form registration statement that allows the issuer to incorporate by reference its past and future filings and reports made under the Exchange Act. In addition, Form S-3 enables eligible issuers to conduct primary offerings "off the shelf" under Rule 415 of the Securities Act. The shelf registration process, combined with the ability to forward incorporate information, allows issuers to avoid delays and interruptions in the offering process and to access the capital markets in a more expeditious and efficient manner than raising capital in a standard registered offering pursuant to a Registration Statement on Form S-1.

As a result of our failure to timely file a Current Report on Form 8-K, we are currently ineligible to use a Registration Statement on Form S-3 through December 2026. Our inability to use Form S-3 may significantly impair our ability to raise capital expeditiously to fund our operations and execute our strategy. If we seek to access the capital markets through a registered offering during the period of time that we are unable to use Form S-3, we may be required to publicly disclose the proposed offering and the material terms thereof before the offering commences, we may experience delays in the offering process due to SEC review of a Form S-1 registration statement and we may incur increased offering and transaction costs and other considerations. Disclosing a public offering prior to the formal commencement of an offering may result in downward pressure on our stock price. If we are unable to raise capital through a registered offering, we would be required to conduct our equity financing transactions on a private placement basis, which may be subject to pricing, size and other limitations imposed under the Nasdaq rules, or seek other sources of capital. Our ability to register securities for resale may also be limited as a result of the loss of Form S-3 eligibility. The foregoing limitations on our financing approaches could prevent us from pursuing transactions or implementing business strategies that would be beneficial to our business.

We may become obligated to pay liquidated damages if we fail to obtain effectiveness and maintain effectiveness of registration statements in accordance with the terms of registration rights agreements related to our December 2025 private placement and Sermonix Securities issuance.

In connection with our December 2025 private placement and Sermonix Securities issuance, we granted to the purchasers of securities in the private placement and to Sermonix, respectively, certain resale registration rights pursuant to registration rights agreements with such parties. In connection with such registration rights, the purchasers and Sermonix may be entitled to receive liquidated damages upon the occurrence, or failure to occur, of a number of events relating to the filing, effectiveness and maintenance of effectiveness of a registration statement related to the securities issued in the December 2025 private placement and the Sermonix Securities, respectively. The liquidated damages will be payable upon the occurrence, or failure to occur, of each of those events and each monthly anniversary thereof until cured. The amount of liquidated damages payable per monthly period would be equal to 1% of the aggregate purchase price paid by the purchasers. While we timely filed the resale registration statements in January 2026, as of the date of this report, these resale registration statements have not yet been declared effective by the SEC and we may be unsuccessful in obtaining effectiveness of the resale registration statements in a timely manner and, as a result, would incur

liquidated damages until we regain compliance with our obligations under the registration rights agreements.

We have incurred significant losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future.

We have not generated any revenue from drug product sales and our drug candidates will require substantial additional investment before they may provide us with any revenue. We had net losses of $105.6 million and $96.9 million for the year ended December 31, 2025 and 2024, respectively, and an accumulated deficit of $511.8 million as of December 31, 2025.

We have devoted most of our financial resources to research and development, including our clinical and nonclinical development activities. To date, we have financed our operations primarily with proceeds from the sale and issuance of common stock, convertible preferred stock, common stock warrants, and convertible notes, and to a lesser extent from grant income and stock option exercises.

We expect to incur significant expenses and operating losses for the foreseeable future, including as a result of the following actions that we may take to:

- continue our research and nonclinical and clinical development of our drug candidates;
- expand the scope of our clinical studies for our current and prospective drug candidates;
- initiate additional nonclinical, clinical or other studies for our drug candidates;
- change or add additional manufacturers or suppliers and manufacture drug supply and drug product for clinical trials and commercialization;
- seek regulatory and marketing approvals for any of our drug candidates that successfully complete clinical trials;
- attract, hire and retain additional personnel;
- operate as a public company;
- maintain our facilities and lab space;
- seek to identify and validate additional drug candidates;
- acquire or in-license other drug candidates and technologies or engage in other strategic transactions;
- make milestone or other payments under our in-license or other agreements, including with respect to our license of lasofoxifene;
- maintain, protect and expand our intellectual property portfolio;
- create additional infrastructure to support our operations and our drug product development efforts; and
- incur expenses in connection with potential future legal proceedings, and addressing potential stockholder activism.

Our expenses could increase beyond expectations for a variety of reasons, including if we experience any delays or encounter issues with any of the above, are required by the FDA, the EMA, or other regulatory agencies, domestic or foreign, to perform clinical and other studies in addition to those that we currently anticipate. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity.

Adverse events or perceptions affecting the financial services industry could adversely affect our operating results, liquidity, financial condition and prospects.

Limited liquidity, defaults, non-performance or other adverse developments affecting financial institutions or parties with which we do business, or perceptions regarding these or similar risks, have in the past and may in the future lead to market-wide liquidity problems. For example, in March 2023, Silicon Valley Bank ("SVB") was closed and placed in receivership and subsequently, additional financial institutions have been placed into receivership. We did not hold cash deposits or other accounts with SVB and did not, and as of the date of this report do not, otherwise have a direct business relationship with SVB or similarly situated financial institutions. However, companies that did have a business relationship with SVB faced:

- delayed access to deposits or other financial assets or the uninsured loss of deposits or other financial assets;
- loss of access to revolving existing credit facilities or other working capital sources or the inability to refund, roll over or extend the maturity of, or enter into new credit facilities or other working capital resources;
- potential or actual breach of obligations, including U.S. federal and state wage laws and contracts that required them to maintain letters or credit or other credit support arrangements; and
- termination of cash management arrangements or delays in accessing or actual loss of funds subject to cash management arrangements.

As a result of U.S. government intervention, account holders subsequently regained access to their accounts, including the uninsured portion of deposit accounts; however, borrowers under credit agreements, letters of credit and certain other financial instruments with SVB and similarly situated financial institutions were unable to access to such sources of liquidity. There is no guarantee that the U.S. government will intervene to provide access to uninsured funds in the future in the event of the failure of other financial institutions, or that they would do so in a timely fashion. In such an event, parties with which we have commercial agreements may be unable to satisfy their obligations to, or enter into new commercial arrangements with, us.

Concerns regarding the U.S. or international financial systems could impact the availability and cost of financing, thereby making it more difficult for us to acquire financing on acceptable terms or at all.

Any of these risks could materially impact our operating results, liquidity, financial condition and prospects.

The value of our investments is subject to significant capital markets risk related to changes in interest rates and credit spreads as well as other investment risks, which may adversely affect our operating results, liquidity, financial condition and prospects.

Our results of operations are affected by the performance of our investment portfolio. Our excess cash is invested by an external investment management service provider, under the direction of our management in accordance with our investment policy. The investment policy defines constraints and guidelines that restrict the asset classes that we may invest in by type, duration, quality and value. Our investments are subject to market-wide risks, and fluctuations, as well as to risks inherent in particular securities. The failure of any of the investment risk strategies that we employ could have a material adverse effect on our operating results, liquidity, financial condition and prospects.

The value of our investments is exposed to capital market risks, and our results of operations, liquidity, financial condition or cash flows could be adversely affected by realized losses, impairments and changes in unrealized positions as a result of: significant market volatility, changes in interest rates, changes in credit spreads and defaults, a lack of pricing transparency, a reduction in market liquidity, declines in equity prices, changes in national, state/provincial or local laws and the strengthening or weakening of foreign currencies against the U.S. dollar. Levels of write-down or impairment are impacted by our assessment of the intent to sell securities that have declined in value as well as actual losses as a result of defaults or deterioration in estimates of cash flows. If we reposition or realign portions of the investment portfolio and sell securities in an unrealized loss position, we will incur realized losses. Any such charge may have a material adverse effect on our results of operations and business.

Our ability to use net operating losses to offset future taxable income may be subject to certain limitations.

As of December 31, 2025, we had federal net operating loss carryforwards("NOLs") to offset future taxable income of approximately $9.5 million and federal tax credit carryforwards of approximately $17.2 million, which expire over a period of 6 to 12 years. Federal NOLs of $259.8 million generated after the 2017 tax year will carry forward indefinitely and will be subject to the 80% of taxable income limitation. As of December 31, 2025, we also had state NOLs of $5 million, which expire over a period of 17 to 20 years. A lack of future taxable income would adversely affect our ability to utilize these NOLs.

In addition, under Section 382 of the Internal Revenue Code of 1986, as amended("the Code"), a corporation that undergoes an "ownership change," generally defined as a greater than 50% change (by value) in ownership by "5 percent shareholders" over a rolling three-year period, is subject to limitations on its ability to utilize its pre-change NOLs and tax credit carryforwards to offset post-change taxable income or taxes. We may have already experienced one or more ownership changes through our equity offerings and other changes in our stock ownership.

Depending on the timing of any future utilization of our pre-change NOLs and tax credit carryforwards, we may be limited as to the amount that can be utilized each year as a result of such previous ownership changes. In addition, future changes in our stock ownership as well as other changes that may be outside of our control, could result in additional ownership changes under Section 382 of the Code. Our utilization of state NOLs may also be limited under similar provisions of state law. We have recorded a full valuation allowance related to our NOLs and other deferred tax assets due to the uncertainty of the ultimate realization of the future benefits of those assets.

Changes in tax laws could have a material adverse effect on our business, cash flows, results of operations or financial condition.

We are subject to the tax laws, regulations, and policies of several taxing jurisdictions. Changes in tax laws, as well as other factors, could cause us to experience fluctuations in our tax obligations and effective tax rates and otherwise adversely affect our tax positions or our tax liabilities. For example, in August 2022, as part of the Inflation Reduction Act of 2022 ("IRA"), the United States enacted a 1% excise tax on stock buybacks and a 15% alternative minimum tax on adjusted financial statement income. Additionally, beginning in 2022, the Code eliminated the right to deduct research and development expenditures and instead requires taxpayers to capitalize and amortize U.S. and foreign research and development expenditures over five and 15 tax years, respectively. The One Big Beautiful Bill Act ("OBBB"), enacted on July 4, 2025, revised these rules, permitting the deduction of certain U.S. research and development expenditures incurred in tax years beginning on or after January 1, 2025, but expenditures attributable to research and development conducted outside the U.S. must continue to be capitalized and amortized over a 15-year period. We do not anticipate a material impact on the Company resulting from the other provisions of OBBB, however, we continue to evaluate the full impact of OBBB on the Company. Additionally, many countries and local jurisdictions and organizations such as the Organization for Economic Cooperation and Development have proposed or implemented new tax laws or changes to existing tax laws, including additional taxes on payroll or employees. Any new or changes to tax laws could adversely affect our effective tax rate, operating results, tax credits or incentives or tax payments, which could have a material adverse effect on our business, cash flows, results of operations or financial condition.

Risks Related to Regulatory Approval and Other Legal Compliance Matters

The regulatory approval processes of the FDA and other comparable foreign regulatory authorities are lengthy, time consuming and inherently unpredictable. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals for our drug candidates, we will not be able to commercialize, or will be delayed in commercializing, our drug candidates, and our ability to generate revenue will be materially impaired.

Our drug candidates and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale, distribution, import and export are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable foreign regulatory authorities. Before we can commercialize any of our drug candidates, we must obtain marketing approval.

Obtaining approval by the FDA and other comparable foreign regulatory authorities is unpredictable, typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the type, complexity and novelty of the product candidates involved. In addition, approval policies, regulations or 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, which may cause delays in the approval or the decision not to approve an application. Further, securing regulatory approval also requires the submission of information about the drug manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional nonclinical, clinical or other data. Even if we eventually complete clinical testing and receive approval for our drug candidates, the FDA and other comparable foreign regulatory authorities may approve our drug candidates for a more limited indication or a narrower patient population than we originally requested or may impose other prescribing limitations or warnings that limit the drug product's commercial potential. We have not submitted for, or obtained, regulatory approval for any drug candidate, and it is possible that none of our drug candidates will ever obtain regulatory approval. Further, development of our drug candidates or regulatory approval may be delayed for reasons beyond our control.

Applications for our drug candidates could fail to receive regulatory approval for many reasons, including the following:

- the FDA or other comparable foreign regulatory authorities may disagree with the design, implementation or results of our clinical trials;
- the FDA or other comparable foreign regulatory authorities may determine that our drug candidates are not safe and effective or have undesirable or unintended side effects, toxicities or other characteristics that preclude our obtaining marketing approval or prevent or limit commercial use;
- the population studied in the clinical trial may not be sufficiently broad or representative to assure efficacy and safety in the full population for which we seek approval;
- the FDA or other comparable foreign regulatory authorities may disagree with our interpretation of data from nonclinical studies or clinical trials;
- we may be unable to demonstrate to the FDA or other comparable foreign regulatory authorities that our drug candidate's risk-benefit ratio for its proposed indication is acceptable;
- the FDA or other comparable foreign regulatory authorities may fail to approve the manufacturing processes, test procedures and specifications 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 other comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval or resulting in delays in our regulatory approval, including, for example, legislation or agency policies that aim to reform the accelerated approval process and FDA's increased scrutiny of post-approval confirmatory studies, which can result in withdrawal of accelerated approval if such studies fail to confirm a clinical benefit.

This lengthy approval process, as well as the unpredictability of the results of clinical trials, may result in our failing to obtain regulatory approval to market any of our drug candidates, which would significantly harm our business, results of operations and prospects. In addition, even if we obtain approval of our drug candidates, regulatory authorities may approve any of our drug candidates for fewer or more limited indications than we request, may impose significant limitations in the form of narrow indications, warnings, or a REMS. Regulatory authorities may not approve the price we intend to charge for drug products we may develop, may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a drug candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that drug candidate. Any of the foregoing scenarios could seriously harm our business.

Of the large number of drugs in development, only a small percentage successfully complete the FDA or comparable foreign regulatory approval processes and are commercialized. The lengthy approval processes as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market our drug candidates, which would significantly harm our business, results of operations and prospects.

Further, under the current administration, agency reorganization, government shutdown, lapse of U.S. government appropriations, and mass layoffs due to the federal government reduction in force initiative may impact the normal operations at the FDA as well as other federal agencies. FDA may lack adequate staff and resources to meet current review, approval, and inspection schedules, which could delay our anticipated timelines. In January 2025, President Trump issued an executive order entitled "Unleashing Prosperity Through Deregulation", which calls for at least 10 existing regulations to be repealed whenever an executive department or agency publicly proposes for notice and comment or otherwise promulgates a new regulation. Fewer agency guidance documents could interfere with FDA programs or lead to more Complete Response Letters or refusals to approve products. Recent developments at the FDA include implementation of Elsa, a generative AI tool, across all centers at the agency, announcement of a plan to phase out animal testing for monoclonal antibodies and certain other drugs, and the announcement of a new Commissioner's National Priority Voucher program to companies supporting certain U.S. national health priorities and interests. FDA has also increased its scrutiny of foreign drug manufacturing facilities and other contractors based in China, especially with respect to the transfer of biological materials, genetic data, and other sensitive data of American patients to parties located in China. Further, FDA's "real-time" release of newly issued Complete Response Letters associated with withdrawn or abandoned applications, if applicable to any of our product candidates, can materially impact our competitive advantage and intellectual property. There is significant uncertainty in the industry and how federal agencies like the FDA will change in the coming years under the current administration. It is unclear how our industry and our clinical programs will be impacted by policies and regulations implemented under the current administration or other executive orders. To the extent agency changes and government shutdown lead to disruptions in FDA's operations, including changes in funding for certain programs at the FDA, correspondence and regulatory review processes with the FDA may be materially delayed.

Our current or future drug candidates may cause significant adverse events, toxicities or other undesirable side effects when used alone or in combination with other approved products or investigational new drugs that may result in a safety profile that could inhibit regulatory approval, prevent market acceptance, limit their commercial potential or result in significant negative consequences.

As is the case with pharmaceuticals generally, it is likely that there may be side effects and adverse events associated with our drug candidates' use. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. Undesirable side effects caused by our drug candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or comparable foreign regulatory authorities. The drug-related side effects could affect patient recruitment or the ability of enrolled patients to complete the clinical trial or result in potential product liability claims. Any of these occurrences may harm our business, financial condition and prospects significantly.

If our drug candidates are associated with undesirable side effects or have unexpected characteristics in nonclinical studies or clinical trials when used alone or in combination with other approved products or investigational new drugs we may need to interrupt, delay or abandon their development or limit development to more narrow uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Treatment-related side effects could also affect patient recruitment or the ability of enrolled subjects to complete the clinical trial, or result in potential product liability claims. Any of these occurrences may prevent us from achieving or maintaining market acceptance of the affected drug candidate and may harm our business, financial condition and prospects significantly.

Patients in our clinical trials may in the future suffer significant adverse events or other side effects not observed in our nonclinical studies or previous clinical trials. Some of our drug candidates may be used as chronic therapies or be used in populations for which safety concerns may be particularly scrutinized by regulatory agencies. In addition, if our drug candidates are used in combination with other therapies, our drug candidates may exacerbate adverse events associated with the therapy. Patients treated with our drug candidates may also be undergoing separate treatments which can cause side effects or adverse events that are unrelated to our drug candidates, but may still impact the success of our clinical trials, including, for example, by interfering with the effects of our drug candidates.

If significant adverse events or other side effects are observed in any of our current or future clinical trials, we may have difficulty recruiting patients to the clinical trials, patients may drop out of our clinical trials, or we may be required to abandon the clinical trials or our development efforts of that drug candidate altogether. We, the FDA other comparable regulatory authorities or an IRB may suspend clinical trials of a drug candidate at any time for various reasons, including a belief that subjects in such clinical trials are being exposed to unacceptable health risks or adverse side effects. Some potential therapeutics developed in the biotechnology industry that initially showed therapeutic promise in early-stage clinical trials have later been found to cause side effects that prevented their further development. Even if the side effects do not preclude the drug candidate from obtaining or maintaining marketing approval, undesirable side effects may inhibit market acceptance due to its tolerability versus other therapies. Any of these developments could materially harm our business, financial condition and prospects.

Further, if any of our drug candidates obtains marketing approval, toxicities associated with such drug candidates and not seen during clinical testing may also develop after such approval and lead to a requirement to conduct additional clinical safety trials, additional contraindications, warnings and precautions being added to the drug label, significant restrictions on the use of the drug product or the withdrawal of the drug product from the market. We cannot predict whether our drug candidates will cause toxicities in humans that would preclude or lead to the revocation of regulatory approval based on nonclinical studies or early-stage clinical trials.

Obtaining and maintaining regulatory approval of our drug candidates in one jurisdiction does not mean that we will be successful in obtaining regulatory approval of our drug candidates in other jurisdictions.

Obtaining and maintaining regulatory approval of our drug candidates in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction. For example, even if the FDA grants marketing approval of a drug candidate, comparable regulatory authorities in foreign jurisdictions must also approve the manufacturing, marketing and promotion and reimbursement of the drug candidate in those countries. However, a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from those in the United States, including additional nonclinical studies or clinical trials as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the United States, a drug candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our drug products is also subject to approval.

We may also submit marketing applications in other countries. Regulatory authorities in jurisdictions outside of the United States have requirements for approval of drug candidates with which we must comply prior to marketing in those jurisdictions. Obtaining foreign regulatory approvals and establishing and maintaining compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our drug products in certain countries. If we or any future collaborator fail to comply with the regulatory requirements in international markets or fail to receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of our potential drug candidates will be harmed.

Even if we receive regulatory approval of our drug candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our drug candidates.

Any regulatory approvals that we receive for our drug candidates will require surveillance to monitor the safety and efficacy of the drug candidate. The FDA may also require a REMS in order to approve our drug candidates, which could entail requirements for a medication guide, physician communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. In addition, if the FDA or a comparable foreign regulatory authority approves our drug candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for our drug candidates will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with cGMP regulations, good laboratory practice regulations and GCP regulations for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with our drug candidates, including adverse events 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 our drug candidates, withdrawal of the drug product from the market or voluntary or mandatory product recalls;
- manufacturing delays and supply disruptions where regulatory inspections identify observations of noncompliance requiring remediation;
- revisions to the labeling, including limitation on approved uses or the addition of additional warnings, contraindications or other safety information, including boxed warnings;
- imposition of a REMS, which may include distribution or use restrictions;

- requirements to conduct additional post-market clinical trials to assess the safety of the drug product;
- fines, warning letters or holds on clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or suspension or revocation of approvals;
- product seizure or detention, or refusal to permit the import or export of our drug candidates; and
- injunctions or the imposition of civil or criminal penalties.

The FDA's and other regulatory authorities' policies may change, and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our drug candidates. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained, and we may not achieve or sustain profitability. We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. It is difficult to predict how current and future legislation, executive actions, and litigation, including the executive orders, will be implemented, and the extent to which they will impact our business, our clinical development, and the FDA's and other agencies' ability to exercise their regulatory authority, including FDA's pre-approval inspections and timely review of any regulatory filings or applications we submit to the FDA. To the extent any executive actions impose constraints on FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted. In 2024, the U.S. Supreme Court overruled the Chevron doctrine, which gave deference to regulatory agencies' statutory interpretations in litigation against federal government agencies, such as the FDA, where the law is ambiguous. This landmark Supreme Court decision may invite more companies and other stakeholders to bring lawsuits against the FDA to challenge longstanding decisions and policies of the FDA, including FDA's statutory interpretations of market exclusivities and the "substantial evidence" requirements for drug approvals, which could undermine the FDA's authority, lead to uncertainties in the industry, and disrupt the FDA's normal operations, any of which could impact the FDA's review of our regulatory submissions. Further, mass layoffs, agency reorganizations, increased attrition, and changes in the leadership of the FDA and other federal agencies under the current administration may lead to new policies and changes in the regulations that could increase our compliance costs or delay our clinical development and timelines. We cannot predict the full impact of this decision, future judicial challenges brought against the FDA, or the nature or extent of government regulation that may arise from future legislation or administrative action.

Moreover, the FDA strictly regulates the promotional claims that may be made about drug products. In particular, a product may not be promoted for uses that are not approved by the FDA as reflected in the product's approved labeling. 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 civil, criminal and administrative penalties. The occurrence of any event or penalty described above may inhibit our ability to commercialize our drug candidates and generate revenue and could require us to expend significant time and resources in response and could generate negative publicity.

Disruptions at the FDA, the SEC and other government agencies under the current administration, funding cuts or shortages, global health concerns, or other events could hinder their ability to hire and retain key leadership and other personnel, or otherwise prevent new or modified products from being developed, approved or commercialized in a timely manner or at all, 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 changes in the administration, changes in the agency leadership, agency reorganization, mass layoffs, budget cuts, lapse in government appropriations, and other initiatives implemented by the Department of Government Efficiency, changes in 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, and other events that may otherwise affect the FDA's ability to perform routine functions. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of 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.

To the extent the FDA's normal operations are disrupted or delayed, for example, due to executive orders and other policies promulgated by the administration, travel restrictions, public health or geopolitical issues, staffing shortages or layoffs, government shutdown, or changes in funding, the FDA may not be able to complete the necessary inspections or provide feedback in a timely manner during our clinical development or review period. If any such delays or disruptions were to occur, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns or delays could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

We may attempt to secure approval from the FDA or comparable foreign regulatory authorities through the use of accelerated approval pathways. If we are unable to obtain such approval, we may be required to conduct additional nonclinical studies or clinical trials beyond those that we contemplate, which could increase the expense of obtaining, and delay the receipt of, necessary marketing approvals. Even if we receive accelerated approval from the FDA, if our confirmatory trials do not verify clinical benefit, or if we do not comply with rigorous postmarketing requirements, the FDA may seek to withdraw accelerated approval.

We may seek an accelerated approval for one or more of our drug candidates. Under the accelerated approval program, the FDA may grant accelerated approval to a product candidate designed to treat a serious or life-threatening condition that provides meaningful therapeutic benefit over available therapies upon a determination that the product candidate has an effect on a surrogate endpoint or intermediate clinical endpoint that is reasonably likely to predict clinical benefit. The FDA considers a clinical benefit to be a positive therapeutic effect that is clinically meaningful in the context of a given disease, such as irreversible morbidity or mortality. For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement or other measure that is thought to predict clinical benefit, but is not itself a measure of clinical benefit. An intermediate clinical endpoint is a clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit. The accelerated approval pathway may be used in cases in which the advantage of a new drug over available therapy may not be a direct therapeutic advantage, but is a clinically important improvement from a patient and public health perspective. If granted, accelerated approval is usually contingent on the sponsor's agreement to conduct, in a diligent manner, additional post-approval confirmatory studies to verify and describe the drug's clinical benefit. If such post-approval studies fail to confirm the drug's clinical benefit, the FDA may withdraw its approval of the drug.

Prior to seeking accelerated approval for any of our drug candidates, we intend to seek feedback from the FDA and will otherwise evaluate our ability to seek and receive accelerated approval. There can be no assurance that after our evaluation of the feedback and other factors we will decide to pursue or submit an NDA for accelerated approval or any other form of expedited development, review or approval. Similarly, there can be no assurance that after subsequent FDA feedback we will continue to pursue or apply for accelerated approval or any other form of expedited development, review or approval, even if we initially decide to do so. Furthermore, if we decide to submit an application for accelerated approval or receive an expedited regulatory designation (e.g., breakthrough therapy designation) for our drug candidates, there can be no assurance that such submission or application will be accepted or that any expedited development, review or approval will be granted on a timely basis, or at all. The FDA or other comparable foreign regulatory authorities could also require us to conduct further studies prior to considering our application or granting approval of any type. A failure to obtain accelerated approval or any other form of expedited development, review or approval for our drug candidates would result in a longer time period to commercialization of such drug candidate, could increase the cost of development of such drug candidate and could harm our competitive position in the marketplace.

Further, to the extent the FDA materially changes its policies or regulatory requirements with respect to the accelerated approval program or its internal review process for such program, our clinical development plans and regulatory approval under such program could be materially impacted or delayed. On December 29, 2022, the Consolidated Appropriations Act, 2023, including the FDORA was signed into law. FDORA made several changes to the FDA's authorities and its regulatory framework, including, among other changes, reforms to the accelerated approval pathway, such as requiring the FDA to specify conditions for post-approval study requirements and setting forth procedures for the FDA to withdraw a product on an expedited basis for non-compliance with post-approval requirements. In January 2025, the Office of Inspector General ("OIG") raised concerns with FDA's accelerated approval of three of the 24 drugs review by OIG. It is unclear how this OIG report, future policy changes, changes in the leadership of FDA, and new FDA regulations, including those that may be implemented under the current administration, will impact NDAs and our clinical development programs.

We may face difficulties from changes to current regulations and future legislation. Healthcare legislative measures aimed at reducing healthcare costs may have a material adverse effect on our business and results of operations.

The United States and many foreign jurisdictions have enacted or proposed legislative and regulatory changes affecting the healthcare system that could prevent or delay marketing approval of our drug candidates or any future drug candidates, restrict or regulate post-approval activities and affect our ability to profitably sell a drug product for which we obtain marketing approval. Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example:

- changes to our manufacturing arrangements;
- additions or modifications to drug product labeling;
- the recall or discontinuation of our drug products; or
- additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. For example, in March 2010, the ACA was passed, which substantially changed the way healthcare is financed by both governmental and private insurers, and significantly impacted the U.S. pharmaceutical industry. The ACA contained provisions that may reduce the profitability of drug products through increased rebates for drugs reimbursed by Medicaid programs, extension of Medicaid rebates to Medicaid managed care plans, mandatory discounts for certain Medicare Part D beneficiaries and annual fees based on pharmaceutical companies' share of sales to federal health care programs. The Medicaid Drug Rebate Program requires pharmaceutical manufacturers to enter into and have in

effect a national rebate agreement with the U.S. Department of Health and Human Services Secretary as a condition for states to receive federal matching funds for the manufacturer's outpatient drugs furnished to Medicaid patients. The ACA made several changes to the Medicaid Drug Rebate Program, including increasing the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program, extending the rebate program to individuals enrolled in Medicaid managed care organizations. The ACA also established annual fees and taxes on manufacturers of certain branded prescription drugs, and created a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 70% (increased pursuant to the Bipartisan Budget Act of 2018, effective as of 2019) point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D. In December 2020, CMS issued a final rule implementing significant manufacturer price reporting changes under the Medicaid Drug Rebate Program, including regulations that affect manufacturer-sponsored patient assistance programs subject to pharmacy benefit manager accumulator programs and Best Price reporting related to certain value-based purchasing arrangements. Under the American Rescue Plan Act of 2021, effective January 1, 2024, the statutory cap on Medicaid Drug Rebate Program rebates that manufacturers pay to state Medicaid programs will be eliminated. Elimination of this cap may require pharmaceutical manufacturers to pay more in rebates than it receives on the sale of products, which could have a material impact on our business.

As discussed above, since its enactment, there have been judicial and Congressional challenges to certain aspects of the ACA. On January 28, 2021, President Biden issued an executive order to initiate a special enrollment period for purposes of obtaining health insurance coverage through the ACA marketplace, which also instructs certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare. In June 2021, the United States Supreme Court held that Texas and other challengers had no legal standing to challenge the ACA, dismissing the case without specifically ruling on the constitutionality of the ACA. Further, on August 16, 2022, President Biden signed the IRA into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in ACA marketplaces through plan year 2025. The IRA also eliminates the "donut hole" under the Medicare Part D program beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost through a newly established manufacturer discount program. It is possible that the ACA will be subject to judicial or Congressional challenges in the future. It is unclear how additional challenges and healthcare reform measures of the current administration will impact the ACA. Complying with any new legislation and regulatory requirements could be time-intensive and expensive, resulting in a material adverse effect on our business.

The Bipartisan Budget Act of 2018 also amended the ACA, effective January 1, 2019, by increasing the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and closing the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole." CMS published a final rule permitting further collections and payments to and from certain ACA qualified health plans and health insurance issuers under the ACA risk adjustment program in response to the outcome of federal district court litigation regarding the method CMS uses to determine this risk adjustment. In addition, CMS has published a final rule to give states greater flexibility, starting in 2020, in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces. The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers, including hospitals, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Other legislative changes include aggregate reductions to Medicare payments to providers of up to 2% per fiscal year pursuant to the Budget Control Act of 2011, which began in 2013 and will remain in effect through 2032 with the exception of a temporary suspension implemented under various COVID-19 relief legislation.

There has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. For example, in July 2021, the Biden administration released an executive order, “Promoting Competition in the American Economy,” with multiple provisions aimed at increasing competition for prescription drugs. In August 2022, Congress passed the IRA, which includes prescription drug provisions that have significant implications for the pharmaceutical industry and Medicare beneficiaries, including allowing the federal government to negotiate a maximum fair price for certain high-priced single source Medicare drugs, imposing penalties and excise tax for manufacturers that fail to comply with the drug price negotiation requirements, requiring inflation rebates for all Medicare Part B and Part D drugs, with limited exceptions, if their drug prices increase faster than inflation, and redesigning Medicare Part D to reduce out-of-pocket prescription drug costs for beneficiaries, among other changes. Further, the Biden administration released an additional executive order on October 14, 2022, directing the U.S. Department of Health and Human Services ("HHS") to submit a report within ninety (90) days on how the Center for Medicare and Medicaid Innovation can be further leveraged to test new models for lowering drug costs for Medicare and Medicaid beneficiaries. Various industry stakeholders, including pharmaceutical companies, the U.S. Chamber of Commerce, the National Infusion Center Association, the Global Colon Cancer Association, and the Pharmaceutical Research and Manufacturers of America, have initiated lawsuits against the federal government asserting that the price negotiation provisions of IRA are unconstitutional. Further, the current administration has issued executive orders focused on decreasing prescription drug prices, including directing the Secretary of Health and Human Services to establish a mechanism through which American patients can buy drugs directly from manufacturers who sell at a most-favored-nation price and directing the U.S. Trade Representative and Secretary of Commerce to take action to ensure foreign countries are not engaged in practices that purposefully and unfairly undercut market prices and drive price hikes in the United States. Government agreements with pharmaceutical companies and other government measures that use most-favored-nation pricing targets for prescription drugs, including the use of international pricing reference to set drug prices in the United States, or that increase generic and biosimilar drug entry sooner than expected, can have a material adverse effect on our industry, ability to set adequate pricing for new drugs to recover R&D costs, ability to attract potential investors and potential buyers in the future. We cannot predict the full impact of the executive orders focused on reducing prescription drug prices or increasing domestic drug manufacturing capacity, or other measures that may be implemented by the current administration related to drug pricing, drug supply chain and manufacturing in the United States. The impact of ongoing and future judicial challenges as well as other legislative, executive, and administrative actions and any future healthcare measures and agency rules implemented by the current administration on us and the pharmaceutical industry as a whole is unclear. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our drug candidates if approved.

Current and future CMS coverage restrictions on classes of drugs that encompass our drug candidates could have a material adverse impact on our ability to commercialize our drug candidates, if approved, generate revenue and attain profitability. It is unclear how future CMS coverage decisions and policies will impact our business.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological 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. In addition, regional health care authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other health care programs. These measures could reduce the ultimate demand for our drug products, once approved, or put pressure on our drug product pricing. A number of states are considering or have recently enacted state drug price transparency and reporting laws that could substantially increase our compliance burdens and expose us

to greater liability under such state laws once we begin commercialization after obtaining regulatory approval for any of our drug products. Further, FDA has authorized the state of Florida to develop Section 804 Importation Programs to import certain prescription drugs from Canada for a limited period to help reduce drug costs, provided that Florida's Agency for Health Care Administration meets the requirements set forth by the FDA. Other states may follow Florida. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our drug candidates or additional pricing pressures.

Our revenue prospects could be affected by changes in healthcare spending and policy in the United States and abroad. We operate in a highly regulated industry and new laws, regulations or judicial decisions, or new interpretations of existing laws, regulations or decisions, related to healthcare availability, the method of delivery or payment for healthcare products and services could negatively impact our business, operations and financial condition.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. We cannot predict the initiatives that may be adopted in the future, including repeal, replacement or significant revisions to the ACA. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare or impose price controls may adversely affect:

- the demand for our drug candidates, if we obtain regulatory approval;
- our ability to set a price that we believe is fair for our drug products;
- our ability to obtain coverage and reimbursement approval for a drug product;
- our ability to generate revenue and achieve or maintain profitability;
- the level of taxes that we are required to pay; and
- the availability of capital.

Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors, which may adversely affect our future profitability.

We expect that other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved drug product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our drug candidates. Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for biotechnology products. We cannot be sure to what extent the trajectory of these legislative and regulatory proposals will be implemented by the federal and state governments, whether additional legislative changes will be enacted, whether FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our drug candidates, if any, may be. In addition, increased scrutiny by Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

Our relationships with healthcare professionals, clinical investigators, CROs and third party payors in connection with our current and future business activities, and our participation in the federal health care programs and acceptance of federal grant funding, such as funding from the NIH, may be subject to federal and state healthcare fraud and abuse laws, false claims laws, transparency laws, government price reporting, and health information privacy and security laws, which could expose us to, among other things, criminal sanctions, civil penalties, contractual damages, exclusion from governmental healthcare programs, reputational harm, administrative burdens and diminished profits and future earnings.

Healthcare providers and third-party payors play a primary role in the recommendation and prescription of any drug candidates for which we obtain marketing approval. Our current and future arrangements with healthcare professionals, clinical investigators, CROs, third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our drug products for which we obtain marketing approval. Similarly, our participation in the federal health care programs and acceptance of federal grant funding from the NIH may subject us to federal false claims laws, civil penalties and assessments, criminal prosecution, and other administrative, civil, and criminal remedies.

The laws that may affect our ability to operate include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, either the referral of an individual, or the purchase, lease, order or recommendation of any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. Violations are subject to civil and criminal fines and penalties for each violation, plus up to three times the remuneration involved, imprisonment, and exclusion from government healthcare programs. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act(“FCA”). There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, but the exceptions and safe harbors are drawn narrowly and require strict compliance in order to offer protection.
- federal civil and criminal false claims laws, including the FCA, which can be enforced through civil “qui tam” or “whistleblower” actions, and civil monetary penalty laws, impose criminal and civil penalties against individuals or entities for, among other things, knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other federal health care programs that are false or fraudulent; knowingly making or causing a false statement material to a false or fraudulent claim or an obligation to pay money to the federal government; or knowingly concealing or knowingly and improperly avoiding or decreasing such an obligation. Manufacturers can be held liable under the FCA even when they do not submit claims directly to government payors if they are deemed to “cause” the submission of false or fraudulent claims. The FCA also permits a private individual acting as a “whistleblower” to bring actions on behalf of the federal government alleging violations of the FCA and to share in any monetary recovery. When an entity is determined to have violated the federal civil FCA, the government may impose civil fines and penalties for each false claim, plus treble damages, and exclude the entity from participation in Medicare, Medicaid and other federal healthcare programs. Under the FCA, a “claim” also includes any request (including grant request) or demand for money or property made to the United States or to a contractor, recipient, if the Federal government provides or will reimburse any portion of the funds claimed. “Funds” include money that the NIH awards as part of research grants. Even if a federal grant is not awarded, the grant applicant may be subject to FCA liability if the

information contained in or submitted as part of a grant application, including its certifications and assurances, is found to be false, fictitious, or fraudulent.

- the federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA") which created new federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity can be found guilty of violating HIPAA without actual knowledge of the statute or specific intent to violate it.
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 ("HITECH"), and their respective implementing regulations, which impose requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates and their subcontractors that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions.
- the federal Physician Payments Sunshine Act, created under the ACA and its implementing regulations, which require manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to CMS information related to payments or other transfers of value made to covered recipients, including physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain non-physician healthcare professionals (such as physician assistants and nurse practitioners, among others), and teaching hospitals, as well as information regarding ownership and investment interests held by physicians and their immediate family members.
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers.
- analogous state and foreign laws and regulations, such as state and foreign anti-kickback, false claims, consumer protection and unfair competition laws which may apply to pharmaceutical business practices, including but not limited to, research, distribution, sales and marketing arrangements as well as submitting claims involving healthcare items or services reimbursed by any third-party payor, including commercial insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government that otherwise restricts payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to file reports with states regarding pricing and marketing information, such as the tracking and reporting of gifts, compensations and other remuneration and items of value provided to healthcare professionals and entities; state and local laws requiring the registration of pharmaceutical sales representatives; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of available statutory exceptions and regulatory safe harbors, it is possible that some of our business activities, including our advisory board arrangements with physicians, some of whom receive stock or stock options as compensation for services provided, and any sales and marketing activities after a drug candidate has been approved for marketing in the United States, could be subject to legal challenge and enforcement actions. If our operations are found to be in violation of any of the federal and state laws described above or any other governmental regulations that apply to us, we may be subject to significant civil, criminal, and administrative penalties, including, without limitation, damages, fines, disgorgement, imprisonment, exclusion from participation in government healthcare programs, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

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

We are exposed to the risk that our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs, suppliers and vendors may engage in misconduct or other improper activities. Misconduct by these parties could include failures to comply with FDA regulations, provide accurate information to the FDA, comply with federal and state health care fraud and abuse laws and regulations, accurately report financial information or data or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the health care industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. 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 of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter misconduct by these parties, 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 comply with these laws or regulations. 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 penalties, including civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from participation in government funded healthcare programs, such as Medicare and Medicaid, integrity oversight and reporting obligations, contractual damages, reputational harm, diminished profits and future earnings and the curtailment or restructuring of our operations.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological and radioactive 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 our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties. Although we maintain 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. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or commercialization efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

We are subject to certain U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions, and other trade laws and regulations. We can face serious consequences for violations.

Among other matters, U.S. and foreign anti-corruption, anti-money laundering, export control, sanctions, and other trade laws and regulations, which are collectively referred to as Trade Laws, prohibit companies and their employees, agents, clinical research organizations, legal counsel, accountants, consultants, contractors, and other partners from authorizing, promising, offering, providing, soliciting, or receiving directly or indirectly, corrupt or improper payments or anything else of value to or from recipients in the public or private sector. Violations of trade laws can result in substantial criminal fines and civil penalties, imprisonment, the loss of trade privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We also expect our non-U.S. activities to increase in time. We plan to engage third parties for clinical trials or to obtain necessary permits, licenses, patent registrations, and other regulatory approvals and we can be held liable for the corrupt or other illegal activities of our personnel, agents, or partners, even if we do not explicitly authorize or have prior knowledge of such activities.

Risks Related to Our Reliance on Third Parties

We may use strategic collaborations, licensing arrangements or partnerships to accelerate the development and maximize the commercial potential of our programs, and we may not realize the benefits of such collaborations, arrangements or partnerships.

We own or in-license worldwide intellectual property rights to our drug candidates, including lasofoxifene and ATH-1105. We have in the past and, where appropriate in the future, may use strategic collaborations, licensing arrangements or partnerships to accelerate the development and maximize the commercial potential of our programs. Any of these relationships may require us to incur non-recurring and other charges, increase our near and long-term expenditures, issue securities that dilute our existing stockholders or disrupt our management and business.

We face significant competition in seeking appropriate strategic partners and the negotiation process is time-consuming and complex. Moreover, we may not be successful in our efforts to establish a strategic partnership or other alternative arrangements for our drug candidates because they may be deemed to be at too early of a stage of development for collaborative effort and third parties may not view our drug candidates as having the requisite potential to demonstrate safety and efficacy and obtain marketing approval.

If and when we seek to enter into collaborations, we may not be able to negotiate 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 a drug candidate, 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 drug candidates or bring them to market and generate product revenue.

Even if we are successful in entering into collaborations involving our drug candidates, these relationships are subject to numerous risks, which may include the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to a collaboration;
- collaborators may not pursue development and commercialization of our drug candidates or may elect not to continue or renew development or commercialization of our drug candidates based on clinical trial results, changes in their strategic focus due to the acquisition of competitive products, availability of funding or other external factors, such as a business combination that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial, stop a clinical trial, abandon a drug candidate, repeat or conduct new clinical trials or require a new formulation of a drug candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our drug candidates;
- a collaborator with marketing and distribution rights to one or more drug products may not commit sufficient resources to their marketing and distribution;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;
- disputes may arise between us and a collaborator that cause the delay or termination of the research, development or commercialization of our drug candidates, or that result in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable drug candidates; and
- collaborators may own or co-own intellectual property covering our drug products that results from our collaborating with them, and in such cases, we would not have the exclusive right to commercialize such intellectual property.

As a result, if we enter into additional strategic collaborations, licensing arrangements or partnerships, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture, which could delay our timelines or otherwise adversely affect our business. We also cannot be certain that, following a strategic collaboration, licensing arrangement or partnership, we will achieve the revenue or specific net income that justifies such transaction. Any delays in entering into new strategic collaborations, licensing arrangements or partnerships related to our drug candidates could delay the development and commercialization of our drug candidates in certain geographies for certain indications, which would harm our business prospects, financial condition and results of operations.

If we engage in future acquisitions or strategic partnerships, this may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities, and subject us to other risks.

We have in the past in-licensed product candidates, including lasofoxifene, and from time to time, we may evaluate future acquisition opportunities and strategic transactions and partnerships, including licensing or acquiring complementary products, intellectual property rights, technologies or businesses. Any acquisition, license, or strategic partnership or potential acquisition or strategic partnership entails and may entail numerous risks, including:

- increased operating expenses and cash requirements;

- the assumption of additional indebtedness or contingent liabilities;
- the issuance of our equity securities;
- assimilation of operations, intellectual property and products of an acquired company, including difficulties associated with integrating new personnel;
- the diversion of our management's attention from our existing programs and initiatives in pursuing such a strategic merger or acquisition;
- retention of key employees, the loss of key personnel and uncertainties in our ability to maintain key business relationships;
- risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their existing products or product candidates and marketing approvals; and
- our inability to generate revenue from acquired technology or products sufficient to meet our objectives in undertaking the acquisition or even to offset the associated acquisition and maintenance costs.

In connection with our in-license of lasofoxifene, we issued dilutive securities, assumed certain payment obligations, and incurred significant transaction expenses. Such obligations and expenses could increase in the future as we integrate this product candidate into our operations and as we progress their development. In addition, if we undertake acquisitions or pursue partnerships in the future, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense.

We rely on third parties to conduct our nonclinical studies and clinical trials. If these third parties do not properly and successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval of or commercialize our drug candidates.

We utilize and depend upon independent investigators and collaborators, such as medical institutions, CROs, commercial manufacturing organizations ("CMOs"), and strategic partners to conduct and support our nonclinical studies and clinical trials under agreements with us.

We expect to have to negotiate budgets and contracts with CROs, clinical trial sites and CMOs and we may not be able to do so on favorable terms, which may result in delays to our development timelines and increased costs. We will rely heavily on these third parties over the course of our nonclinical studies and clinical trials, and we control only certain aspects of their activities. As a result, we will have less direct control over the conduct, timing and completion of these nonclinical studies and clinical trials and the management of data developed through nonclinical studies and clinical trials than would be the case if we were relying entirely upon our own staff. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with applicable protocol, legal and regulatory requirements and scientific standards, and our reliance on third parties does not relieve us of our regulatory responsibilities. We and these third parties are required to comply with GCPs, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for drug candidates in clinical development. Regulatory authorities enforce these GCPs through periodic inspections of clinical trial sponsors, principal investigators and clinical trial sites. If we or any of these third parties fail to comply with applicable GCP regulations, 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. In particular, protocol deviations or non-compliance with GCP requirements, or other data integrity reasons, can cause us or the FDA or other regulatory authorities to exclude data from non-compliant sites or investigators, which may cause the trial to be underpowered to meet the endpoints. We cannot assure you that, upon inspection, such regulatory authorities will determine that any of our clinical trials comply with the GCP regulations. In addition, our clinical trials must be conducted with pharmaceutical drug product produced under cGMP regulations and

will require a large number of test patients. Our failure or any failure by these third parties to comply with these regulations or to recruit a sufficient number of patients may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, our business may be implicated if any of these third parties violates federal or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

Any third parties conducting our clinical trials are not and will not be our employees and, except for remedies available to us under our agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our ongoing, clinical and non-clinical drug candidates. These third parties may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities, which could affect their performance on our behalf. 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 or regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to complete development of, obtain regulatory approval of or successfully commercialize our drug candidates. As a result, our financial results and the commercial prospects for our drug candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed. As a result of our in-license of lasofoxifene, we are working with a number of third parties involved in the development of those product candidates with whom we do not have a prior relationship. Our new relationships with these parties are subject to the risks described above and we will need to develop a strong working relationship with these parties in the near-term to help drive the clinical development of lasofoxifene in a timely manner and we may not be successful in these efforts.

Switching or adding third parties to conduct our nonclinical studies and clinical trials involves substantial cost and requires extensive management time and focus. In addition, there is a natural transition period when a new third party commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines.

We contract with third parties for the manufacture of our drug candidates for nonclinical studies and our clinical trials, and expect to continue to do so for additional clinical trials and ultimately for commercialization. This reliance on third parties increases the risk that we will not have sufficient quantities of our drug candidates or drugs or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We do not currently have the infrastructure or internal capability to manufacture supplies of our drug candidates for use in development and commercialization. We rely, and expect to continue to rely, on third-party manufacturers for the production of our drug candidates for nonclinical studies and clinical trials under the guidance of members of our organization. We do not have long-term supply agreements. Furthermore, the raw materials for our drug candidates are sourced, in some cases, from a single-source supplier and sometimes involve long lead times from order to receipt of the materials. If we were to experience an unexpected loss of supply of any of our drug candidates or any of our future drug candidates for any reason, whether as a result of manufacturing, supply or storage issues or otherwise, we could experience delays, disruptions, suspensions or terminations of, or be required to restart or repeat, any pending or ongoing clinical trials. We expect to continue to rely on third-party manufacturers for the commercial supply of any of our drug candidates for which we obtain marketing approval. We may be unable to maintain or establish required agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- the failure of the third party to manufacture our drug candidates according to our schedule, or at all, including if our third-party contractors give greater priority to the supply of other products over our drug candidates or otherwise do not satisfactorily perform according to the terms of the agreements between us and them;
- the reduction or termination of production or deliveries by suppliers, or the raising of prices or renegotiation of terms;

- the termination or nonrenewal of arrangements or agreements by our third-party contractors at a time that is costly or inconvenient for us;
- the breach by the third-party contractors of our agreements with them;
- the failure of third-party contractors to comply with applicable regulatory requirements;
- the failure of the third party to manufacture our drug candidates according to our specifications;
- the mislabeling of clinical supplies, potentially resulting in the wrong dose amounts being supplied or active drug or placebo not being properly identified;
- clinical supplies not being delivered to clinical sites on time, leading to clinical trial interruptions, or of drug supplies not being distributed to commercial vendors in a timely manner, resulting in lost sales; and
- the misappropriation of our proprietary information, including our trade secrets and know-how.

We do not have complete control over all aspects of the manufacturing process of, and are dependent on, our contract manufacturing partners for compliance with cGMP regulations for manufacturing both active drug substances and finished drug products. Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside of the United States. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, they will not be able to secure or maintain marketing approval for their manufacturing facilities. In addition, we do not have control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our drug 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 marketing approval for or market our drug 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 fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of drug candidates or drugs, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our drug candidates or drugs and harm our business and results of operations. Our current and anticipated future dependence upon others for the manufacture of our drug candidates or drugs may adversely affect our future profit margins and our ability to commercialize any drug candidates that receive marketing approval on a timely and competitive basis.

In addition, there is currently significant uncertainty about the future relationship between the United States and various other countries, most significantly China, with respect to trade policies, treaties, tariffs, taxes, and other limitations on cross-border operations. For example, in 2025, new legislation known as the BIOSECURE Act was passed into law and imposes limits on the extension of certain specific types of government contracts or renewals, loans, or grants, to companies that may do business with select Chinese biotechnology equipment or service providers. Others in Congress have advocated for the use of existing executive branch authorities to limit certain Chinese service providers' ability to engage in business in the U.S. Although we do not currently use equipment or services produced or provided by such Chinese biotechnology companies, such changes in applicable trade policy may result in us being unable to obtain or use necessary equipment or services, may limit our ability to seek foreign regulatory approvals for our drug candidates, or may cause significant industry-wide supply delays or capacity limitations that could materially disrupt our operations, supply chain, and ability to produce, sell and distribute our drug candidates.

In a recent example of a change in policy that may impact our reliance on foreign third parties, the U.S. government instituted a new set of rules, effective April 8, 2025, that prohibit or restrict transactions involving certain types and amounts of sensitive data – including, e.g., certain genomic data, human biosamples, personal health data, etc., even when de-identified – between U.S. persons and foreign

persons associated with specific countries of concern, including China. These new rules require U.S. businesses to seek assurances from all foreign parties with which they share sensitive data (under certain types of agreements) that those parties will not further share that data with parties in countries of concern. This change in trade policy may impact our collection of data for non-clinical and clinical trials and sharing of that data and other data with foreign third parties, and may lead to the potential impacts on our operations, supply chain, and ability to produce, sell and distribute our drug candidates mentioned above.

Furthermore, the U.S. government has made and continues to make significant additional changes in U.S. trade policy and may continue to take future actions that could negatively impact U.S. trade. We cannot predict whether any proposed legislation will be enacted, what executive actions may implicate these kinds of service relationships, or what other actions may ultimately be taken with respect to trade relations between the United States and China or other countries, including countries which the U.S. government has identified as a foreign adversary that poses national security risks to the United States.

Relatedly, the United States has recently enacted and proposed to enact significant new tariffs on a number of countries. President Trump has directed various federal agencies to further evaluate key aspects of U.S. trade policy and there have been and may continue to be significant changes to U.S. trade policies, treaties and tariffs. There continues to exist significant uncertainty about the future relationship between the U.S. and other countries with respect to such trade policies, treaties and tariffs. These developments, or the perception that any of them could occur, have caused and may continue to cause significant volatility in global financial markets and may have a material adverse effect on global economic conditions, and may significantly reduce global trade and, in particular, trade between the impacted nations and the U.S. Any of these factors could depress economic activity and restrict our access to third party services. In addition, we rely on third parties located outside of the United States to source and manufacture our drug candidates. To the extent their business is impacted by global market or economic volatility, the cost and supply of our drug candidates could be materially and adversely affected.

The tariff situation remains volatile. For example, a February 20, 2026, ruling from the U.S. Supreme Court invalidated the President's recent invocation of the International Emergency Economic Powers Act ("IEEPA") to authorize certain recent tariff actions, and these tariffs were rescinded on February 24, 2026. Details regarding the availability, timing, and amount of any refunds remains uncertain and subject to further litigation and legal, regulatory, and administrative actions. The U.S. government subsequently announced plans to implement a new global "temporary import surcharge" of 15% on many of the same imported products beginning February 24, 2026, under authorities provided for in Section 122 of the Trade Act of 1974, supplementing non-IEEPA tariff measures. Further tariffs may also be forthcoming following the initiation and completion of additional Section 301 tariff investigations. We cannot predict what products and services may be subject to such actions or what actions may be taken by the other countries in retaliation, any of which may materially and adversely affect our business, financial condition, results of operations, and cash flows.

Our manufacturing process needs to comply with FDA regulations relating to the quality and reliability of such processes. Any failure to comply with relevant regulations could result in delays in or termination of our clinical programs and suspension or withdrawal of any regulatory approvals.

In order to commercially produce our drug products either at our own facility or at a third party's facility, we will need to comply with the FDA's cGMP regulations and guidelines. We may encounter difficulties in achieving quality control and quality assurance and may experience shortages in qualified personnel. We are subject to inspections by the FDA and comparable foreign regulatory authorities to confirm compliance with applicable regulatory requirements. Any failure to follow cGMP or other regulatory requirements or delay, interruption or other issues that arise in the manufacture, fill-finish, packaging, or storage of our precision medicines as a result of a failure of our facilities or the facilities or operations of third parties to comply with regulatory requirements or pass any regulatory authority inspection could significantly impair our ability to develop and commercialize our drug candidates, including leading to significant delays in the availability of our precision medicines for our clinical trials or

the termination of or suspension of a clinical trial, or the delay or prevention of a filing or approval of marketing applications for our drug candidates. Significant non-compliance could also result in the imposition of sanctions, including warning or untitled letters, fines, injunctions, civil penalties, failure of regulatory authorities to grant marketing approvals for our drug candidates, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of products, operating restrictions and criminal prosecutions, any of which could damage our reputation and our business.

If our third-party manufacturers use hazardous materials in a manner that causes injury or violates applicable law, we may be liable for damages.

Our research and development activities involve the controlled use of potentially hazardous substances, including chemical materials, by our third-party manufacturers. Our manufacturers are subject to federal, state and local laws and regulations in the United States governing the use, manufacture, storage, handling and disposal of medical and hazardous materials. Although we believe that our manufacturers' procedures for using, handling, storing and disposing of these materials comply with legally prescribed standards, we cannot completely eliminate the risk of contamination or injury resulting from medical or hazardous materials. As a result of any such contamination or injury, we may incur liability or local, city, state or federal authorities may curtail the use of these materials and interrupt our business operations. In the event of an accident, we could be held liable for damages or penalized with fines, and the liability could exceed our resources. We do not have any insurance for liabilities arising from medical or hazardous materials. Compliance with applicable environmental laws and regulations is expensive, and current or future environmental regulations may impair our research, development and production efforts, which could harm our business, prospects, financial condition or results of operations.

Risks Related to Our Ability to Commercialize our Drug Products

Even if approved, our drug candidates may not achieve adequate market acceptance among physicians, patients, healthcare payors and others in the medical community necessary for commercial success.

Even if our drug candidates receive regulatory approval, they may not gain adequate market acceptance among physicians, patients, healthcare payors and others in the medical community. The degree of market acceptance or reimbursement of any of our approved drug candidates will depend on a number of factors, including:

- the efficacy and safety profile as demonstrated in clinical trials compared to alternative treatments;
- the timing of market introduction of the drug candidate as well as competitive products;
- the clinical indications for which the drug candidate is approved;
- the extent of physician acceptance of FDA-approved therapies for target indications;
- restrictions on the use of our drug candidates, such as boxed warnings or contraindications in labeling, or a REMS, if any, which may not be required of alternative treatments and competitor products;
- the potential and perceived advantages of our drug candidates over alternative treatments;
- the cost of treatment in relation to alternative treatments;
- pricing and the availability of coverage and adequate reimbursement by third-party payors, including government authorities;
- the availability of the approved drug candidate for use as a combination therapy;
- relative convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;

- the effectiveness of sales and marketing efforts;
- unfavorable publicity relating to our drug products or drug candidates or similar approved products or product candidates in development by third parties; and
- the approval of other new therapies for the same indications.

If any of our drug candidates are approved but do not achieve an adequate level of acceptance by physicians, hospitals, healthcare payors and patients, we may not generate or derive sufficient revenue from such drug candidates and our financial results could be negatively impacted.

We have never commercialized a drug candidate before and may lack the necessary expertise, personnel and resources to successfully commercialize any drug products on our own or together with suitable collaborators.

We have never commercialized a drug candidate. We may license certain rights with respect to our drug candidates to collaborators, and, if so, we will rely on the assistance and guidance of those collaborators. For drug candidates for which we retain commercialization rights and marketing approval, we will have to develop our own sales, marketing and supply organization or outsource these activities to a third party.

Factors that may affect our ability to commercialize our drug candidates, if approved, on our own include recruiting and retaining adequate numbers of effective sales and marketing personnel, developing adequate educational and marketing programs to increase public acceptance of our approved drug candidates, ensuring regulatory compliance of our company, employees and third parties under applicable healthcare laws, and other unforeseen costs associated with creating an independent sales and marketing organization. Developing a sales and marketing organization will be expensive and time-consuming and could delay the launch of our drug candidates upon approval. We may not be able to build an effective sales and marketing organization. If we are unable to build our own distribution and marketing capabilities or to find suitable partners for the commercialization of our drug candidates, we may not generate revenues from them or be able to reach or sustain profitability.

If the market opportunity for any drug candidate that we develop is smaller than we believe, our revenue may be adversely affected and our business may suffer.

Certain of our drug candidates (currently ATH-1105) are being developed as treatments for various CNS and PNS disorder indications, and lasofoxifene is being developed as a treatment for breast cancer. The addressable patient populations that may benefit from treatment with our drug candidates, if approved, are based on our estimates. These estimates, which have been derived from a variety of sources, including scientific literature, surveys of clinics, patient foundations and market research, may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of the relevant disorders. Any regulatory approval of our drug candidates would be limited to the therapeutic indications examined in our clinical trials and as determined by the FDA, which would not permit us to market our drug products for any other therapeutic indications not expressly approved by the FDA. Additionally, the potentially addressable patient population for our drug candidates may not ultimately be amenable to treatment with our drug candidates. Even if we receive regulatory approval for any of our drug candidates, such approval could be conditioned upon label restrictions that materially limit the addressable patient population. Our market opportunity may also be limited by future competitor treatments that enter the market. If any of our estimates prove to be inaccurate, the market opportunity for any drug candidate that we or our strategic partners develop could be significantly diminished and have an adverse material impact on our business.

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our drug candidates.

We face an inherent risk of product liability as a result of the planned clinical testing of our drug candidates and will face an even greater risk if we commercialize any drug products. For example, we may be sued if our drug candidates cause or are perceived to cause injury or are found to be otherwise unsuitable during clinical testing, manufacturing, marketing or sale. 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, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our drug candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our drug candidates or drug products that we may develop;
- injury to our reputation;
- withdrawal of clinical trial participants;
- initiation of investigations by regulators;
- costs to defend the related litigation;
- diversion of management's time and our resources;
- substantial monetary awards to clinical trial participants or patients;
- drug product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of revenue;
- exhaustion of any available insurance and our capital resources;
- the inability to commercialize any drug candidate; and
- a decline in our share price.

Failure to obtain or retain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of drug products we develop, alone or with corporate collaborators. Although we have clinical trial insurance, our insurance policies also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts. Even if our agreements with any future corporate collaborators entitle us to indemnification against losses, such indemnification may not be available or adequate should any claim arise.

Patients with cancer and other diseases targeted by lasofoxifene and our product candidates are often already in severe and advanced stages of disease and have both known and unknown significant pre-existing and potentially life-threatening health risks. During the course of treatment, patients may suffer adverse events, including death, for reasons that may be related to lasofoxifene or our product candidates. Such events could subject us to costly litigation, require us to pay substantial amounts of money to injured patients, delay, negatively impact or end the opportunity to receive or maintain regulatory approval to market lasofoxifene or our product candidates, or require us or our strategic partners to suspend or abandon commercialization efforts. Even in circumstances in which we do not believe that an adverse event is related to lasofoxifene or our product candidates, the investigation into the circumstance may be time-consuming or inconclusive, and may result in reputational harm. These investigations may interrupt our sales efforts, delay our regulatory approval process in other countries, or impact and limit the type of regulatory approvals lasofoxifene or our product candidates receive or

maintain. As a result of these factors, a product liability claim, even if successfully defended, could have a material adverse effect on our business, financial condition or results of operations.

The insurance coverage and reimbursement status of newly approved products is uncertain. Our drug candidates may become subject to unfavorable pricing regulations, third-party coverage and reimbursement practices, or healthcare reform initiatives, which would harm our business. Failure to obtain or maintain adequate coverage and reimbursement for new or current drug products could limit our ability to market those drug products and decrease our ability to generate revenue.

The regulations that govern marketing approvals, pricing, coverage and reimbursement for new drugs vary widely from country to country. In the United States, recently enacted 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 drug product in a particular country, but then be subject to price regulations that delay our commercial launch of the drug product, possibly for lengthy time periods, and negatively impact the revenue we are able to generate from the sale of the drug product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more drug candidates, even if any drug candidates we may develop obtain marketing approval.

In the United States 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. Our ability to successfully commercialize our drug candidates will depend in part on the extent to which coverage and adequate reimbursement for these drug products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. The availability of coverage and extent of reimbursement by governmental and private payors is essential for most patients to be able to afford treatments such as gene therapy products. Sales of these or other future drug candidates that we may identify will depend substantially, both domestically and abroad, on the extent to which the costs of our drug candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, private health coverage insurers and other third-party payors. If coverage and adequate reimbursement is not available, or is available only to limited levels, we may not be able to successfully commercialize our drug candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment. Reimbursement by a third-party payor may depend upon a number of factors, including, but not limited to, 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.

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. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines

under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medicines, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our drug candidates. Accordingly, in markets outside the United States, the reimbursement for products may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenue and profits.

There is also significant uncertainty related to the insurance coverage and reimbursement of newly approved products and coverage may be more limited than the purposes for which the medicine is approved by the FDA or comparable foreign regulatory authorities. In the United States, the principal decisions about reimbursement for new medicines are typically made by CMS, an agency within HHS. CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare and private payors tend to follow CMS to a substantial degree. No uniform policy of coverage and reimbursement for products exists among third-party payors and coverage and reimbursement levels for products can differ significantly from payor to payor. As a result, the coverage determination process is often a time consuming and costly process that may require us to provide scientific and clinical support for the use of our drug products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. It is difficult to predict what CMS will decide with respect to reimbursement for fundamentally novel products such as ours. Reimbursement agencies in Europe may be more conservative than CMS. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale, and distribution. 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. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for any approved drug products we may develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize drug candidates, and our overall financial condition.

Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or 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 United States. Our inability to promptly obtain coverage and profitable reimbursement rates third-party payors for any approved drug products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize drug products and our overall financial condition.

Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any drug candidate that we commercialize and, if reimbursement is available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any drug candidate for which we obtain marketing approval. In order to obtain reimbursement, physicians may need to show that patients have superior treatment outcomes with our drug products compared to standard of care drugs, including lower-priced generic versions of standard of care drugs. We expect to experience pricing pressures in connection with the sale of any of our drug candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products.

A variety of risks associated with marketing our drug candidates internationally may materially adversely affect our business.

We plan to eventually seek regulatory approval of our drug candidates outside of the United States and, accordingly, we expect that we will be subject to additional risks related to operating in foreign countries if we obtain the necessary approvals, including:

- differing regulatory requirements in foreign countries, such as the lack of pathways for accelerated drug approval, may result in foreign regulatory approvals taking longer and being more costly than obtaining approval in the United States;
- foreign regulatory authorities may disagree with the design, implementation or results of our clinical trials or our interpretation of data from nonclinical studies or clinical trials;
- approval policies or regulations of foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval;
- impact of health epidemics on our ability to produce our drug candidates and conduct clinical trials in foreign countries;
- unexpected changes in U.S. or non-U.S. tariffs, trade barriers, price and exchange controls and other regulatory requirements, including any changes that nations may impose as a result of political tensions;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with legal requirements applicable to privacy, data protection, information security and other matters;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- complexities associated with managing multiple payor reimbursement regimes and government payors in foreign countries;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- potential liability under the Foreign Corrupt Practices Act of 1977 or comparable foreign regulations;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geo-political actions, including war and terrorism.

These and other risks associated with international operations may materially adversely affect our ability to attain or maintain profitable operations.

Risks Related to Our Intellectual Property

Our success depends on our ability to protect our intellectual property and our proprietary technologies.

Our commercial success depends in part on our ability to obtain and maintain patent protection and trade secret protection for our drug candidates, proprietary technologies and their uses as well as our ability to operate without infringing upon the proprietary rights of others. We generally seek to protect our proprietary position by filing patent applications in the United States and abroad related to our drug candidates, proprietary technologies and their uses that are important to our business. We may also seek to protect our proprietary position by acquiring or in-licensing relevant issued patents or pending applications from third parties.

Pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless, and until, patents issue from such applications, and then only to the extent the issued claims cover the technology. There can be no assurance that our patent applications or the patent applications of any current or future licensors will result in additional patents being issued or that issued patents will afford sufficient protection against competitors with similar technology, nor can there be any assurance that the patents issued will not be infringed, designed around, found unenforceable or invalidated by third parties.

Even issued patents may later be found invalid or unenforceable or may be modified or revoked in proceedings instituted by third parties or the patent owner before various patent offices or in courts. Thus, the degree of future protection for our and any current or future licensors' proprietary rights is uncertain. Only limited protection may be available and may not adequately protect our rights or permit us to gain or keep any competitive advantage. These uncertainties or limitations in our ability to properly protect the intellectual property rights relating to our drug candidates could have a material adverse effect on our financial condition and results of operations.

We cannot be certain that the claims in our pending patent applications or those of any current or future licensors will be considered patentable by the United States Patent and Trademark Office courts in the United States or by the patent offices and courts in foreign countries, nor can we be certain that the claims in our owned or in-licensed patents will not be found invalid or unenforceable if challenged.

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or any of our potential future collaborators will be successful in protecting our drug candidates by obtaining and defending patents. These risks and uncertainties include the following:

- the USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process, the noncompliance with which can result in abandonment or lapse of a patent or patent application, and partial or complete loss of patent rights in the relevant jurisdiction;
- patent applications may not result in any patents being issued;
- patents and patent applications may be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable or otherwise may not provide any competitive advantage;
- changes to patent laws in the United States or in other countries may limit the ability to obtain, defend or enforce patents, or may apply retroactively to affect the term or scope of patents;
- our competitors, many of whom have substantially greater resources than we do and many of whom have made significant investments in competing technologies, may seek or may have already obtained patents that will limit, interfere with or eliminate our ability to make, use and sell our potential drug candidates;

- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope or term of patent protection both inside and outside the United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns; and
- countries other than the United States may have patent laws less favorable to patentees than those upheld by U.S. courts, allowing foreign competitors a better opportunity to create, develop and market competing product candidates.

The patent prosecution process is also expensive and time-consuming, and we and any current or future licensors may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner or in all jurisdictions where protection may be commercially advantageous. It is also possible that we or any current or future licensors will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection.

In addition, although we enter into non-disclosure and confidentiality agreements with parties who have access to patentable aspects of our research and development output, such as our employees, outside scientific collaborators, CROs, third-party manufacturers, consultants, advisors and other third parties, any of these parties may breach such agreements and disclose such output before a patent application is filed. Certain of these parties may also be subject to public information disclosure statutes and could determine to disclose patentable aspects of our research and development output pursuant to a request thereunder, notwithstanding the existence of a non-disclosure and confidentiality agreement. Any of these actions could jeopardize our ability to seek patent protection.

Given the amount of time required for the development, testing and regulatory review of new drug candidates, patents protecting such candidates or their use might expire before or shortly after such candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

If the scope of any patent protection we obtain is not sufficiently broad or the term is not sufficiently long, or if we lose any of our patent protection, our ability to prevent our competitors from commercializing similar or identical product candidates would be adversely affected.

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

Moreover, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Even if patent applications we own or in-license currently or in the future issues as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us, or otherwise provide us with any competitive advantage. Any patents that we own or in-license may be challenged or circumvented by third parties or may be narrowed, invalidated or rendered unenforceable as a result of challenges by third parties. Consequently, we do not know whether our drug candidates will be protectable or remain protected by valid and enforceable patents. Our competitors or other third parties may be able to circumvent our patents or the patents of any current or future licensors by developing similar or alternative technologies or products in a non-infringing manner which could materially adversely affect our business, financial condition, results of operations and prospects.

The issuance of a patent is not conclusive as to its inventorship, scope, term, validity, or enforceability, and our patents or the patents of any current or future licensors may be challenged in the courts or patent offices in the United States and abroad. We may be subject to a third-party pre-issuance submission of prior art to the USPTO, or become involved in opposition, derivation, revocation,

reexamination, post-grant review("PGR"), and inter partes review("IPR"), or other similar proceedings challenging our patent rights. An adverse determination in any such submission, proceeding or litigation could reduce the scope or term of, or invalidate or render unenforceable, our patent rights, allow third parties to commercialize our drug candidates and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights.

Moreover, our patents or the patents of any current or future licensors may become subject to post-grant challenge proceedings, such as oppositions in a foreign patent office, that challenge our claim of priority of invention, scope, validity or patentability with respect to our patents and patent applications and those of any current or future licensors.

For example, third parties may challenge the validity or enforceability of our current or future in-licensed patents and patent applications. The outcome following legal assertions of invalidity and unenforceability is unpredictable. Such challenges may result in loss of patent rights, loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar technology and drug products not covered by our issued patents. Such proceedings also may result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us. In addition, if the breadth or strength of protection provided by our current or future in-licensed patents and patent applications is threatened, regardless of the outcome, it could dissuade companies from collaborating with us to license, develop, or commercialize current or future drug candidates. Further, these proceedings could have a material adverse effect on our business, results of operations and financial condition.

Even though we own patents and patent applications covering our small molecule drug candidates, our patents and any future patents we obtain may not effectively prevent others from developing or commercializing products similar to our drug candidates. Third parties may seek to cast doubt on the validity and enforceability of our owned patents or patent applications. Such events may result in substantial cost and require significant time from our scientists and management, and could dissuade companies from collaborating with us to license, develop, or commercialize current or future drug candidates, even if the eventual outcome is favorable to us.

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. For example:

- others may be able to develop products that are similar to our drug candidates but that are not covered by the claims of the patents that we own or license;
- we or any current or future licensors or collaborators might not have been the first to make the inventions covered by the issued patents or patent application that we own or license;
- we or any current or future licensors or collaborators might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that the pending patent applications we own or license will not lead to issued patents;
- issued patents that we own or license may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- 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;

- we may not develop additional proprietary technologies that are patentable;
- the patents of others may have an adverse effect on our business; and
- we may choose not to file a patent application in order to maintain certain trade secrets or know-how, and a third party may subsequently file a patent application covering such intellectual property.

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

Our commercial success depends significantly on our ability to operate without infringing the patents and other proprietary rights of third parties. Claims by third parties that we infringe their proprietary rights may result in liability for damages or prevent or delay our developmental and commercialization efforts.

Our commercial success depends in part on avoiding infringement of the patents and proprietary rights of third parties. However, our research, development and commercialization activities may be subject to claims that we infringe or otherwise violate patents or other intellectual property rights owned or controlled by third parties. Other entities may have or obtain patents or proprietary rights that could limit our ability to make, use, sell, offer for sale or import our drug candidates and drug products that may be approved in the future, or impair our competitive position. There is a substantial amount of litigation and other legal actions, both within and outside the United States, involving patent and other intellectual property rights in the biopharmaceutical industry, including patent infringement lawsuits, reexaminations, IPR proceedings and PGR proceedings and oppositions before the USPTO and/or corresponding foreign patent offices. Numerous third-party U.S. and foreign issued patents and pending patent applications exist in the fields in which we are developing drug candidates. There may be third-party patents or patent applications with claims to compositions, materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our drug candidates.

As the biopharmaceutical industry expands and more patents are issued, the risk increases that our drug candidates may be subject to claims of infringement of the patent rights of third parties. Because patent applications are maintained as confidential for a certain period of time, until the relevant application is published, we may be unaware of third-party patents or patent applications that may be infringed by commercialization of any of our drug candidates, and we cannot be certain that we were the first to file a patent application related to a drug candidate, its use, or our technology. Moreover, because patent applications can take many years to issue, there may be currently-pending patent applications that may later result in issued patents that our drug candidates or their use may infringe. In addition, identification of third-party patent rights that may be relevant to our technology is difficult because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. There is also no assurance that there is not prior art of which we are aware, but which we do not believe is relevant to our business, which may, nonetheless, ultimately be found to limit our ability to make, use, sell, offer for sale or import our drug candidates that may be approved in the future, or impair our competitive position. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. Any defense to claims of patent infringement asserted by third parties would be time consuming and could:

- result in costly litigation that may cause negative publicity;
- divert the time and attention of our technical personnel and management;
- cause development delays;
- prevent us from commercializing any of our drug candidates until the asserted patent expires or is held finally invalid or not infringed in a court of law;
- require us to develop non-infringing technology, which may not be possible on a cost-effective basis;

- subject us to significant liability to third parties; or
- require us to enter into royalty or licensing agreements, which may not be available on commercially reasonable terms, or at all, or which might be non-exclusive, which could result in our competitors gaining access to the same technology.

Although no third party has asserted a claim of patent infringement against us as of the date of this report, others may hold proprietary rights that could prevent our drug candidates from being marketed. Any patent-related legal action against us claiming damages and seeking to enjoin commercial activities relating to our drug candidates, treatment indications, or processes could subject us to significant liability for damages, including treble damages if we were determined to willfully infringe, and require us to obtain a license to manufacture or market our drug candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion for management and other personnel. We cannot predict whether we would prevail in any such actions or that any license required under any of these patents would be made available on commercially acceptable terms, if at all. Moreover, even if we or a future strategic partner were able to obtain a license, the rights may be nonexclusive, which could result in our competitors gaining access to the same intellectual property. In addition, we cannot be certain that we could redesign our drug candidates, our treatment indications, or processes to avoid infringement, if necessary. Accordingly, an adverse determination in a judicial or administrative proceeding, or the failure to obtain necessary licenses, could prevent us from developing and commercializing our drug candidates, which could harm our business, financial condition and operating results. In addition, intellectual property litigation, regardless of its outcome, may cause negative publicity and could prohibit us from marketing or otherwise commercializing our drug candidates and technology.

Parties making claims against us may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or administrative proceedings, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

We may not be successful in obtaining or maintaining necessary rights to our drug candidates through acquisitions and in-licenses.

Because our development programs may in the future require the use of proprietary rights held by third parties, the growth of our business may depend in part on our ability to acquire, in-license, or use these third-party proprietary rights. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify as necessary for our drug candidates. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of 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. 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, or if we are unable to maintain the existing intellectual property rights we have, we may have to abandon development of the relevant program or drug candidate, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

We may be involved in lawsuits to protect or enforce our patents or any current or future licensors' patents, which could be expensive, time consuming and unsuccessful. Further, our issued patents or any current or future licensors' patents could be found invalid or unenforceable if challenged in court.

Competitors may infringe our intellectual property rights. To prevent infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in a patent infringement proceeding, a court may decide that a patent we own or in-license is not valid, is unenforceable, or is not infringed. If we or any of our potential future collaborators were to initiate legal proceedings against a third party to enforce a patent directed at one of our drug candidates or their method of use, the defendant could counterclaim that our patent or the patent of any current or future licensors is invalid or unenforceable in whole or in part. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge include an alleged failure to meet any of several statutory requirements, including allegations of a lack of novelty, obviousness, lack of sufficient written description, non-enablement, or obviousness-type double patenting. Grounds for an unenforceability assertion could include an allegation that someone connected with prosecution of the patent application misrepresented or fraudulently withheld relevant information from the USPTO or made a misleading statement during prosecution.

Third parties may also raise similar invalidity claims before the USPTO or patent offices abroad, even outside the context of litigation. Such mechanisms include re-examination, PGR, IPR, derivation proceedings, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in the revocation of, cancellation of, or amendment to our patents or any current or future licensors' patents in such a way that they no longer cover our technology or any drug candidates that we may develop. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to a validity claim, for example, we cannot be certain that there is no invalidating prior art of which we and the patent examiner were unaware during prosecution. If a third party were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our technology or any drug candidates that we may develop. Such a loss of patent protection would have a material adverse impact on our business, financial condition, results of operations and prospects.

The outcome following legal assertions of invalidity or unenforceability is unpredictable, and prior art could render our patents or any current or future licensors' patents invalid. There is no assurance that all potentially relevant prior art relating to our patents and patents applications or the patents and patent applications of any current or future licensors has been found. There is also no assurance that there is not prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim in our patents and patent applications or the patents and patent applications of any current or future licensors, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. Additionally, a finding that issued claims lack sufficient written description or are not enabled could render our patent or any current or future licensors' patent invalid. A finding that issued claims are obvious under the standard for obviousness-type double patenting could result in a shortened term for our patent or any current or future licensors' patent, or render our patent or any current or future licensors' patent invalid.

If a third party were to prevail on a legal assertion of invalidity or unenforceability, we may lose at least part, and perhaps all, of the patent protection on such drug candidate. In addition, if the breadth or strength of protection provided by our patents and patent applications or the patents and patent applications of any current or future licensors is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future drug candidates. Such a loss of patent protection would have a material adverse impact on our business.

Even if resolved in our favor, litigation or other legal proceedings relating to our intellectual property rights may cause us to incur significant expenses, and could distract our management and other personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of

our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or other legal proceedings relating to our intellectual property rights, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation or other proceedings. 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.

In addition, the issuance of a patent does not give us the right to practice the patented invention. Third parties may have blocking patents that could prevent us from marketing our own patented drug product and practicing our own patented technology.

Intellectual property litigation or legal proceedings may lead to unfavorable publicity that harms our reputation and causes the market price of our common stock to decline.

During the course of any intellectual property litigation or legal proceeding, there could be public announcements of the initiation of the litigation or legal proceeding as well as results of hearings, rulings on motions, and other interim proceedings. If securities analysts or investors regard these announcements as negative, the perceived value of our existing drug candidates, programs or intellectual property could be diminished. Accordingly, the market price of shares of our common stock may decline. Such announcements could also harm our reputation or the market for our future drug products, which could have a material adverse effect on our business.

Derivation proceedings may be necessary to determine priority of inventions, and an unfavorable outcome may require us to cease using the related technology or to attempt to license rights from the prevailing party.

Derivation proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of any current or future licensors or of third parties. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of derivation proceedings may fail and, even if successful, may result in substantial costs and distract our management and other personnel. In addition, the uncertainties associated with such proceedings could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties or enter into development or manufacturing partnerships that would help us bring our drug candidates to market.

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications or those of any current or future licensors and the enforcement or defense of our issued patents or those of any current or future licensors.

On September 16, 2011, the Leahy-Smith America Invents Act (the "Leahy-Smith Act") was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted and may also affect patent litigation. In particular, under the Leahy-Smith Act, the United States transitioned in March 2013 to a “first inventor to file” system in which, assuming that other requirements of patentability are met, the first inventor to file a patent application will be entitled to the patent regardless of whether a third party was first to invent the

claimed invention. A third party that filed a patent application in the USPTO after March 2013 but before us could therefore be awarded a patent covering an invention of ours or our current or future licensors even if we or our current or future licensors had made the invention before it was made by such third party. This requires us to be cognizant going forward of the time from invention to filing of a patent application. Furthermore, our ability to obtain and maintain valid and enforceable patents depends on whether the differences between our technology and the prior art allow our technology to be patentable over the prior art. Since patent applications in the United States and most other countries are confidential for a period of time after filing or until issuance, we may not be certain that we or any current or future licensors are the first to either (1) file any patent application related to our drug candidates, their use, or our technology or (2) invent any of the inventions claimed in the patents or patent applications.

The Leahy-Smith Act also included a number of significant changes that affect the way patent applications are prosecuted and also may affect patent litigation. These include allowing third-party submission of printed publications to the USPTO during patent prosecution and additional procedures to attack the validity or enforceability of a patent by USPTO-administered post-grant proceedings, including PGR, IPR, and derivation proceedings. An adverse determination in any such submission or proceeding could reduce the scope or enforceability of, or invalidate, our patent rights, which could adversely affect our competitive position.

Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in United States federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims or those of our current or future licensors that would not have been invalidated if first challenged by the third party in a district court action. Thus, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications or those of any current or future licensors and the enforcement or defense of our issued patents or those of any current or future licensors, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Changes in U.S. patent law, or laws in other countries, could diminish the value of patents in general, thereby impairing our ability to protect our drug candidates.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve a high degree of technological and legal complexity. Therefore, obtaining and enforcing biopharmaceutical patents is costly, time consuming and inherently uncertain. Changes in either the patent laws or in the interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property and may increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. We cannot predict the breadth of claims that may be allowed or enforced in our patents or in third-party patents. In addition, Congress or other foreign legislative bodies may pass patent reform legislation that is unfavorable to us.

For example, the U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our 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 decisions by the U.S. Congress, the U.S. federal courts, the USPTO, or similar authorities in foreign jurisdictions, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patent and the patents we might obtain or license in the future. As an example, European patent applications now provide the option, upon grant of a patent, of becoming a Unitary Patent, which is subject to the jurisdiction of the Unitary Patent Court (“UPC”), in member states that have acceded to and ratified the EU Patent Package. The option of a Unitary Patent is a significant change in European patent practice. As the UPC is a new court system, there is no precedent for the court, increasing the uncertainty

of any litigation in the UPC. European patents granted on applications we file may be subject to loss or revocation via the UPC, which could have a material adverse effect on our business and our ability to commercialize or license our technology and products. Moreover, the controlling laws and regulations of the UPC will develop over time and may adversely affect our ability to enforce or defend the validity of any European patents obtained. We have opted out of the UPC with respect to our European patents to date, and we may decide to opt out of the UPC with respect to any pending or future published European patent applications or patents. If certain formalities and requirements are not met, however, such European patents and patent applications could be challenged for non-compliance and brought under the jurisdiction of the UPC. We cannot be certain that future European patents and patent applications will avoid falling under the jurisdiction of the UPC, even if we decide to opt out of the UPC.

We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may also be subject to claims that former employees or other third parties have an ownership interest in our patents or other intellectual property. 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 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 distraction to management and other employees.

Patent terms may be inadequate to protect our competitive position on our drug candidates for an adequate amount of time.

Patents have a limited lifespan. In the United States, 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 patents covering our drug candidates or their use are obtained, once the patent life has expired, we may be open to competition from competitive products, including generic versions of our drug products. Given the amount of time required for the development, testing and regulatory review of new drug candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing drug products similar or identical to ours.

If we do not obtain patent term extension for our drug candidates, our business may be materially harmed.

Depending upon the timing, duration and specifics of FDA marketing approval of our drug candidates, one or more of our U.S. patents or those of any current or future licensors may be eligible for limited patent term restoration under the Drug Price Competition and Patent Term Restoration Act of 1984 (“Hatch-Waxman Amendments”). The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. A maximum of one patent may be extended per FDA approved product as compensation for the patent term lost during clinical trials 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 and only those claims covering such approved drug product, a method for using it or a method for manufacturing it may be extended. Patent term extension may also be available in certain foreign countries upon regulatory approval of our drug candidates, although the requirements and terms of such extensions vary country-by-country. However, we may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration or the term of any such extension is less than we request, our competitors may obtain approval of competing products or launch generic versions of our drug products following our patent expiration, and our revenue could be reduced, possibly materially. Further, if this

occurs, our competitors may take advantage of our investment in development and clinical trials by referencing our clinical and nonclinical data and launch their drug product earlier than might otherwise be the case.

We will not be able to protect our intellectual property rights throughout the world.

We own and in-license patents and pending patent applications in the United States and in jurisdictions outside of the United States. However, filing, prosecuting and defending patents in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States may be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we will not be able to prevent third parties from practicing our inventions in all countries outside the United States or from selling or importing drug products made using our inventions in and into the United States or other jurisdictions. Competitors may use our inventions in jurisdictions where we have not obtained patent protection to develop their own drug products and, further, may export otherwise infringing drug products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These drug products may compete with our drug candidates, and our patents, the patents of any current or future licensors, or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of many foreign countries do not favor the enforcement of patents and other intellectual property protection, which could make it difficult for us to stop the infringement of our patents or any current or future licensors' patents or marketing of competing drug products in violation of our proprietary rights. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents or the patents of any current or future licensors at risk of being invalidated or interpreted narrowly and our patent applications or the patent applications of any current or future licensors at risk of not issuing 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.

Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these 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, and our business, financial condition, results of operations and prospects may be adversely affected.

Geopolitical actions could increase the uncertainties and costs surrounding the prosecution or maintenance of our patent applications or those of any current or future licensors and the maintenance, enforcement or defense of our issued patents or those of any current or future licensors. For example, the United States and foreign government actions related to Russia's invasion of Ukraine may limit or prevent filing, prosecution and maintenance of patent applications in Russia. Government actions may also prevent maintenance of issued patents in Russia. These actions could result in abandonment or lapse of our patents or patent applications, resulting in partial or complete loss of patent rights in Russia. If such an event were to occur, it could have a material adverse effect on our business. In addition, a decree was adopted by the Russian government in March 2022, allowing Russian companies and individuals to exploit inventions owned by patentees that have citizenship or nationality in, are registered in, or have a predominately primary place of business or profit-making activities in the United States and other countries that Russia has deemed unfriendly without consent or compensation. Consequently, we would not be able to prevent third parties from practicing our inventions in Russia or from selling or importing products made using our inventions in and into Russia. Accordingly, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

Obtaining and maintaining our patent protection depends on compliance with various procedural, documentary, fee payment, and other requirements imposed by regulations and governmental 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 governmental fees on patents or applications will be due to the USPTO and various foreign patent offices at various points over the lifetime of our patents or applications and those of any current or future licensors. We have systems in place to remind us to pay these fees, and we rely on our outside patent annuity service to pay these fees when due. Additionally, the USPTO and various foreign patent offices require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply, and in many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with rules applicable to the particular jurisdiction. However, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. If such an event were to occur, it could have a material adverse effect on our business.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

We intend to use registered or unregistered trademarks or trade names to brand and market ourselves and our drug products. Our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest, and it may be difficult and costly to register, maintain or protect our rights to these trademarks and trade names in jurisdictions in and outside of the United States. 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. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to 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, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely affect our financial condition or results of operations.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition, we rely on the protection of our trade secrets, including unpatented know-how, technology and other proprietary information to maintain our competitive position. Although we have taken steps to protect our trade secrets and unpatented know-how, including entering into confidentiality agreements with third parties, and confidential information and inventions agreements with our employees, consultants and advisors, we cannot provide any assurances that all such agreements have been duly executed, and any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party improperly disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets.

Moreover, third parties may still obtain this information or may come upon this or similar information independently, and we would have no right to prevent them from using that technology or information to compete with us. If any of these events occurs or if we otherwise lose protection for our trade secrets, the value of this information may be greatly reduced, and our competitive position would be harmed. If we do

not apply for patent protection prior to such publication or if we cannot otherwise maintain the confidentiality of our proprietary technology and other confidential information, then our ability to obtain patent protection or to protect our trade secret information may be jeopardized.

We may be subject to claims that we or our employees have wrongfully used or disclosed alleged confidential information or trade secrets of third parties.

We have entered into and may enter in the future into non-disclosure and confidentiality agreements to protect the proprietary positions of third parties, such as outside scientific collaborators, CROs, third-party manufacturers, consultants, advisors, potential partners, lessees of shared multi-company property and other third parties. We may become subject to litigation where a third party asserts that we or our employees inadvertently or otherwise breached the agreements and used or disclosed their trade secrets or other information proprietary to the third parties. Defense of such matters, regardless of their merit, could involve substantial litigation expense and be a substantial diversion of employee resources from our business. We cannot predict whether we would prevail in any such actions. Moreover, intellectual property litigation, regardless of its outcome, may cause negative publicity and could prohibit us from marketing or otherwise commercializing our drug candidates. Failure to defend against any such claim could subject us to significant liability for monetary damages or prevent or delay our developmental and commercialization efforts, which could adversely affect our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to our management team and other employees.

Parties making claims against us may be able to sustain the costs of complex intellectual property litigation more effectively than we can because they have substantially greater resources. 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. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, operating results, financial condition and prospects.

We may be subject to claims that we have wrongfully hired an employee from a competitor or that we or our employees have wrongfully used or disclosed alleged confidential information or trade secrets of their former employers.

As is common in the biopharmaceutical industry, in addition to our employees, we engage the services of consultants to assist us in the development of our drug candidates. Many of these consultants, and many of our employees, were previously employed at, or may have previously provided or may be currently providing consulting services to, other biopharmaceutical companies including our competitors or potential competitors. We may become subject to claims that we, our employees or a consultant inadvertently or otherwise used or disclosed trade secrets or other information proprietary to their former employers or their former or current clients. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely affect our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to our management team and other employees.

Our rights to develop and commercialize our drug candidates may be subject, in part, to the terms and conditions of licenses granted to us by others.

We have entered into license agreements with third parties and we may enter into additional license agreements in the future with others to advance our research and development or allow commercialization of drug candidates. These and other licenses may not provide exclusive rights to use such intellectual property and other rights in all relevant fields of use and in all territories in which we may wish to develop or commercialize our drug candidates in the future. Further, these and other licenses may also include certain restrictions or obligations that limit our ability to engage with third parties, including potential restrictions on sublicensing or outsourcing certain activities.

In addition, subject to the terms of any such license agreements, we may not have the right to control the preparation, filing, prosecution, maintenance, enforcement, and defense of patents and patent applications covering our drug candidates that we license from third parties. In such an event, we cannot be certain that these patents and patent applications will be prepared, filed, prosecuted, maintained, enforced, and defended in a manner consistent with the best interests of our business. If any of our current or future licensors fail to prosecute, maintain, enforce, and defend such patents, or 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 drug candidates that are subject of such licensed rights could be adversely affected.

Our licensors and any future licensors may have relied on third party consultants or collaborators or on funds from third parties such that our licensors are not the sole and exclusive owners of the patents we in-license. If it is later determined that third parties own the rights to our in-licensed patents, or if other third parties have ownership rights to our in-licensed patents, such third parties may be able to license such patents to our competitors, and our competitors could market drug products similar or identical to our drug candidates. This could have a material adverse effect on our competitive position, business, financial condition, results of operations, and prospects.

It is possible that we may be unable to obtain additional licenses at a reasonable cost or on 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 rights licensed to us. In that event, we may be required to expend significant time and resources to redesign our drug candidates, or the methods for manufacturing them or to develop or license replacement technology, 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 drug candidates, which could harm our business, financial condition, results of operations, and prospects significantly. We cannot provide any assurances that third party patents do not exist which might be enforced against our current manufacturing methods, drug candidates, methods of use, or future methods or drug candidates resulting in either an injunction prohibiting our manufacture or future sales, or, with respect to our future sales, an obligation on our part to pay royalties or other forms of compensation to third parties, which could be significant.

If we fail to comply with our obligations in the agreements under which we license intellectual property rights or other rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

Disputes may arise between us and our current or future licensors regarding intellectual property and other rights subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our drug candidates infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense to third parties;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- our right to transfer or assign the license;
- the inventorship 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; and
- the priority of invention of patented technology.

In addition, the agreements under which we license intellectual property or other rights from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or other rights, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations, and prospects. Moreover, if disputes over intellectual property or other rights that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected drug candidates, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

In spite of our best efforts, our licensors might conclude that we have materially breached our license agreements and might therefore terminate the license agreements, thereby removing our ability to develop and commercialize drug candidates covered by these license agreements. If these in-licenses are terminated, or if the underlying patents fail to provide the intended exclusivity, competitors would have the freedom to seek regulatory approval of, and to market, drug products identical to ours. This could have a material adverse effect on our competitive position, business, financial condition, results of operations, and prospects.

The patent protection and patent prosecution for some of our drug candidates may be dependent on third parties.

While we normally seek to obtain the right to control prosecution, maintenance and enforcement of the patent applications and patents relating to our drug candidates and their use, there may be times when the filing and prosecution activities for patent applications and patents relating to our drug candidates are controlled by licensors or collaboration partners. If a licensor or collaboration partner fails to prosecute, maintain and enforce such patents and patent applications in a manner consistent with the best interests of our business, including by payment of all applicable fees for patent applications and patents covering our drug candidates, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, our ability to develop and commercialize those drug candidates may be adversely affected and we may not be able to prevent competitors from making, using and selling drug products similar or identical to our drug candidates. In addition, even where we have the right to control patent prosecution of patents and patent applications we have licensed to and from third parties, we may still be adversely affected or prejudiced by actions or inactions of our licensees, our licensors and their counsel that took place prior to the date upon which we assumed control over patent prosecution.

Intellectual property discovered or developed through government funded programs may be subject to federal regulations such as “march-in” rights, certain reporting requirements and a manufacturing preference for U.S.-based companies. Compliance with such regulations may limit our exclusive rights and limit our ability to contract with non-U.S. manufacturers, which could adversely affect our ability to successfully develop and commercialize our drug products.

Pursuant to the Bayh-Dole Act of 1980 (the "Bayh-Dole Act”), the U.S. government may have certain rights in any invention developed or reduced to practice with government funding. We have in the past discovered, developed, acquired, or licensed new intellectual property that has been generated through the use of U.S. government funding or grants in which the U.S. government may have certain rights pursuant to the Bayh-Dole Act, and we may do so in the future. These U.S. government rights include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right, under certain limited circumstances, to require us to grant exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third party if it determines that: (1) adequate steps have not been taken to commercialize the invention; (2) government action is necessary to meet public health or safety needs; or (3) government action is necessary to meet requirements for public use under federal regulations (also referred to as “march-in rights”). Such “march-in” rights would apply to new subject matter arising from the use of such government funding or grants and would not extend to pre-existing subject matter or subject matter arising from funds unrelated to the government funding or grants. If the U.S. government exercises

its march-in rights in our intellectual property rights that are generated through the use of U.S. government funding or grants, we could be required to license or sublicense intellectual property discovered or developed by us or that we license on terms unfavorable to us, and there can be no assurance that we would receive compensation from the U.S. government for the exercise of such rights. The U.S. government also has the right to take title to these inventions if the grant recipient fails to disclose the invention to the government or fails to file an application to register the intellectual property within specified time limits. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us to expend substantial resources. Should any of these events occur, it could significantly harm our business, results of operations and prospects. In addition, the Bayh-Dole Act requires that, in certain circumstances, any products embodying intellectual property generated with the use of U.S. government funds or produced through the use of any such intellectual property be manufactured substantially in the United States. This preference for U.S. industry may be waived by the federal agency that provided the funding if the owner or assignee of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. industry may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property, which could adversely affect our ability to successfully develop and commercialize our drug products and have a material adverse effect on our business, financial condition, results of operations, and prospects.

Risks Related to Cybersecurity

We are dependent on networks, infrastructure and data, which exposes us to data security risks, including security failures or breaches of our systems or those used by our CROs or other contractors or consultants. We are dependent upon our own or third-party information technology systems, infrastructure and data, including mobile technologies, to operate our business. The multitude and complexity of our computer systems may fail or suffer security breaches.

As discussed in the section of this report titled “Part 1, Item 1.C—Cybersecurity”, we have implemented various processes and policies for identifying, assessing, and managing material risks from cybersecurity threats. However, despite the implementation of such safeguards and security measures, our internal computer systems and those of our CROs and other contractors and consultants may nevertheless be vulnerable to damage from computer viruses and unauthorized access. Likewise, data privacy or security incidents or breaches by employees or others may pose a risk that sensitive data, including our intellectual property, trade secrets or personal information of our employees, patients, customers or other business partners may be exposed to unauthorized persons or to the public or may otherwise be misused. Cyberattacks, malicious internet-based activity, online and offline fraud, and other similar activities threaten the confidentiality, integrity, and availability of our personal, sensitive, confidential or proprietary information and information technology systems, and those of the third parties upon which we rely. For example, in April 2023, CRO Evotec SE faced a cybersecurity attack that temporarily disrupted its systems and operations. 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. During times of war and other major conflicts, we, the third parties upon which we rely, may be vulnerable to a heightened risk of these attacks, including retaliatory cyber-attacks, that could materially disrupt our systems and operations, supply chain, and ability to produce, sell and distribute our goods and services.

We and the third parties upon which we rely may be 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), 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, telecommunications failures, earthquakes, fires, floods, and other similar threats. Increases in remote work impacting how our employees work and access our systems could lead to additional opportunities for bad actors to launch cyberattacks or for employees to cause inadvertent security risks or incidents and may amplify the impacts of any security breach or incident. Future or past 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.

We may rely on third-party service providers and technologies to operate critical business systems to process sensitive information and other company data in a variety of contexts. We may also rely on third-party service providers to provide certain products or services, or otherwise to operate our business. 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. Security incidents or other interruptions suffered by our third-party service providers could cause us to 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. In addition, supply-chain attacks have increased in frequency and severity, and we cannot guarantee that third parties' infrastructure in our supply chain or our third-party partners' supply chains have not been compromised.

Our business partners face similar risks, and any security breach of, or security incident impacting, their systems or that they otherwise suffer could adversely affect our security posture. A security breach or incident or privacy violation that leads to loss of or unauthorized use, disclosure or modification of, or access to trade secrets, company resources, personal, sensitive, confidential or proprietary information, including protected health information or other patient information, or that prevents access to patient information, as well as the perception that any of the foregoing has occurred, could harm our reputation, compel us to comply with federal or state breach notification laws and foreign law equivalents, subject us to mandatory corrective action, cause us to provide other notification or take other steps in response to such breach or violation, require us to verify the correctness of database contents and otherwise subject us to litigation, claims, investigations, penalties or other liability under laws and regulations, any of which could disrupt our business or result in increased costs or loss of revenue or company resources. Moreover, the prevalent use of mobile devices that access confidential information, increase the risk of security breaches and incidents.

Despite efforts to create security barriers to the above-described threats, it is impossible for us to entirely mitigate these risks. To date, we have not experienced any material impact to our business, financial position or results of operations resulting from cyberattacks or other information security incidents such as phishing, social engineering, ransomware or malware attacks; however, because of the frequently changing attack techniques, along with the increased volume and sophistication of such attacks, our business, financial position or results of operations could be adversely impacted in the future. We may be unable to anticipate or prevent techniques used to obtain unauthorized access or to compromise our systems because they change frequently and are generally not detected until after an incident has occurred. If a compromise or other security breach or incident were to occur and cause the loss or corruption of data or interruptions in our operations, it could result in a material disruption of our development programs and our business operations. For example, the loss, unavailability, or corruption of clinical trial data from completed, ongoing or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Any disruption or security breach or incident resulting in loss or unavailability of, or damage to, our data or systems, or inappropriate use, disclosure or modification of personal, sensitive, confidential or proprietary information, could result in us incurring liability and in delays to further development and commercialization of our drug candidates could be delayed. While we have invested, and continue to invest, in the protection of our data and information technology infrastructure, there can be no assurance that our efforts will prevent service interruptions, or prevent or identify vulnerabilities or security breaches or incidents, that could adversely affect our business and operations or result in the loss, unavailability, or corruption of, or inappropriate access to or use of, critical or sensitive information or company resources. Any such interruptions,

breaches or incidents, or the perception any have occurred, could result in financial, legal, business or reputational harm to us. In addition, our liability insurance may not be sufficient in type or amount to cover us against claims related to security breaches, cyberattacks and other privacy and security breaches or incidents.

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.

Data collection is governed by restrictive regulations governing the use, processing and cross-border transfer of personal information.

As we conduct our clinical trials and continue to enroll patients in our current and future clinical trials, we may be subject to additional restrictions relating to privacy, data protection and data security. The collection, use, storage, disclosure, transfer, or other processing of personal data, including personal health data, regarding individuals in the European Economic Area("EEA"), is subject to the EU General Data Protection Regulation("GDPR"). The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive 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, providing notification of data breaches, 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 EEA, including the United States, and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 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, the GDPR includes restrictions on cross-border data transfers. Certain aspects of cross-border data transfers under the GDPR are subject to uncertainty, including as the result of legal proceedings in the EU. For example, in 2020, the Court of Justice for the EU invalidated the EU-U.S. Privacy Shield and imposed additional obligations in connection with the use of standard contractual clauses approved by the EU Commission. The United Kingdom("UK") also made amendments to its data protection regime on June 19, 2025, in the UK Data (Use and Access) Act("DUAA"). The European Commission has renewed its decision that the UK provides an adequate level of data protection and that generally permits personal data transfers from the EEA to the UK after assessing the DUAA, but it remains subject to potential revocation or modification. These and other developments with respect to cross-border data transfers may increase the complexity of transferring personal data across borders and may require us to review and amend our mechanisms relating to cross-border data transfer.

Further, the exit of the UK from the EU has created uncertainty regarding data protection regulation in the UK. The UK has implemented legislation similar to the GDPR, referred to as the UK GDPR, which provides for fines of up to the greater of up to the greater of £17.5 million or 4% of global turnover, and made targeted amendments to it in the UK Data (Use and Access) Act 2025. The GDPR and UK GDPR have increased our responsibility and liability in relation to personal data that we process where subject to these regimes, and we may be required to put in place or modify policies and measures to ensure compliance with the GDPR, including as implemented by individual countries, and the UK GDPR, each of which may require us to modify our policies and procedures and engage in additional contractual negotiations, and which may cause us to incur liabilities, expenses, costs, and operational losses. Compliance with the GDPR and UK GDPR will be a rigorous and time-intensive process that may increase our cost of doing business or require us to change our business practices, and despite our efforts, there is a risk that we may be subject to fines and penalties, litigation, and reputational harm in connection with our activities in the EEA and the UK.

In addition, in the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal data privacy laws, consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), and other similar laws (e.g., wiretapping laws). California has enacted the California Consumer Privacy Act("CCPA"), which creates new individual privacy rights for California consumers (as defined in the law) and places increased privacy and security obligations on entities handling personal data of consumers or households. The CCPA requires covered companies to provide new disclosure to consumers about such companies' data collection, use and sharing practices, provide such consumers new ways to opt-out of certain sales or transfers of personal information, and provide consumers with additional causes of action. The California Privacy Rights Act of 2020 (CPRA), which became operative January 1, 2023, expands the CCPA's requirements, including applying to personal information of business representatives and employees and establishing a new regulatory agency to implement and enforce the law. Although the CCPA exempts some data processed in the context of clinical trials, the CCPA and CPRA may increase compliance costs and potential liability with respect to other personal data we maintain about California residents. Additionally, numerous other states have proposed or enacted laws addressing privacy and security, including Washington's My Health, My Data Act, and several laws imposing obligations similar to those of the CCPA. The U.S. Department of Justice also has issued rules regarding certain bulk sensitive personal data transfers. The U.S. federal government also is contemplating federal privacy legislation. The CCPA, CPRA, and other evolving legislation relating to privacy, data protection, and information security may impact our business activities and exemplify the vulnerability of our business to the evolving regulatory environment related to personal data and protected health information.

We may also be bound by contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. For example, certain privacy laws, such as the GDPR and the CCPA/CPRA, require us to impose specific contractual restrictions on our service providers, and we may also be subject to use and disclosure limitations in our contracts with providers who share information with us for clinical trials. Additionally, we may publish privacy policies, marketing materials and other statements, such as compliance with certain certifications or self-regulatory principles, regarding data privacy and security. If these policies, materials, or statements 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 or other adverse consequences.

Obligations related to data privacy and security are quickly changing, becoming increasingly stringent, and creating regulatory uncertainty. Additionally, these obligations may be subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions. Preparing for and complying with these obligations requires us to devote significant resources. These obligations may necessitate changes to our business model, information technologies, systems, and practices and to those of any 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.

Compliance with U.S. and international data protection laws and regulations could require us to take on more onerous obligations in our contracts, restrict our ability to collect, use and disclose data, or in some cases, impact our ability to operate in certain jurisdictions. Any actual or alleged failure to comply with U.S. or international laws and regulations relating to privacy, data protection, and information security could result in governmental investigations, proceedings and enforcement actions (which could result in civil or criminal penalties), private litigation or adverse publicity, harm to our reputation, and could negatively affect our operating results and business. Moreover, clinical trial subjects about whom we or our potential collaborators obtain information, as well as the providers who share this information with us, may contractually limit our ability to use and disclose the information or impose other obligations or restrictions in connection with our use, retention, and other processing of information, and we may otherwise face contractual restrictions applicable to our use, retention, and other processing of information. Claims that we have violated individuals' privacy rights, failed to comply with laws relating to privacy, data protection, or information security, or breached our contractual obligations, even if we are not found liable, could be expensive and time consuming to defend and could result in adverse publicity that could harm our business.

Risks Related to Ownership of Our Common Stock

We do not know whether an active market for our common stock will be sustained, and, as a result, it may be difficult for you to sell your shares of our common stock.

If an active market for our common stock is not sustained, it may be difficult for you to sell your shares of common stock at an attractive price or at all. We cannot predict the prices at which our common stock will trade. It is possible that in one or more future periods our results of operations and progression of our drug product pipeline may not meet the expectations of public market analysts and investors, and, as a result of these and other factors, the price of our common stock may fall.

The market price of our common stock has been and may continue to be volatile, which could result in substantial losses for investors.

The market price of our common stock may be volatile. As a result, you may not be able to sell your common stock at or above the price that you paid for such shares. Some of the factors that may cause the market price of our common stock to fluctuate include:

- results of nonclinical studies and clinical trials;
- the success of existing or new competitive products or technologies;
- the timing and results of clinical trials for our current drug candidates and any future drug candidates that we may develop;
- commencement or termination of collaborations for our drug candidates;
- failure or discontinuation of any of our drug candidates;
- results of nonclinical studies, clinical trials or regulatory approvals of drug candidates of our competitors, or announcements about new research programs or drug candidates of our competitors;
- investor reactions to other companies' drug development results, including product failures or negative responses from regulatory authorities;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- negative press coverage;
- the potential commencement of any litigation and/or governmental investigations;
- the level of expenses related to any of our research programs, drug candidates that we may develop;
- the results of our efforts to develop additional drug candidates or drug products;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- announcement or expectation of additional financing efforts, including, but not limited to, under our ATM offering;
- sales of our common stock by us, our insiders, or other stockholders;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in estimates or recommendations by securities analysts, if any, that cover our stock;
- changes in the structure of healthcare payment systems;

- market conditions in the pharmaceutical and biotechnology sectors;
- volatility with the banking system;
- the potential impact of health epidemics on our business;
- direct or indirect impacts on our business, our suppliers and other third parties and our clinical sites as a result of geopolitical events;
- changes in regulations and customs, tariffs or trade barriers, or the perception that any such changes could occur;
- general economic, industry, and market conditions; and
- the other factors described in this "Risk Factors" section.

In recent years, the stock market in general, and the market for pharmaceutical and biotechnology companies in particular, has experienced significant price and volume fluctuations that have often been unrelated or disproportionate to changes in the operating performance of the companies whose stock is experiencing those price and volume fluctuations. Broad market and industry factors may seriously affect the market price of our common stock, regardless of our actual operating performance. Following periods of such volatility in the market price of a company's securities, securities class action litigation has often been brought against that company. Because of the potential volatility of our stock price, we may become the target of securities litigation in the future. Securities litigation could result in substantial costs and divert management's attention and resources from our business.

Future sales, or the perception of future sales, of a substantial amount of our common stock could depress the trading price of our common stock.

Our stock price could decline as a result of sales of a large number of shares of our common stock or the perception that these sales could occur. These sales, or the possibility that these sales may occur, also might make it more difficult for us to sell equity securities in the future at a time and at a price that we deem appropriate.

For example, in connection with our December 2025 private placement and our in-license of lasofoxifene, we entered into registration rights agreements with various parties that require us to prepare and file resale registration statements upon request, which when filed and declared effective by the SEC will permit the resale by such stockholders of approximately 5.4 million shares of our common stock as well as approximately 58.6 million shares of common stock issuable upon the exercise of prefunded warrants and warrants. We have in the past and may again in the future issue shares of our common stock to strategic partners in connection with license agreements, as we did in December 2025 in connection with our in-license of lasofoxifene.

We also register the offer and sale of all shares of common stock that we may issue under our equity incentive plans. Once we register the offer and sale of shares for the holders of registration rights and option holders, they can be freely sold in the public market upon issuance, subject to any related lock-up agreements or applicable securities laws. Our equity incentive plan also includes an evergreen provision, pursuant to which the share reserve used to make equity grants under such plan automatically increases on the first day of each calendar year unless our board of directors takes action to prevent the increase prior to such date. We believe this evergreen provision is an important feature of our equity incentive plan as it enables us to hire and retain key contributors to our business. As we look to expand the size of our organization to advance the development of our product candidates, including lasofoxifene and ATH-1105, our existing share reserve and evergreen provision under our current equity incentive plans may be insufficient to support our growth plans and we expect that we may need to consider amendments to such equity incentive plans or adoption of new equity incentive plans to provide additional flexibility for our hiring and retention plans. Any increase to the share reserves for our equity incentive plans in the future would further dilute existing stockholders.

Our executive officers and directors may enter into Rule 10b5-1 trading plans in the future. Under a Rule 10b5-1 trading plan, a broker executes trades pursuant to parameters established by the employee, director, or officer when entering into the plan, without further direction from the employee, officer, or director.

In addition, in the future, we may issue additional shares of common stock or other equity or debt securities convertible into common stock in connection with a financing, acquisition, litigation settlement, employee arrangements or otherwise. Any such future issuance, including any additional issuances pursuant to our "at-the-market" equity offering sales agreement with Cantor Fitzgerald, could result in substantial dilution to our existing stockholders and could cause our stock price to decline.

Our reverse stock split to regain compliance with the Nasdaq Capital Market Minimum Bid Requirement may not result in a lasting proportional increase in the per share price of our common stock and may decrease the liquidity of our common stock.

Nasdaq Listing Rule 5450(a)(1) requires listed securities to maintain the Minimum Bid Requirement, and Nasdaq Listing Rule 5810(c)(3)(A) provides that a failure to meet the Minimum Bid Requirement exists if the deficiency continues for a period of 30 consecutive business days. On October 16, 2024, we received written notice from the Listing Qualifications Department of Nasdaq notifying us that, based on the closing bid price of our common stock for the previous 30 consecutive business days, we did not comply with the Minimum Bid Requirement for continued listing on The Nasdaq Global Market, and that we had until April 14, 2025 to regain such compliance. On April 15, 2025, we received a letter from Nasdaq approving the transfer of the listing of the Company's common stock from the Nasdaq Global Select Market to the Nasdaq Capital Market, granting an additional 180-day grace period, or until October 13, 2025, to regain compliance with the Minimum Bid Requirement. To regain compliance, our board of directors and stockholders approved a 1-for-10 reverse stock split. Although we have regained compliance with the Nasdaq's listing standards, the long-term effect of the reverse stock split, if any, on the market price for our common stock cannot be accurately predicted. Since the effective date of the reverse stock split on September 17, 2025, the trading price of our common stock increased. From time to time it has traded below the proportionately-adjusted closing price on September 17, 2025. Because some investors may view a reverse stock split negatively, any such decrease may be the result, at least in part, of the reverse stock split. Further, the liquidity of the shares of our common stock may be affected adversely by the reverse stock split given the reduced number of shares that are outstanding following the reverse stock split.

We and certain of our directors and executive officers have previously been, and may in the future be, named as defendants in lawsuits that could result in substantial costs and divert management's attention.

We and certain of our executive officers and directors have in the past been and may in the future be named as defendants in lawsuits concerning our business. Such litigation has caused, and may in the future cause, our management and board of directors to divert time and attention to the litigation and could adversely impact our reputation and further divert management and our board of directors' attention and resources from other priorities, including the execution of our operating plan and strategies that are important to our ability to grow our business and advance our drug candidates, any of which could have a material adverse effect on our business. In addition, resolution of litigation in a manner adverse to us and for which we incur substantial costs or damages not covered by our directors' and officers' liability insurance would have a material adverse effect on our financial condition and business.

Actions by activist stockholders have in the past been, and may in the future be, disruptive and could cause uncertainty about the strategic direction of our business.

Our business could be negatively affected as a result of stockholder activism, which could be disruptive and cause uncertainty about the strategic direction of our business. For example, in February 2022, an activist stockholder announced his intention to nominate himself and one other candidate for election to our board of directors at our 2022 annual meeting of stockholders. While this proxy contest

was unsuccessful, stockholder activism could recur. At times our market capitalization has been less than the aggregate value of our cash, cash equivalents and investments, and other biotechnology companies in this situation have received proposals from shareholder activists to liquidate and return capital to investors. Any such proxy contest or other activist efforts could have an adverse effect on our business, results of operations, and financial condition.

Even if any such actions were not successful, the increased costs that we would bear and the distraction of our board of directors and senior management could negatively impact our business, although we cannot predict with certainty the extent of such negative impacts.

A significant portion of our total outstanding shares may be sold into the market in the near future, which could cause the market price of our common stock to decline 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. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock. In addition, shares issued upon the exercise of stock options outstanding under our equity incentive plans or pursuant to future awards granted under those plans will become available-for-sale in the public market to the extent permitted by the provisions of applicable vesting schedules, any applicable market standoff and lock-up agreements, and Rule 144 and Rule 701 under the Securities Act.

We also register shares of common stock that we may issue under our equity compensation plans. Once we register these shares, they can be freely sold in the public market upon issuance and once vested, subject to volume limitations applicable to affiliates. If any of these additional shares are sold, or if it is perceived that they will be sold, in the public market, the market price of our common stock could decline.

Our directors, executive officers and significant stockholders collectively own a substantial percentage of our common stock, which could limit your ability to affect the outcome of key transactions, including a change of control.

Our directors, executive officers, significant holders of our outstanding common stock and their respective affiliates collectively beneficially own a substantial amount of our outstanding common stock as of December 31, 2025. As a result, these stockholders, if they act together, will be able to influence our management and affairs and all matters requiring stockholder approval, including the election of directors and approval of significant corporate transactions. This concentration of ownership may have the effect of delaying or preventing a change in control of our company and might affect the market price of our common stock.

We are a "smaller reporting company" and a "non-accelerated filer", and the reduced disclosure requirements available to us may make our common stock less attractive to investors.

We are a "smaller reporting company" and a "non-accelerated filer." For so long as we remain a smaller reporting company, we are permitted and plan to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not smaller reporting companies, which include reduced disclosure obligations regarding executive compensation. We qualify as a smaller reporting company and we will remain a smaller reporting company so long as, as of June 30 of the preceding year, (i) the market value of our common shares held by non-affiliates, or our public float, is less than $250 million; or (ii) we have annual revenues less than $100 million and either we have no public float or our public float is less than $700 million. In addition, so long as we qualify as a non-accelerated filer, we are not required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002 (the "Sarbanes-Oxley Act"). As a result, the information we provide stockholders will be different than the information that is available with respect to certain other public companies. We cannot predict whether investors will find our common stock less attractive if we 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.

Failure to build and maintain our finance infrastructure and improve our accounting systems and controls could impair our ability to comply with the financial reporting and internal controls requirements for publicly traded companies.

As a public company, we operate in an increasingly demanding regulatory environment, which requires us to comply with the Sarbanes-Oxley Act, the regulations of The Nasdaq Capital Market, the rules and regulations of the SEC, expanded disclosure requirements, accelerated reporting requirements and more complex accounting rules. Company responsibilities required by the Sarbanes-Oxley Act include establishing corporate oversight and adequate internal control over financial reporting and disclosure controls and procedures. Effective internal controls are necessary for us to produce reliable financial reports and are important to help prevent financial fraud. We must perform system and process evaluation and testing of our internal controls over financial reporting to allow management to report on the effectiveness of our internal controls over financial reporting, as required by Section 404 of the Sarbanes-Oxley Act. We may experience difficulty in meeting these reporting requirements in the future.

The process of building and maintaining our accounting and financial functions and infrastructure has required and will continue to require significant additional professional fees, internal costs and management efforts. Any disruptions or difficulties in implementing or using such a system could adversely affect our controls and harm our business. Moreover, such disruption or difficulties could result in unanticipated costs and diversion of management attention. In addition, we may discover weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our 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, we may not be able to produce timely and accurate financial statements. If we cannot provide reliable financial reports or prevent fraud, our business and results of operations could be harmed, investors could lose confidence in our reported financial information and we could be subject to sanctions or investigations by Nasdaq, the SEC or other regulatory authorities.

Anti-takeover provisions in our charter documents and under Delaware or Washington law could make an acquisition of us difficult, limit attempts by our stockholders to replace or remove our current management and limit our stock price.

Provisions of our amended and restated certificate of incorporation and amended and restated bylaws may delay or discourage transactions involving an actual or potential change in our control or change in our management, including transactions in which stockholders might otherwise receive a premium for their shares, or transactions that our stockholders might otherwise deem to be in their best interests. Therefore, these provisions could adversely affect the price of our common stock. Among other things, the amended and restated certificate of incorporation and amended and restated bylaws:

- permit the board of directors to issue up to 100,000,000 shares of preferred stock, with any rights, preferences and privileges as they may designate;
- provide that the authorized number of directors may be changed only by resolution of the board of directors;

- provide that all vacancies, including newly-created directorships, may, except as otherwise required by law, be filled by the affirmative vote of a majority of directors then in office, even if less than a quorum;
- divide the board of directors into three classes;
- provide that a director may only be removed from the board of directors by the stockholders for cause;
- require that any action to be taken by our stockholders must be effected at a duly called annual or special meeting of stockholders and may not be taken by written consent;
- provide that stockholders seeking to present proposals before a meeting of stockholders or to nominate candidates for election as directors at a meeting of stockholders must provide notice in writing in a timely manner, and meet specific requirements as to the form and content of a stockholder's notice;
- prevent cumulative voting rights (therefore allowing the holders of a plurality of the shares of common stock entitled to vote in any election of directors to elect all of the directors standing for election, if they should so choose);
- provide that special meetings of our stockholders may be called only by the chairman of the board, our chief executive officer or by the board of directors; and
- provide that stockholders are permitted to amend the bylaws only upon receiving at least two-thirds of the total votes entitled to be cast by holders of all outstanding shares then entitled to vote generally in the election of directors, voting together as a single class.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which generally prohibits a Delaware corporation from engaging in any of a broad range of business combinations with any "interested" stockholder for a period of three years following the date on which the stockholder became an "interested" stockholder. Likewise, because our principal executive offices are located in Washington, the anti-takeover provisions of the Washington Business Corporation Act may apply to us under certain circumstances now or in the future. These provisions prohibit a "target corporation" from engaging in any of a broad range of business combinations with any stockholder constituting an "acquiring person" for a period of five years following the date on which the stockholder became an "acquiring person."

Our amended and restated bylaws provide that the Court of Chancery of the State of Delaware and the federal district courts of the United States are the exclusive forums for substantially all disputes between us and 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 amended and restated bylaws provide that the Court of Chancery of the State of Delaware (or, if the Court of Chancery does not have jurisdiction, another state court in Delaware or the federal district court for the District of Delaware) will be the exclusive forum for any derivative action or proceeding brought on our behalf; any action asserting a breach of fiduciary duty; any action asserting a claim against us arising under the Delaware General Corporation Law, our certificate of incorporation or our amended and restated bylaws; any action to interpret, apply, enforce or determine the validity of our certificate of incorporation or our amended and restated bylaws; and any action asserting a claim against us that is governed by the internal affairs doctrine. This provision would not apply to suits brought to enforce a duty or liability created by the Exchange Act or any other claim for which the U.S. federal courts have exclusive jurisdiction.

Our amended and restated bylaws further provide that the federal district courts of the United States will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act. The enforceability of similar exclusive federal forum provisions in other companies' organizational documents has been challenged in legal proceedings, and while the Delaware Supreme Court has ruled that this type of exclusive federal forum provision is facially valid under Delaware law, there is uncertainty as to whether other courts would enforce such provisions and that investors cannot waive compliance with the federal securities laws and the rules and regulations thereunder.

These exclusive forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage such lawsuits against us and our directors, officers and other employees. Alternatively, if a court were to find either exclusive forum provision in our amended and restated bylaws to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving such action in other jurisdictions, all of which could have a material adverse effect on our business, financial condition, and results of operations.

General Risk Factors

The loss of any of our key personnel could significantly harm our business, results of operations and competitive position.

In order to compete, we must attract, retain, and motivate executives and other key employees. Hiring and retaining qualified executives, scientists, technical and legal and accounting staff are critical to our business, and competition for experienced employees in our industry can be high. We expect to hire a significant number of such employees in the near term as we pursue the clinical development of lasofoxifene, and as we continue to develop ATH-1105. The loss of one or more of these key employees, or our inability to hire additional key personnel when needed, could have a material adverse effect on our business and prospects.

In addition, the organizational restructuring we undertook in September 2024 that significantly reduced our workforce, including the departure of our chief business officer and chief financial officer and our chief operating officer and chief development officer, may negatively impact employee morale for those who are not directly impacted, which may increase employee attrition and hurt future recruiting efforts, hindering our ability to achieve our key priorities. Any failure to achieve the expected benefits from the reduction in workforce could adversely affect our stock price, financial condition and ability to achieve our key priorities, as well as lead to litigation.

Our advisors and consultants are classified as independent contractors, and we can face consequences if it is determined that they are misclassified as such.

There is often uncertainty in the application of worker classification laws, and consequently there is risk to us that our independent contractors could be deemed to be misclassified under applicable law. The tests governing whether a service provider is an independent contractor or an employee are typically highly fact sensitive and can vary by governing law. Laws and regulations that govern the status and misclassification of independent contractors are also subject to divergent interpretations by various authorities, which can create uncertainty and unpredictability. A misclassification determination or allegation creates potential exposure for us, including but not limited to monetary exposure arising from or relating to failure to withhold and remit taxes, unpaid wages, and wage and hour laws and requirements (such as those pertaining to minimum wage and overtime); claims for employee benefits, social security, workers' compensation and unemployment; claims of discrimination, harassment, and retaliation under civil rights laws; claims under laws pertaining to unionizing, collective bargaining, and other concerted activity; and other claims, charges, or other proceedings under laws and regulations applicable to employers and employees, including risks relating to allegations of joint employer liability. Such claims could result in monetary damages (including but not limited to wage-based damages or restitution, compensatory damages, liquidated damages, and punitive damages), interest, fines, penalties, costs, fees (including but not limited to attorneys' fees), criminal and other liability, assessment, or settlement.

Such an allegation, claim, adverse determination, including but not limited to with respect to advisors and consultants that provide services to us could also harm our brand and reputation, which could adversely impact our business.

If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock will rely in part on the research and reports that industry or financial analysts publish about us or our business. If no or few analysts commence coverage of us, the trading price of our stock could decrease. Even if we do obtain analyst coverage, if one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

We will continue to incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, and particularly after we are no longer a smaller reporting company, we will incur significant legal, accounting, and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of The Nasdaq Capital Market, and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. We have hired, and expect that we will continue to need to hire, additional accounting, finance, and other personnel in connection with our efforts to comply with the requirements of being a public company, and our management and other personnel will need to devote a substantial amount of time towards maintaining compliance with these requirements. These requirements will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that the rules and regulations applicable to us as a public company may make it more difficult and more expensive for us to obtain director and officer liability insurance, which could make it more difficult for us to attract and retain qualified members of our board of directors. We continue to evaluate and monitor these rules and regulations and cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

If we are unable to maintain effective internal controls, our business, financial position and results of operations could be adversely affected.

As a public company, we are subject to reporting and other obligations under the Exchange Act, including the requirements of Section 404 of the Sarbanes-Oxley Act, which require annual management assessments of the effectiveness of our internal control over financial reporting.

The rules governing the standards that must be met for management to determine that our internal control over financial reporting is effective are complex and require significant documentation, testing and possible remediation to meet the detailed standards under the rules. During the course of our testing, our management may identify material weaknesses or deficiencies which may not be remedied in time to meet the deadline imposed by the Sarbanes-Oxley Act. These reporting and other obligations place significant demands on our management and administrative and operational resources, including accounting resources.

Our management is responsible for establishing and maintaining adequate internal control over financial reporting. Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with accounting principles generally accepted in the United States. Any failure to maintain effective internal controls could have an adverse effect on our business, financial position and results of operations.

The potential effects of health epidemics could adversely impact our business, including our nonclinical studies and clinical trials.

Our business could in the future be adversely impacted by the effects of possible health epidemics and other outbreaks which could cause disruptions that could severely impact our business, nonclinical studies and clinical trials. Such disruptions may include:

- delays or difficulties in enrolling and retaining patients in our clinical trials;
- difficulties interpreting data from our clinical trials due to the possible effects of such diseases on cognition of the subjects enrolled in our clinical trials;
- delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;
- diversion of healthcare resources away from the conduct of clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;
- interruption of key clinical trial activities, such as clinical trial site data monitoring, due to limitations on travel imposed or recommended by federal or state governments, employers and others or interruption of clinical trial subject visits and study procedures (such as endoscopies that are deemed non-essential), which may impact the integrity of subject data and clinical study endpoints;
- interruption or delays in the operations of the FDA or other regulatory authorities, which may impact review and approval timelines;
- interruption of, or delays in receiving, supplies of our drug candidates from our contract manufacturing organizations due to staffing shortages, production slowdowns or stoppages and disruptions in delivery systems;
- interruptions in nonclinical studies due to restricted or limited operations at our laboratory facility;
- limitations on employee resources that would otherwise be focused on the conduct of our nonclinical studies and clinical trials, including because of sickness of employees or their families or the desire of employees to avoid contact with large groups of people;
- interruptions, difficulties or delays arising in our existing operations and company culture as a result of some or all of our employees working remotely;
- interruption or delays to our sourced discovery and clinical activities; and
- changes in clinical site procedures and requirements as well as regulatory requirements for conducting clinical trials during the pandemic.

The trading prices for shares of biopharmaceutical companies have in the past been and could in the future be highly volatile as a result of health epidemics, and the trading prices for shares of our common stock could also experience high volatility. In the event of an emergence of new disease outbreaks or a resurgence of COVID-19, we could face difficulties raising capital through sales of our common stock or such sales may be on unfavorable terms. In addition, a recession, depression or other sustained adverse market event resulting from a health epidemic, could materially and adversely affect our business and the value of our common stock.

The ultimate impact of a possible health epidemic or other outbreak, including a resurgence of COVID-19, on our business operations is highly uncertain and subject to change and will depend on future developments, which cannot be accurately predicted. In addition, our business could be significantly adversely affected by other business disruptions to us or our third-party providers that could seriously harm our potential future revenue and financial condition and increase our costs and expenses. Our operations, and those of our CROs, CMOs, and other contractors, consultants, and third parties could be subject to other global pandemics, earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man-made disasters or business interruptions, for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. We rely on third-party manufacturers to produce and process our drug candidates. Our ability to obtain clinical supplies of our drug candidates could be disrupted if the operations of these suppliers are affected by a man-made or natural disaster or other business interruption.

Item 1B. Unresolved Staff Comments.

None.

Item 1C. Cybersecurity.

Cybersecurity Risk Management Strategy

We have implemented various processes and policies for identifying, assessing, and managing material risks from cybersecurity threats. Our cybersecurity risk management strategy is designed following the Cybersecurity Framework set by the National Institute of Standard and Technology ("NIST).

We assess our information technology ("IT") environment against the NIST Cybersecurity Framework, as well as various cyber-attack vectors, working to identify and remediate risks. We implement reasonable administrative, technical and procedural safeguards to manage cybersecurity risks, for example, by enforcing single sign-on or multi-factor authentication where supported, and the use of mobile device management to secure company resources on employee personal devices. Additionally, we engage third-party cybersecurity experts to assess the security of our network and perform continuous system monitoring, and we engage a third party to perform internal audits of our IT General Controls ("ITGCs"). We have implemented certain processes to oversee and identify risks from cybersecurity threats associated with our use of third-party service providers, for example, by evaluating such service providers' own cybersecurity processes and reviewing available certification and audit reports, including International Organization for Standardization certifications for information security management systems, and System and Organization Controls reports.

At this time, we have not experienced cybersecurity incidents, or are aware of any risks from cybersecurity threats, that have materially affected or are reasonably likely to materially affect us, including our business strategy, results of operations, or financial condition.

Cybersecurity Governance

Board of Directors

Our board of directors is responsible for general oversight and regular review of information regarding our risks, including cybersecurity risks. Members of management communicate an overview of our current cybersecurity environment to our board of directors at least annually and provide updates to our board of directors regarding cybersecurity matters periodically throughout the year. Additionally, our third-party auditors inform the audit committee of our board of directors of our ITGC framework and control testing results, which include controls related to cybersecurity risks. Further, management has established cybersecurity incident response processes for escalating the communication of cybersecurity incidents up to the board of directors, as appropriate.

Management

Material risks from cybersecurity threats are assessed and managed by a dedicated team comprised of internal and external IT professionals experienced in cybersecurity threat risk management, who ultimately report to our senior vice president, finance and accounting. Our chief financial officer has extensive operational and leadership experience overseeing accounting and finance functions at various organizations, including oversight of critical accounting and finance IT systems. Our director of IT has over 15 years of experience with IT and cybersecurity risk management.

Our team of IT professionals, which continuously monitors our IT environment for cybersecurity threats and incidents, routinely reports on cybersecurity incident prevention, detection, mitigation, and remediation efforts to our chief financial officer and chief compliance officer. Additionally, we have established policies addressing processes for responding to potential cybersecurity incidents, including assessment, communication, and remediation protocols. Our incident response processes further provide for the escalation of cybersecurity incidents to our executive management team and board of directors, as appropriate.

Item 2. Properties.

Our corporate headquarters is located in Bothell, Washington, where we currently lease approximately 19,326 square feet of laboratory and office space, which leases expire in August 2027. We believe these facilities will be adequate for the foreseeable future and that suitable additional or substitute space will be available as and when needed.

Item 3. Legal Proceedings.

From time to time, we are subject to various legal proceedings or claims that arise in the ordinary course of business. As of December 31, 2025, we are not a party to any legal proceedings that, in the opinion of our management, would reasonably be expected to have a material adverse effect on our business, financial condition, operating results or cash flows if determined adversely to us. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.

Market Information for Common Stock

Our common stock is traded on The Nasdaq Capital Market under the symbol "LONA".

Holders of Record

As of March 15, 2026, there were approximately 38 holders of record of our common stock. Because many of our shares are held by brokers and other institutions on behalf of shareholders, we are unable to estimate the total number of shareholders represented by these record holders.

Dividend Policy

We currently intend to retain all available funds and future earnings to fund the development and growth of our business and we do not anticipate paying any cash dividends in the foreseeable future.

Recent Sales of Unregistered Securities

None.

Stock Performance Graph

As a smaller reporting company, we are not required to provide the information requested by this item pursuant to Item 201(e) of Regulation S-K.

Issuer Repurchases of Equity Securities

None.

Item 6. [Reserved].

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

You should read the following management's discussion and analysis of financial condition and results of operations in conjunction with our consolidated financial statements and notes thereto included in Part II, Item 8 of this Annual Report on Form 10-K. This discussion and analysis and other parts of this report contain forward-looking statements based upon current beliefs, plans and expectations related to future events and our future financial performance that involve risks, uncertainties and assumptions, such as statements regarding our intentions, plans, objectives, expectations, forecasts and projections. Our actual results and the timing of selected events could differ materially from those anticipated in these forward-looking statements as a result of several factors, including those set forth under the section of this Annual Report on Form 10-K titled "Risk Factors" and elsewhere in this report. You should carefully read the "Risk Factors" to gain an understanding of the important factors that could cause actual results to differ materially from our forward-looking statements. Please also see the section of this report titled "Special Note Regarding Forward-Looking Statements."

Overview

We are a clinical-stage biopharmaceutical company dedicated to the development of novel therapeutics for high unmet medical needs, including treatment-resistant metastatic breast cancer and amyotrophic lateral sclerosis ("ALS"), with the goal of improving patients' lives.

Following a review of potential therapeutic and business opportunities, we acquired rights to a promising late-stage asset, lasofoxifene, for the potential treatment of ESR1-mutated ("mESR1") metastatic breast cancer in December 2025. This late-stage program has generated promising clinical data and we believe it represents a unique and exciting opportunity to diversify our pipeline. We believe this program fits well with our later-stage clinical development experience, and complements our in-house asset, ATH-1105, a Phase-2 ready program for the potential treatment of ALS.

The drug development landscape in both oncology and neuroscience is evolving rapidly, driven by breakthroughs in genetics, disease biology, and molecular pathway research. We are leveraging this scientific momentum to advance a pipeline of innovative, late-stage assets both internally developed and strategically in-licensed with the goal of accelerating their path to market and maximizing their clinical and commercial impact.

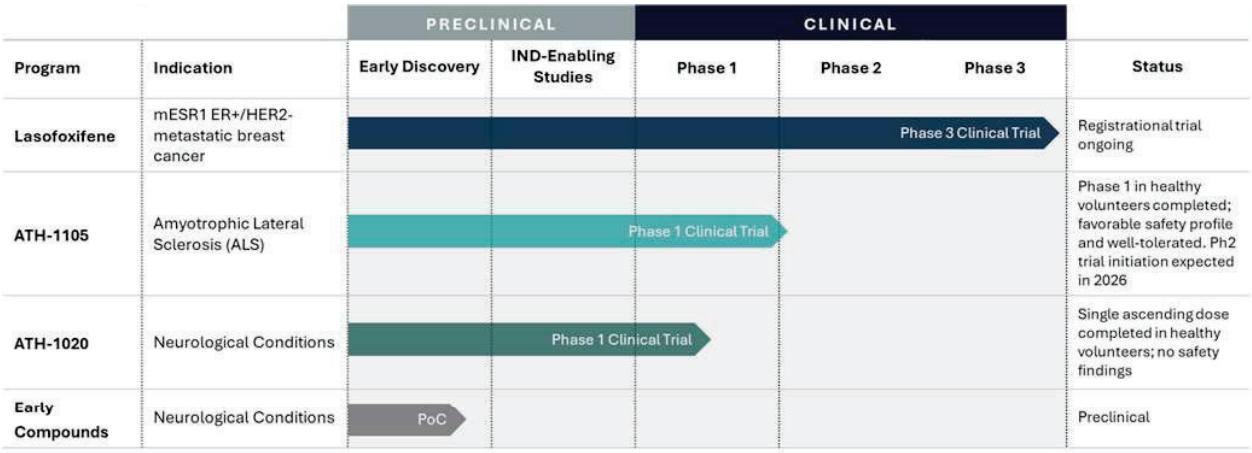
Our lead drug candidates, lasofoxifene and ATH-1105 , are novel, small molecule therapies with the potential to address devastating diseases where current treatment options are limited or ineffective. With a strong commitment to scientific excellence and patient-centered innovation, we aim to advance meaningful new therapies that are designed to treat patients with treatment-resistant metastatic breast cancer and ALS.

LeonaBio's Pipeline

Figure 1 below illustrates the current development stage of our proprietary drug candidates and early discovery and development programs, of which only lasofoxifene and ATH-1105 are currently in clinical development. Each program targets unmet needs in either oncology or neurology, leveraging differentiated mechanisms of action supported by preclinical and clinical evidence. Lasofoxifene, a selective estrogen receptor modulator ("SERM"), is currently being evaluated in an ongoing registrational Phase 3 trial for metastatic breast cancer with ESR1 mutations. ATH-1105, a small-molecule positive modulator of the neurotrophic HGF system, has completed a first-in-human Phase 1 trial, and planning for a Phase 2 clinical trial for the treatment of ALS is underway. We aim to advance these programs with a focus on disciplined clinical strategy and execution, data-driven decision making, and potential commercialization following receipt of applicable regulatory approvals. We are exploring the use of our

drug candidates that enhance the neurotrophic HGF system, including ATH-1020, with the goal of improving neuronal health and function in multiple neurological diseases. In addition, our drug discovery efforts have focused on designing and testing new early compounds directed towards novel targets for a variety of clinical applications.

Figure 1. Summary of Our Preclinical and Clinical Programs.



We were incorporated in March 2011 and since our inception, we have devoted substantially all of our resources to our research and development efforts such as small molecule compound discovery, nonclinical studies and clinical trials, as well as manufacturing activities, establishing and maintaining our intellectual property portfolio, hiring personnel, raising capital, and providing general and administrative support for these operations. We do not have any drug products approved for commercial sale, and we have not generated any revenues related to our drug products since inception. Our ability to generate drug product revenue sufficient to achieve profitability, if ever, will depend on the successful development of one or more of our drug candidates which we expect will take a number of years.

We are focused on the development of small molecule therapeutics which enables us to use well-established and widely available manufacturing processes and infrastructure, formulation processes and drug administration technologies or devices. We do not currently operate our own facilities for manufacturing, storing, or distributing our drug candidates. We utilize third-party CMOs to manufacture and supply our preclinical and clinical materials during the development of our drug candidates. We believe the synthesis of lasofoxifene and ATH-1105 is reliable and reproducible and the synthetic methods can be further optimized to enable large-scale production that continues to avoid use of toxic materials or specialized equipment or handling during the manufacturing process. We plan to continue to optimize the manufacturing process to support future large-scale and commercial supply that may be needed. Our goal is to identify and develop small molecule drug candidates that are cost-effective to manufacture and easily transferable to third party CMOs. We expect to use similar contract resources for commercialization of our drug products, at least until our resources and operations are at a scale that justifies investment in internal manufacturing capabilities.

To date, we have funded our operations primarily through proceeds from the sale of equity securities, including proceeds from the sale and issuance of common stock in our IPO, in a subsequent follow-on public offering and in our December 2025 private placement, the sale and issuance of convertible preferred stock, common stock warrants, and convertible notes, and to a lesser extent from grant income and stock option exercises. From inception to December 31, 2025, we have raised

aggregate net cash proceeds of approximately $489.8 million primarily from the issuance of our common stock (excluding option exercises), convertible preferred stock, common stock warrants, and convertible notes. We have incurred significant operating losses to date. Our net losses were $105.6 million and $96.9 million for the years ended December 31, 2025 and 2024, respectively. As of December 31, 2025, we had an accumulated deficit of $511.8 million and cash, cash equivalents and investments of $88.3 million.

We expect to continue to incur operating losses for the foreseeable future as we:

- continue to advance lasofoxifene, ATH-1105 and any other drug candidates through preclinical studies and clinical trials;
- advance our pipeline of drug candidates;
- continue to invest in our drug development programs;
- continue manufacturing activities;
- attract, hire and retain personnel;
- obtain, maintain, expand, protect and enforce our intellectual property portfolio;
- operate as a public company;
- maintain our laboratory and office facilities;
- implement and maintain operational, financial and management information systems; and
- seek regulatory approval for any drug candidates that successfully complete clinical trials.

Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our clinical trials and our expenditures on other research and development activities.

We will require substantial additional funding to support our continuing operations and further the development of our drug candidates. Until such time as we can generate significant revenue from drug product sales, if ever, we expect to finance our operations through the sale of equity, debt financings, or other capital sources, which could include income from collaboration, licensing or similar arrangements, for the foreseeable future. Adequate funding may not be available when needed or on terms acceptable to us, or at all. If we are unable to raise additional capital as needed, we may have to significantly delay, scale back or discontinue development of our drug candidates or other operations. Our ability to raise additional funds may be negatively impacted by potential adverse global economic conditions and disruptions to, and volatility in, the credit and financial markets in the United States and worldwide. If we fail to obtain necessary capital when needed on acceptable terms, or at all, it could force us to delay, limit, reduce or terminate our drug product development programs, commercialization efforts or other operations. Insufficient liquidity may also require us to relinquish rights to drug candidates at an earlier stage of development or on less favorable terms than we would otherwise choose. We cannot assure you that we will ever be profitable or generate positive cash flows from operating activities. Based upon our current operating plan, we estimate that our existing cash, cash equivalents and investments will be sufficient to fund our operating expenses and capital expenditure requirements through at least the next 12 months following the date of this report.

Recent Developments

December 2025 Private Placement Financing

In December 2025, in connection with our entry into the Sermonix License and the Ligand Agreement, we entered into a securities purchase agreement (the “PIPE Securities Purchase Agreement”) with a select group of investors, including Commodore Capital LP ("Commodore"), TCG Crossover Management LLC ("TCGX"), Perceptive Life Sciences Master Fund, Ltd, Perceptive Xontogeny Venture Fund II, LP (together with Perceptive Life Sciences Master Fund, Ltd, “Perceptive”)

and other accredited investors, one of which is affiliated with a member of our board of directors (collectively, the "PIPE Purchasers"), pursuant to which we issued and sold, by way of a private placement which closed on December 23, 2025 (the "Private Placement"), an aggregate of (a) 5,356,547 shares of our common stock, (b) pre-funded warrants to purchase 8,816,684 shares of our common stock and (c) accompanying warrants to purchase 23,031,494 shares of our common stock and/or shares underlying pre-funded warrants and (d) accompanying warrants to purchase 21,259,842 shares of our common stock and/or shares underlying pre-funded warrants. Gross proceeds from the Private Placement were approximately $90 million, excluding placement agent fees and offering expenses.

Workforce Reduction

On September 15, 2024, we committed to a workforce reduction that resulted in the termination of approximately 70% of our workforce. We took this step to decrease our costs, extend our cash runway, and create a more streamlined organization to support our strategic priorities, including the continued development of ATH-1105. We substantially completed the Restructuring by December 31, 2024. See Note 14 to our consolidated financial statements included elsewhere in this report for additional information.

Components of Operating Results

Operating Expenses

Research and Development

Research and development expenses consist primarily of direct and indirect costs incurred for our research activities, including our drug discovery efforts and the development of our drug candidates, including lasofoxifene and ATH-1105. Direct costs include laboratory materials and supplies, contracted research and manufacturing, clinical trial costs, consulting fees, and other expenses incurred to sustain our research and development program. Indirect costs include personnel-related expenses, consisting of employee salaries, related benefits, and stock-based compensation expense for employees engaged in research and development activities, and facilities and other expenses consisting of direct and allocated expenses for rent and depreciation, and lab consumables.

We expense research and development costs as incurred. Non-refundable advance payments for goods and services that will be used over time for research and development are capitalized and recognized as goods are delivered or as the related services are performed. In-licensing fees and other costs to acquire technologies used in research and development that have not yet received regulatory approval and that are not expected to have an alternative future use are expensed when incurred. We track direct costs by stage of program, clinical or preclinical. However, we do not track indirect costs on a program specific basis because these costs are deployed across multiple programs and, as such, are not separately classified.

We cannot reasonably determine the nature, timing, and estimated costs of the efforts that will be necessary to complete the development of, and obtain regulatory approval for, any of our drug candidates, including lasofoxifene or ATH-1105. Drug candidates in later stages of development generally have higher development costs than those in earlier stages. We expect to continue to incur increased research and development expenses for the foreseeable future as we continue to engage in research and development activities related to developing our drug candidates, including lasofoxifene and ATH-1105, our drug candidates advance into later stages of development, we conduct larger clinical trials, we seek regulatory approvals for any drug candidates that successfully complete clinical trials, we expand or advance our drug product pipeline, we maintain, expand, protect and enforce our intellectual property portfolio, and we incur expenses associated with hiring or retaining personnel to support our research and development efforts.

The process of conducting the necessary clinical research to obtain regulatory approval is costly and time-consuming, and the successful development of our drug candidates is highly uncertain. Our research and development expenses may vary significantly based on factors such as:

- research and development activities related to lasofoxifene and ATH-1105;
- the number and scope of preclinical and IND-enabling studies;
- the phases of development of our drug candidates;
- the progress and results of our research and development activities;
- per subject trial costs;
- the number of trials required for regulatory approval;
- the number of sites included in the trials;
- the countries in which the trials are conducted;
- the length of time required to enroll eligible subjects and initiate clinical trials;
- the number of subjects that participate in the trials;
- the drop-out and discontinuation rate of subjects;
- potential additional safety monitoring requested by regulatory agencies;
- the duration of subject participation in the trials and follow-up;
- the cost and timing of manufacturing of our drug candidates;
- the receipt of regulatory approvals from applicable regulatory authorities;
- the timing, receipt and terms of any marketing approvals from applicable regulatory authorities;
- the hiring and retention of research and development personnel;
- the degree to which we obtain, maintain, defend and enforce our intellectual property rights;
- the impact of health epidemics on timelines and clinical operations, which may lead to increased costs; and
- the extent to which we establish collaborations, licensing or similar arrangements and the performance of any related third parties.

A change in the outcome of any of these variables with respect to the development of any of our drug candidates could significantly change the costs and timing associated with the development of that drug candidate.

General and Administrative

General and administrative expenses consist primarily of personnel-related costs, consisting of employee salaries, related benefits, and stock-based compensation expense for our employees in the executive, legal, finance and accounting, human resources, and other administrative functions. General and administrative expenses also include third-party costs such as legal costs, insurance costs, accounting, auditing and tax related fees, business development fees, consulting fees and facilities and other expenses not otherwise included as research and development expenses. We expense general and administrative costs as incurred.

We expect to continue to incur general and administrative expenses for the foreseeable future as we maintain our headcount to support our continued research activities and development of our programs. We also anticipate that we will continue to incur expenses as a result of operating as a public company, including expenses related to compliance with the rules and regulations of the SEC, and those of any national securities exchange on which our securities are traded, legal, auditing, additional insurance expenses, investor relations activities, and other administrative and professional services.

Other Income, Net

Other income, net consists primarily of interest earned on our cash, cash equivalents and investments and the amortization of premiums and accretion of discounts on our available-for-sale securities. Absent further fundraising, we expect interest earned on our cash, cash equivalents and investments to decrease over the next several quarters as we continue to expend our cash balances to fund our ongoing operations.

Results of Operations

Comparison of the Years Ended December 31, 2025 and 2024

The following table summarizes our results of operations for the periods presented:

	Year Ended December 31,			
	2025	2024	Dollar Change	% Change
	(in thousands)			
Operating expenses:				
Research and development	$ 17,500	$ 70,682	$ (53,182)	-75%
Acquired in-process research and development	68,088	—	68,088	*
General and administrative	16,678	26,093	(9,415)	-36%
Legal expense	—	4,127	(4,127)	*
Total operating expenses	102,266	100,902	1,364	1%
Loss from operations	(102,266)	(100,902)	(1,364)	1%
Other income, net	1,236	3,962	(2,726)	-69%
Sermonix pre-funded warrant change in fair value	(4,579)	—	(4,579)	*
Net loss	$ (105,609)	$ (96,940)	$ (8,669)	9%

Research and Development Expenses

The following table shows the primary components of our research and development expenses for the periods presented:

	Year Ended December 31,			
	2025	2024	Dollar Change	% Change
	(in thousands)			
Direct costs:				
Acquired in-process research and development	$ 68,088	—	$ 68,088	*
Lasofoxifene	3,568	—	3,568	*
Fosgonimeton (ATH-1017)	2,335	41,510	(39,175)	-94%
ATH-1105	3,415	8,567	(5,152)	-60%
ATH-1020	5	495	(490)	-99%
Preclinical programs and other direct costs	327	1,580	(1,253)	-79%
Total direct costs	77,738	52,152	25,586	49%
Indirect costs:				
Personnel-related costs, including stock-based compensation	6,537	16,332	(9,795)	-60%
Facilities and other costs	1,313	2,198	(885)	-40%
Total research and development expenses	$ 85,588	$ 70,682	$ 14,906	21%

Research and development expenses increased by $14.9 million, from $70.7 million for the year ended December 31, 2024 to $85.6 million for the year ended December 31, 2025. The increase was driven primarily by acquired in-process research and development costs related to our license of lasofoxifene. Refer to Note 3 to our consolidated financial statements included elsewhere in this report for further discussion of this cost. Additionally there were costs associated with the ELAINE-3 trial for lasofoxifene in 2025. This increase was primarily offset by reductions in spend related to ATH-1017 and ATH-1105 in 2025 compared to 2024, as well as smaller reductions in spend related to ATH-1020 and other preclinical programs and direct costs. The decrease in ATH-1017 costs of $39.2 million were driven by decreases in contract research organization, other third party vendors and clinical site visit costs of $30.0 million following the completion of our Phase 2/3 LIFT-AD clinical trial and the conclusion of our corresponding open-label extension for our Phase 2 ACT-AD and Phase 2/3 LIFT-AD clinical trials in the prior year and a decrease in contract manufacturing costs of $7.0 million. The decrease in ATH-1105 costs of $5.5 million were driven by decreases in contract research organization, other third party vendors and clinical site visit costs of $3.0 million following the completion of the Phase 1 study at the end of 2024 and a decrease in research and development costs of $1.6 million.

General and Administrative Expenses

General and administrative expenses decreased by $9.4 million, from $26.1 million for the year ended December 31, 2024 to $16.7 million for the year ended December 31, 2025. The decrease was primarily due to a decrease in professional services expenses of $5 million, and a decrease in personnel-related expenses of $7.1 million as we operated in 2025 with fewer employees than 2024 as we pursued our strategic alternative review process.

Legal Expense

In connection with the Department of Justice investigation, we recorded a legal expense of $4.1 million during the year ended December 31, 2024. No additional legal expense was incurred in 2025 related to this matter.

Other Income, Net

Other income, net, decreased by $2.7 million, from $4.0 million for the year ended December 31, 2024 to $1.2 million for the year ended December 31, 2025 due to lower income from accretion of discounts on debt securities purchased below par value and held to maturity and lower interest income earned on our available-for-sale securities. These decreases resulted from lower balances of available-for-sale securities held during the year ended December 31, 2025 compared to the year ended December 31, 2024.

Sermonix Pre-Funded Warrant Change in Fair Value

Sermonix pre-funded warrant change in fair value increased by $4.6 million, from $0 million for the year ended December 31, 2024 to $4.6 million for the year ended December 31, 2025. Refer to Note 4 to our consolidated financial statements included elsewhere in this report for further discussion.

Liquidity and Capital Resources

Sources of Liquidity

Since our inception, we have funded our operations primarily with proceeds from the sale and issuance of common stock, convertible preferred stock, common stock warrants, prefunded common stock warrants and convertible notes, and to a lesser extent from grant income and stock option exercises. From our inception through December 31, 2025, we have raised aggregate net cash proceeds of approximately $489.8 million primarily from the issuance of our common stock (excluding option exercises), convertible preferred stock, common stock warrants, prefunded common stock warrants and convertible notes, and as of December 31, 2025, we had $88.3 million in cash, cash equivalents and investments.

On December 18, 2025, we entered into the PIPE Securities Purchase Agreement with a select group of investors, including Commodore, TCGX, Perceptive and other accredited investors, one of which is affiliated with a member of our board of directors, pursuant to which the PIPE Purchasers agreed to purchase and we agreed to issue and sell, by way of the Private Placement, an aggregate of (a) 5,356,547 shares (the “PIPE Initial Shares”) of our common stock and (b) pre-funded warrants (the “PIPE Pre-Funded Warrants”) to purchase 8,816,684 shares of our common stock (the “PIPE Pre-Funded Warrant Shares”) and (c) accompanying warrants (the “PIPE Series A Common Warrants”) to purchase 23,031,494 shares of our common stock and/or shares underlying pre-funded warrants, representing 162.5% of the aggregate of PIPE Initial Shares and the shares of our common stock underlying the PIPE Pre-Funded Warrants (the shares, or the shares issuable pursuant to such pre-funded warrants, the “PIPE Series A Common Warrant Shares”) and (d) accompanying warrants (the “PIPE Series B Common Warrants” and, together with the PIPE Series A Common Warrants and the PIPE Pre-Funded Warrants, the “PIPE Warrants”, and the PIPE Warrants, together with the PIPE Initial Shares, the “PIPE Securities”) to purchase 21,259,842 shares of our common stock and/or shares underlying pre-funded warrants, representing 150% of the aggregate of PIPE Initial Shares and the shares of our common stock underlying the PIPE Pre-Funded Warrants (the shares, or the shares issuable pursuant to such pre-funded warrants, the “PIPE Series B Common Warrant Shares” and, together with the PIPE Initial Shares, the PIPE Pre-Funded Warrant Shares and the PIPE Series A Common Warrant Shares, the “PIPE Shares”). The purchase price for each PIPE Initial Share (including the accompanying PIPE Series A Common Warrants and PIPE Series B Common Warrants) was $6.35 and the purchase price for each PIPE Pre-Funded Warrant (including the accompanying PIPE Series A Common Warrants and PIPE Series B Common Warrants) was $6.349 per each underlying PIPE Pre-Funded Warrant Share. Gross proceeds from the Private Placement were approximately $90 million, excluding placement agent fees and offering expenses. The closing of the Private Placement (the “Private Placement Closing”) occurred on December 23, 2025.

At the Private Placement Closing, we entered into a registration rights agreement (the "PIPE Registration Rights Agreement") with the PIPE Purchasers pursuant to which we are required to prepare and file a registration statement with the SEC to register the resale of the PIPE Shares within 30 calendar days after the date of the Private Placement Closing, and to use reasonable best efforts to have the registration statement declared effective at the earliest possible date but no later than the earlier of (a) 75 calendar days after the filing date if the SEC notifies us that it will review the registration statement and (b) the fifth business day after we are notified by the SEC that the registration statement will not be reviewed or will not be subject to further review. If we fail to meet certain of the requirements in the PIPE Registration Rights Agreement following the Private Placement Closing, we will pay liquidated damages to the PIPE Purchasers as provided in the PIPE Registration Rights Agreement.

The PIPE Pre-Funded Warrants are exercisable at an exercise price of $0.001 per share, subject to customary adjustments under the terms thereof and are exercisable at any time, subject to the restriction discussed below. The PIPE Series A Common Warrants have an exercise price of $6.35 per share to be paid in cash (unless a resale registration statement is unavailable at the time of exercise, in which case the PIPE Series A Common Warrants will be exercisable on a cashless net exercise basis), subject to adjustments as provided therein, and will be exercisable, subject to the restrictions discussed below, after the earlier of (1) the latest of (a) June 30, 2026, (b) the date on which we publicly announce, by means of a widely disseminated press release or a Current Report on Form 8-K, the enrollment of the 500th subject or the last subject, whichever is earlier, in the ELAINE-3 trial; provided that the total enrollment will be no less than 500 subjects unless the study's Data Safety Monitoring Board recommends stopping enrollment at an earlier time or unless the FDA permits a different number for subject enrollment in a protocol amendment; and (c) the date on which the FDA approves or issues a complete response letter to Eli Lilly & Co.'s marketing application, including a supplement to a new drug application, for imlunestrant in combination with abemaciclib in breast cancer, and (2) October 31, 2026 (the "Series A Common Warrant Initial Exercise Date"). The PIPE Series A Common Warrants will remain exercisable until the 30th day following the Series A Common Warrant Initial Exercise Date (the "Series A Common Warrant Termination Date"), except that if the Series A Common Warrant Initial Exercise Date has not occurred by December 23, 2030, then December 23, 2030 will be the Series A Common Warrant Termination Date. The PIPE Series B Common Warrants have an exercise price of $7.62 per share payable on a cashless net exercise basis, subject to adjustments as provided therein, and will be exercisable, subject to the restrictions discussed below, after the later of (1) June 30, 2026 and (2) the date of the completion of the public readout of topline results of the ELAINE-3 trial (the later of (1) and (2), the "Series B Common Warrant Initial Exercise Date"). The PIPE Series B Common Warrants will remain exercisable until the 30th day following the Series B Common Warrant Initial Exercise Date (the "Series B Common Warrant Termination Date"), except that if the Series B Common Warrant Initial Exercise Date has not occurred by December 23, 2030, then December 23, 2030 will be the Series B Common Warrant Termination Date.

None of the PIPE Pre-Funded Warrants, the PIPE Series A Common Warrants or the PIPE Series B Common Warrants can be exercised if, immediately prior to or following such exercise, the applicable PIPE Purchaser, together with its affiliates and any other persons whose beneficial ownership of shares of our common stock would be aggregated with the PIPE Purchaser for purposes of Section 13(d) of the Exchange Act, would beneficially own more than 4.99%, 9.99% or 19.99%, at the election of the PIPE Purchaser (in each case, the "PIPE Maximum Percentage") of the total number of issued and outstanding shares of our common stock following such exercise. If, upon exercise of the PIPE Series A Common Warrants or PIPE Series B Common Warrants, a PIPE Purchaser would exceed the PIPE Maximum Percentage, then the PIPE Series A Common Warrants and PIPE Series B Common Warrants may be exercised for pre-funded warrants to purchase shares of our common stock, which pre-funded warrants will have terms substantially the same as the PIPE Pre-Funded Warrants. The PIPE Maximum Percentage may be increased or decreased by a PIPE Purchaser with 61 days' written notice to us; provided, however, that such percentage may in no event exceed (x) 19.99% prior to a vote of our stockholders approving the removal of such exercise limitation or (y) with respect to certain PIPE Purchasers, 4.99%.

We will use the net proceeds from the Private Placement for working capital and will not use such proceeds for (1) the repayment of borrowed debt, (2) to redeem any shares of our common stock or any of our securities that would entitle the holder thereof to acquire at any time shares of our common stock, subject to certain exceptions, (3) for the settlement of any litigation that is outstanding as of the Private Placement Closing, and (4) to in-license or develop a drug candidate other than lasofoxifene or any drug or drug candidate in our pipeline as of the Private Placement Closing until the earlier of such time as the topline results of ELAINE-3 become available and the second anniversary of the effective date of the PIPE Securities Purchase Agreement.

Since our inception, we have not generated positive cash flows from operations and we have devoted substantially all of our resources to our research and development efforts such as small molecule compound discovery, nonclinical studies and clinical trials, as well as manufacturing activities, establishing and maintaining our intellectual property portfolio, hiring personnel, raising capital, and providing general and administrative support for these operations.

Material Cash and Future Funding Requirements

Our material cash requirements include our operating leases for laboratory and office facilities. As of December 31, 2025, we had lease payment obligations of $0.9 million, with $0.5 million payable within 12 months. For additional information regarding our lease commitments, see Note 7 to our consolidated financial statements included elsewhere in this report. Additionally, we have purchase obligations and open purchase orders that support normal operations and are primarily due in the next 12 months. We also have payment obligations to certain third-party service providers that total approximately $4.0 million, that are primarily due in the near term. These purchase obligations and open purchase orders are generally cancellable in full or in part through the contractual provisions. We expect to continue to incur increased research and development expenses for the foreseeable future as we continue to engage in research and development activities related to developing our drug candidates, including lasofoxifene and ATH-1105, our drug candidates advance into later stages of development, we conduct larger clinical trials, we seek regulatory approvals for any drug candidates that successfully complete clinical trials, we expand or advance our drug product pipeline, we maintain, expand, protect and enforce our intellectual property portfolio, and we incur expenses associated with hiring or retaining personnel to support our research and development efforts We expect to continue to incur increased general and administrative expenses for the foreseeable future as we support our continued research activities and development of our programs.

Based upon our current operating plan, we estimate that our $88.3 million of cash, cash equivalents and investments at December 31, 2025 will be sufficient to fund our operating expenses and capital expenditure requirements through at least the next 12 months following the date of this report. We will need to raise substantial additional capital to fund the development of our drug candidates. Until such time as we can generate significant revenue from drug product sales, we expect to finance our operations through the sale of equity securities, debt financings, or other capital, which could include income from collaboration, licensing or similar arrangements with third parties, or receiving research contributions, or grants. For example, in January 2023, we entered into a sales agreement with Cantor Fitzgerald and BTIG to sell shares of our common stock having aggregate sales proceeds of up to $75.0 million, from time to time, subject to any applicable limitations on sales pursuant to SEC rules and regulations, through an ATM equity offering program under which Cantor Fitzgerald and BTIG are acting as sales agents. As of the date of this report, we have not sold any securities pursuant to this ATM offering. 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 or could be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Debt financing and preferred 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 funds through collaborations, licenses and other similar arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or drug candidates or grant licenses on terms that may not be favorable to us or may reduce the value of our common stock. Adequate funding may not be available

when needed or on terms acceptable to us, or at all. Our ability to raise additional funds may be negatively impacted by potential adverse global economic conditions and disruptions to, and volatility in, the credit and financial markets in the United States and worldwide. If we fail to obtain necessary capital when needed on acceptable terms, or at all, it could force us to delay, limit, reduce or terminate our drug product development programs, commercialization efforts or other operations. Insufficient liquidity may also require us to relinquish rights to drug candidates at an earlier stage of development or on less favorable terms than we would otherwise choose. We cannot assure you that we will ever be profitable or generate positive cash flows from operating activities.

We have based our projections of operating capital requirements on assumptions that may prove to be incorrect and we may use all our available capital resources sooner than we expect. Because of the numerous risks and uncertainties associated with research, development and commercialization of biotechnology products, we are unable to estimate the exact amount of our operating capital requirements. Our future funding requirements will depend on many factors, including, but not limited to:

- the scope, timing, progress and results of our ongoing preclinical studies and clinical trials of our drug candidates;
- the number of trials required for regulatory approval;
- the willingness of the FDA, EMA and any other regulatory agencies to accept lasofoxifene or ATH-1105 clinical trial data, as well as data from any completed and planned clinical and nonclinical studies and other work, as the basis for review and approval of lasofoxifene for breast cancer and ATH-1105 for ALS, and the potential need for additional clinical trials;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials of other drug candidates that we may pursue;
- our ability to establish and maintain collaborations, licensing or other similar arrangements, and the financial terms of any such arrangements, including the timing and amount of any future milestone, royalty or other payments due thereunder;
- the costs, timing and outcome of regulatory review of our drug candidates;
- the costs and timing of future commercialization activities, including drug product manufacturing, marketing, sales and distribution, for any of our drug candidates for which we receive marketing approval;
- the revenue, if any, received from commercial sales of our drug 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;
- the costs related to any legal proceedings;
- any expenses needed to attract, hire and retain skilled personnel;
- the costs of operating as a public company;
- the costs associated with any expansion of our laboratory and office facilities; and
- the extent to which we acquire or in-license other companies' product candidates and technologies or engage in other strategic transactions.

A change in the outcome of any of these or other factors with respect to the development of any of our drug candidates could significantly change the costs and timing associated with the development of that drug candidate. Furthermore, our operating plan may change in the future, and we may need additional funds to meet operational needs and capital requirements associated with such operating plan.

Cash Flows

The following table summarizes our cash flows for the periods indicated:

	Year Ended December 31,	
	2025	2024
	(in thousands)	
Net cash (used in) provided by:		
Operating activities	$ (45,729)	$ (97,170)
Investing activities	(15,894)	54,830
Financing activities	82,461	194
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 20,838	$ (42,146)

Operating Activities

During the year ended December 31, 2025, net cash used in operating activities was $45.7 million. This consisted primarily of a net loss of $105.6 million, offset by non-cash charges of $79.3 million and a decrease in our net operating assets of $19.4 million. The non-cash charges primarily consisted of acquired in-process research and development, stock-based compensation expense, depreciation expense, and amortization of premiums and accretion of discounts on our available-for-sale securities. The decrease in our net operating assets was primarily due to a decrease in accrued liabilities and payment of Sermonix assumed liabilities.

During the year ended December 31, 2024, net cash used in operating activities was $97.2 million. This consisted primarily of a net loss of $96.9 million, partially offset by non-cash charges of $11.7 million and an increase in our net operating assets of $12.0 million. The non-cash charges primarily consisted of stock-based compensation expense, depreciation expense, and amortization of premiums and accretion of discounts on our available-for-sale securities. The increase in our net operating assets was primarily due to a decrease in the accrual for legal settlement expenses related to the securities class action litigation and a net decrease in accounts payable and accrued expenses, partially offset by decreases in prepaid expenses and other current and long-term assets, and the insurance recovery receivable related to the securities class action litigation.

Investing Activities

During the year ended December 31, 2025, net cash used in investing was $15.9 million. This consisted of purchases of available-for-sale securities of $38 million, partially offset by maturities of available-for-sale securities of $22 million.

During the year ended December 31, 2024, net cash provided by investing was $54.8 million. This consisted of maturities of available-for-sale securities of $69.0 million, partially offset by purchases of available-for-sale securities of $14.1 million and property and equipment of less than $0.1 million.

Financing Activities

During the year Ended December 31, 2025, net cash provided by financing activities was $82.5 million, consisting primarily of net proceeds from the Private Placement financing.

During the year ended December 31, 2024, net cash provided by financing activities was $0.2 million, consisting of proceeds received from participation in the Company's employee stock purchase plan and exercises of stock options.

Critical Accounting Policies, Significant Judgments and Use of Estimates

Our financial statements have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenue and expenses incurred during the reporting periods. Our estimates are based on our historical experience 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 value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. We believe that the accounting policies discussed below are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving management's judgments and estimates.

Research and Development Costs

Research and development expenses consist primarily of direct and indirect costs incurred for research activities, including development of the pipeline from the Company's drug discovery efforts and the development of its drug candidates. Direct costs include laboratory materials and supplies, contracted research and manufacturing, clinical trial costs, consulting fees, and other expenses incurred to sustain the Company's research and development program. Indirect costs include personnel-related expenses, consisting of employee salaries, related benefits, and stock-based compensation expense for employees engaged in research and development activities, and facilities and other expenses consisting of direct and allocated expenses for rent and depreciation and lab consumables.

Research and development costs, including costs associated with our clinical trials, are expensed as incurred. In-licensing fees and other costs to acquire technologies used in research and development that have not yet received regulatory approval and that are not expected to have an alternative future use are expensed when incurred. Non-refundable advance payments for goods and services that will be used over time for research and development are capitalized and recognized as goods are delivered or as the related services are performed. We estimate the period over which such services will be performed and the level of effort to be expended in each period. If actual timing of performance or the level of effort varies from the estimate, we will adjust the amounts recorded accordingly. We have not experienced any material differences between accrued or prepaid costs and actual costs since inception.

Acquired in-process research and development costs represent amounts incurred to acquire externally developed in-process research and development ("IPR&D") projects in transactions other than business combinations when the acquired assets have no alternative future use. These costs are expensed as incurred. Amounts included in acquired in-process research and development consist of upfront cash payments, equity instruments issued as consideration, contingent consideration that meets the criteria for recognition, and liabilities incurred to acquire the rights to the underlying IPR&D projects.

In connection with the asset acquisition from Sermonix in December 2025, we recorded $68.1 million of acquired IPR&D expense, consisting of various components of consideration measured at fair value on the acquisition date. Included in this amount were the Sermonix Pre-Funded Warrant, valued at $32.9 million, and a liability-classified contingent milestone payment, valued at $15.1 million. These instruments required significant estimates and judgment in determining their respective fair values on the acquisition date and at December 31, 2025, due to the use of unobservable inputs and are classified in Level 3 of the fair value hierarchy.

The Sermonix Pre-Funded Warrant was redeemable prior to obtaining the Sermonix Stockholder Approval and as a result, is classified as a liability and measured at fair value at the acquisition date and each subsequent reporting period, with changes recognized in earnings. We estimate fair value with reference to the market value of our common stock on the measurement date, adjusted for a discount for lack of marketability (DLOM) to reflect the inability to exercise until stockholder approval is obtained, which exposes the holder to interim changes in our stock price. In selecting the DLOM, we consider the expected time to stockholder approval, estimated volatility of our common stock price, and the risk-free rate. The Sermonix Stockholder Approval was obtained on March 18, 2026 and, as a result, the Sermonix Pre-Funded Warrant is no longer redeemable.

The valuation of the Sermonix Pre-Funded Warrant requires significant judgment and is sensitive to movements in our stock price and to assumptions used in determining the DLOM. Different reasonable assumptions could have resulted in a materially different amount of acquired IPR&D expense at the acquisition date, as well as gains or losses recognized in our consolidated statement of operations and comprehensive loss upon recurring fair value remeasurement.

The first contingent milestone payment due to Sermonix under the agreement may be settled in cash or a variable number of shares equal to a fixed monetary amount and is therefore classified as a liability and measured at fair value at the acquisition date and each subsequent reporting period, with changes recognized in earnings. We estimate the fair value using a probability-weighted present value of the expected net milestone payment, which incorporates our assumptions regarding the cumulative probability of achieving the milestone across the remaining phases of development and approval, expected timing of the payment, and a weighted average cost of capital to discount expected cash flows.

The valuation of the milestone liability requires significant judgment and is sensitive to changes in assumed probabilities, expected timing of payment, and, to a lesser extent, the discount rate. Different reasonable assumptions could have resulted in a materially different amount of acquired IPR&D expense at the acquisition date, as well as gains or losses recognized in our consolidated statement of operations and comprehensive loss upon recurring fair value remeasurement.

Stock-based Compensation

We maintain a stock-based compensation plan as a long-term incentive for employees, non-employee directors and consultants. The plan allows for the issuance of incentive stock options, non-qualified stock options, restricted stock units, and other forms of equity awards.

We recognize stock-based compensation expense for stock options on a straight-line basis over the requisite service period and account for forfeitures as they occur. Our stock-based compensation costs for stock options are based upon the grant date fair value of options estimated using the Black-Scholes option pricing model. To the extent any stock option grants are made subject to the achievement of a performance-based milestone, management evaluates when the achievement of any such performance-based milestone is probable based on the relative satisfaction of the performance conditions as of the reporting date.

The Black-Scholes option pricing model utilizes inputs which are highly subjective assumptions and generally require significant judgment. These assumptions include:

- *Fair Value of Common Stock*. The fair value of each share of common stock is based on the closing price of the Company's common stock on the date of grant, or other relevant determination date, as reported on The Nasdaq Capital Market.
- *Risk-Free Interest Rate*. The risk-free interest rate is based on the U.S. Treasury zero coupon issues in effect at the time of grant for periods corresponding with the expected term of the option.

- *Expected Volatility*. Because we were previously privately held and did not have sufficient trading history for our common stock prior to our initial public offering, the expected volatility was estimated based on the average volatility for comparable publicly traded life sciences companies over a period equal to the expected term of the stock option grants. The comparable companies were chosen based on the similar size, stage in life cycle or area of specialty. We will continue to apply this process until a sufficient amount of historical information regarding the volatility of our own stock price becomes available.
- *Expected Term*. The expected term represents the period that the stock-based awards are expected to be outstanding and is determined using the simplified method (based on the mid-point between the vesting date and the end of the contractual term), as we have limited history of relevant stock option exercise activity.
- *Expected Dividend Yield*. We have never paid dividends on our common stock and have no plans to pay dividends on our common stock. Therefore, we used an expected dividend yield of zero.

See Note 10 to our consolidated financial statements included elsewhere in this report for more 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. Certain of such assumptions involve inherent uncertainties and the application of significant judgment. As a result, if factors or expected outcomes change and we use significantly different assumptions or estimates, our stock-based compensation could be materially different.

We recorded stock-based compensation expense of $6 million and $11.0 million for the years ended December 31, 2025 and 2024, respectively. As of December 31, 2025, there was $4.5 million of total unrecognized stock-based compensation expense related to non-vested stock options which we expect to recognize over a remaining weighted-average period of 2.49 years. We expect to continue to grant stock options and other equity-based awards in the future, and to the extent that we do, our stock-based compensation expense recognized in future periods will likely increase.

Income Taxes

We recognize deferred income taxes for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carry forwards. In evaluating our valuation allowance, we consider all available positive and negative evidence, including scheduled reversals of deferred tax liabilities, projected future taxable income, tax planning strategies, and recent financial performance. Due to our lack of earnings history and uncertainties surrounding our ability to generate future taxable income, the net deferred tax assets have been fully offset by a valuation allowance.

As of December 31, 2025, we had $9.5 million of federal NOL carryforwards and $17.2 million of tax credit carryforwards which expire over a period of 6 to 12 years. As of December 31, 2025, we had $259.8 million of such NOLs that do not expire. As of December 31, 2025, we also had state net operating loss carryforwards of $5 million, which expire over a period of 17 to 20 years.

Under Sections 382 and 383 of the Internal Revenue Code of 1986 (as amended, the "Code'), substantial changes in our ownership may limit the amount of NOL and research and development credit carryforwards that could be used annually in the future to offset taxable income. The tax benefits related to future utilization of federal and state NOL carryforwards, credit carryforwards, and other deferred tax assets may be limited or lost if cumulative changes in ownership exceeds 50% within any three-year period. We have not completed a Section 382/383 analysis under the Code regarding the limitation of NOL and credit carryforwards. If a change in ownership were to have occurred, the annual limitation may result in the expiration of NOL carryforwards and credits before utilization.

We record unrecognized tax benefits as liabilities or reduce the underlying tax attribute, as applicable, and adjust them when our judgment changes as a result of the evaluation of new information not previously available. Because of the complexity of some of these uncertainties, the ultimate resolution may result in a payment that is materially different from our current estimate of the unrecognized tax benefit liabilities. These differences will be reflected as increases or decreases to income tax expense in the period in which new information is available.

Recent Accounting Pronouncements

See Note 2 to our consolidated financial statements included elsewhere in this report for additional information.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk.

As a smaller reporting company, we are not required to provide the information requested by this item pursuant to Item 305 of Regulation S-K.

Item 8. Financial Statements and Supplementary Data.

LeonaBio, Inc.

Index to Financial Statements

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of LeonaBio, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of LeonaBio, Inc. (the Company) as of December 31, 2025 and 2024, the related consolidated statements of operations and comprehensive loss, stockholders' equity and cash flows for each of the two years in the period ended December 31, 2025, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2025 and 2024, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2025, in conformity with U.S. generally accepted accounting principles.

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.

Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current period audit of the financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective, or complex judgments. The communication of the critical audit matter does not alter in any way our opinion on the consolidated financial statements taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Fair Value of Milestone Liability — Sermonix License Transaction

Description of the Matter	As discussed in Notes 3 and 4 to the consolidated financial statements, in connection with the Company's December 2025 license agreement with Sermonix Pharmaceuticals, Inc. ("Sermonix"), the Company recognized a

	milestone liability for its contingent obligation to pay Sermonix $50 million in cash or shares upon the first U.S. commercial sale of a licensed product. Auditing the fair value measurement of the milestone liability was challenging due to the significant estimation uncertainty and judgment in determining the probability of milestone achievement.
How We Addressed the Matter in Our Audit	To test the estimated fair value of the milestone liability, we performed audit procedures that included, among others, involving our valuation specialists to assist in evaluating the Company's use of the selected valuation model. In addition, we compared the probability of achievement to published clinical development success-rate data for oncology compounds at comparable stage of development. We also performed a sensitivity analysis of the probability of milestone achievement to evaluate the change in the fair value of the milestone liability resulting from changes in the probability of milestone achievement.

/s/ Ernst & Young LLP

We have served as the Company’s auditor since 2018.

Seattle, Washington
March 31, 2026

LeonaBio, Inc.
Consolidated Balance Sheets
(in thousands, except share and per share amounts)

	December 31, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 69,276	$ 48,438
Short-term investments	19,055	2,837
Prepaid expenses and other current assets	1,127	3,566
Total current assets	89,458	54,841
Restricted cash	631	631
Property and equipment, net	1,472	2,444
Operating lease right-of-use asset	535	808
Other long-term assets	55	55
Total assets	$ 92,151	$ 58,779
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 4,587	$ 319
Accrued liabilities	4,963	12,402
Sermonix pre-funded warrant	37,488	—
Current operating lease liability	465	414
Total current liabilities	$ 47,503	$ 13,135
Operating lease liability, less current portion	338	803
Milestone liability	15,116	—
Other long-term liabilities	1,404	—
Total liabilities	$ 64,361	$ 13,938
Stockholders' equity:		
Common stock, $0.0001 par value; 90,000,000 shares authorized at December 31, 2025 and December 31, 2024, respectively; 9,335,913 and 3,904,049 shares issued and outstanding at December 31, 2025 and December 31, 2024, respectively (1)	1	—
Additional paid-in capital	539,548	450,986
Accumulated other comprehensive (loss) income	(4)	1
Accumulated deficit	(511,755)	(406,146)
Total stockholders' equity	27,790	44,841
Total liabilities and stockholders' equity	$ 92,151	$ 58,779

The accompanying notes are an integral part of these consolidated financial statements.

(1) *The Company effected a reverse stock split of its outstanding shares of common stock on September 17, 2025 where every ten shares of its common stock issued and outstanding was converted into one share of common stock. Any fractional post-split shares as a result of the reverse stock split were rounded down to the nearest whole post-split share. Stockholders of the Company previously authorized the Board of Directors to approve a reverse stock split at the Company's annual meeting on May 29, 2025. All share amounts and per share amounts disclosed in this Annual Report on Form 10-K have been restated to reflect the reverse stock split on a retroactive basis in all periods presented.*

LeonaBio, Inc.
Consolidated Statements of Operations and Comprehensive Loss
(in thousands, except share and per share amounts)

	Year Ended December 31,	
	2025	2024
Operating expenses:		
Research and development	$ 17,500	$ 70,682
Acquired in-process research and development	68,088	$ —
General and administrative	16,678	26,093
Legal expense	—	4,127
Total operating expenses	102,266	100,902
Loss from operations	(102,266)	(100,902)
Other income, net	1,236	3,962
Sermonix pre-funded warrant change in fair value	(4,579)	—
Net loss	$ (105,609)	$ (96,940)
Unrealized (loss) gain on available-for-sale securities	(5)	350
Comprehensive loss attributable to common stockholders	$ (105,614)	$ (96,590)
Net loss per share attributable to common stockholders, basic and diluted	$ (24.70)	$ (25.19)
Weighted-average shares used in computing net loss per share attributable to common stockholders, basic and diluted (1)	4,275,762	3,848,044

The accompanying notes are an integral part of these consolidated financial statements.

(1) The Company effected a reverse stock split of its outstanding shares of common stock on September 17, 2025 where every ten shares of its common stock issued and outstanding was converted into one share of common stock. Any fractional post-split shares as a result of the reverse stock split were rounded down to the nearest whole post-split share. Stockholders of the Company previously authorized the Board of Directors to approve a reverse stock split at the Company's annual meeting on May 29, 2025. All share amounts and per share amounts disclosed in this Annual Report on Form 10-K have been restated to reflect the reverse stock split on a retroactive basis in all periods presented.

LeonaBio, Inc.
Consolidated Statements of Stockholders' Equity
(in thousands, except share amounts)

	Common Stock(1)		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Income (Loss)	Deficit	Equity
Balance as of January 1, 2024	3,817,216	$ —	$ 439,743	$ (349)	$ (309,206)	$ 130,188
Issuance of common stock upon exercise of common stock options	7,566	—	12	—	—	12
Issuance of common stock upon vesting of restricted stock units	62,066	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	17,201	—	182	—	—	182
Stock-based compensation	—	—	11,049	—	—	11,049
Unrealized gain on available-for-sale securities	—	—	—	350	—	350
Net loss	—	—	—	—	(96,940)	(96,940)
Balance as of December 31, 2024	3,904,049	$ -	$ 450,986	$ 1	$ (406,146)	$ 44,841
Proceeds from PIPE financing, net of underwriters' discounts and commissions	5,356,547	1	82,423	—	—	82,424
Issuance of common stock upon exercise of common stock options	—		—	—	—	—
Issuance of common stock upon vesting of restricted stock units	59,186	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	16,131	—	37	—	—	37
Stock-based compensation	—	—	6,102	—	—	6,102
Unrealized gain on available-for-sale securities	—	—	—	(5)	—	(5)
Net loss	—	—	—	—	(105,609)	(105,609)
Balance as of December 31, 2025	9,335,913	$ 1	$ 539,548	$ (4)	$ (511,755)	$ 27,790

The accompanying notes are an integral part of these consolidated financial statements.

(1) The Company effected a reverse stock split of its outstanding shares of common stock on September 17, 2025 where every ten shares of its common stock issued and outstanding was converted into one share of common stock. Any fractional post-split shares as a result of the reverse stock split were rounded down to the nearest whole post-split share. Stockholders of the Company previously authorized

the Board of Directors to approve a reverse stock split at the Company's annual meeting on May 29, 2025. All share amounts and per share amounts disclosed in this Annual Report on Form 10-K have been restated to reflect the reverse stock split on a retroactive basis in all periods presented.

LeonaBio, Inc.
Consolidated Statements of Cash Flows
(in thousands)

	Year Ended December 31,	
	2025	2024
Operating activities		
Net loss	$ (105,609)	$ (96,940)
Adjustments to reconcile net loss to net cash used in operating activities:		
Non-cash consideration for acquired in-process research and development	67,659	—
Change in fair value of Sermonix pre-funded warrant	4,579	—
Stock-based compensation	6,102	11,049
Depreciation expense	972	970
Non-cash lease expense	273	241
Amortization of premiums and accretion of discounts on available-for-sale securities, net	(329)	(515)
Loss on disposal of equipment	—	7
Changes in operating assets and liabilities:		
Prepaid expenses and other current and long-term assets, net	2,439	2,509
Insurance recovery receivable related to legal settlement	—	1,628
Accounts payable and accrued liabilities	(21,342)	(5,751)
Accrued legal settlement	—	(10,000)
Operating lease liability	(414)	(368)
Other long-term liabilities	(59)	—
Net cash used in operating activities	(45,729)	(97,170)
Investing activities		
Purchases of available-for-sale securities	(37,643)	(14,134)
Maturities of available-for-sale securities	21,749	68,997
Purchases of property and equipment	—	(33)
Net cash (used in) provided by investing activities	(15,894)	54,830
Financing activities		
Proceeds from exercise of common stock options and issuance of common stock under employee stock purchase plan	37	194
Proceeds from PIPE financing	89,991	—
Issuance costs from PIPE financing	(7,567)	—
Net cash provided by financing activities	82,461	194
Net increase (decrease) in cash, cash equivalents and restricted cash	20,838	(42,146)
Cash, cash equivalents and restricted cash, beginning of period	49,069	91,215
Cash, cash equivalents and restricted cash, end of period	$ 69,907	$ 49,069

The accompanying notes are an integral part of these consolidated financial statements.

LeonaBio, Inc.
Notes to Consolidated Financial Statements

1. Description of Business

Organization

LeonaBio, Inc. (the "Company") was incorporated as M3 Biotechnology, Inc. in the state of Washington on March 31, 2011 and reincorporated in the state of Delaware on October 27, 2015. In April 2019, the Company changed its name to Athira Pharma, Inc and in January 2026 the Company changed its name to LeonaBio, Inc. The Company currently has office and laboratory space in Bothell, Washington. The Company is a clinical-stage biopharmaceutical company dedicated to the development of novel therapeutics for high unmet medical needs, including treatment-resistant metastatic breast cancer and amyotrophic lateral sclerosis.

Liquidity and Capital Resources

Since the Company's inception, it has funded its operations primarily with proceeds from the sale and issuance of common stock, convertible preferred stock, common stock warrants, prefunded common stock warrants, and convertible notes, and to a lesser extent from grant income and stock option exercises. From the Company's inception through December 31, 2025, it has raised aggregate net cash proceeds of $489.8 million primarily from the issuance of its common stock (excluding option exercises), convertible preferred stock, common stock warrants, prefunded common stock warrants and convertible notes. In December 2025, the Company raised approximately $90 million in a Private Placement (as defined below), excluding placement agent fees and offering expenses. See Note 9 for more information.

As of December 31, 2025, the Company had cash, cash equivalents and investments of $88.3 million. The Company's net loss for the year ended December 31, 2025 was $105.6 million and cash used in operations for the year ended December 31, 2025 was $45.7 million. Since the Company's inception, it has devoted substantially all of its resources to its research and development efforts such as small molecule compound discovery, nonclinical studies and clinical trials, as well as manufacturing activities, establishing and maintaining the Company's intellectual property portfolio, hiring personnel, raising capital, and providing general and administrative support for these operations.

Based upon the Company's current operating plan, it estimates that its $88.3 million of cash, cash equivalents and investments at December 31, 2025 will be sufficient to fund its operating expenses and capital expenditure requirements through at least the next 12 months following the date of the Company's Annual Report on Form 10-K. Historically, the Company has incurred net losses from continuing operations and negative operating cash flows. The Company has not yet established an ongoing source of revenue sufficient to cover its operating costs; therefore, the Company expects it will need to continue to raise additional capital to accomplish its operating plan. The Company has a sales agreement in place with Cantor Fitzgerald & Co. and BTIG, LLC for an "at the market" equity offering facility through which it may offer and sell shares of its common stock, however, the Company is currently unable to offer and sell shares under this facility due to its ineligibility to use a Registration Statement on Form S-3 through December 2026. The Company has not sold any securities pursuant to this ATM offering. Should it be determined to be strategically advantageous, the Company could pursue public and private offerings of its equity securities similar to those the Company has previously completed, debt financings, or other strategic transactions, which could include income from collaboration, licensing or similar arrangements with third parties, or receiving research contributions, or grants.

2. Significant Accounting Policies

Basis of Presentation

The consolidated financial statements have been prepared in conformity with U.S. generally accepted accounting principles ("U.S. GAAP"). The consolidated financial statements include the operations of LeonaBio, Inc., and its wholly owned Australian subsidiary. All intercompany balances and transactions have been eliminated upon consolidation.

The Company effected a reverse stock split on September 17, 2025 of its outstanding shares of common stock at a ratio of 1-for-10 pursuant to a Certificate of Amendment to the Company's Certificate of Incorporation filed with the Secretary of State of the State of Delaware (the "reverse stock split"). The reverse stock split was reflected on the Nasdaq Capital Market beginning with the opening of trading on September 18, 2025. The reverse stock split did not change the par value of the Company's common stock. The reverse stock split reduced the total number of authorized number of shares of the Company's common stock from 900,000,000 to 90,000,000 and the total number of authorized shares of the Company's capital from 1,000,000,000 to 190,000,000. All share amounts and per share amounts disclosed in this Annual Report on Form 10-K have been restated to reflect the reverse stock split on a retroactive basis in all periods presented.

Fair Value Measurements

The Company has certain assets and liabilities that are measured at fair value on a recurring basis according to a fair value hierarchy that prioritizes the inputs, assumptions and valuation techniques used to measure fair value. The three levels of the fair value hierarchy are:

Level 1—Unadjusted quoted prices in active markets that are accessible at the measurement date for identical, unrestricted assets or liabilities.

Level 2—Quoted prices in markets that are not active or financial instruments for which all significant inputs are observable, either directly or indirectly.

Level 3—Inputs are generally unobservable and reflect management's estimates of assumptions that market participants would use in pricing the asset or liability. The fair values are determined using model-based techniques, including probability-based simulation methodologies.

The determination of a financial instrument's level within the fair value hierarchy is based on an assessment of the lowest level of any input that is significant to the fair value measurement. The Company considers observable data to be market data, which is readily available, regularly distributed or updated, reliable and verifiable, not proprietary, and provided by independent sources that are actively involved in the relevant market.

The carrying amounts of certain financial instruments, including cash, cash equivalents, restricted cash, investments, accounts payable and accrued expenses approximate their fair values due to the short-term nature of those amounts.

Cash, cash equivalents and restricted cash

Cash and cash equivalents have a maturity date of less than three months to maturity when acquired by the Company. Restricted cash consists of collateral pledged in connection with the Company's corporate credit cards. The table below reconciles the balances of cash and cash equivalents

and restricted cash reported on the consolidated balance sheets to the balances of cash, cash equivalents and restricted cash reported on the consolidated statements of cash flows.

	December 31, 2025	December 31, 2024
Cash and cash equivalents	$ 69,276	$ 48,438
Restricted cash	631	631
Cash, cash equivalents and restricted cash	$ 69,907	$ 49,069

Short-term Investments

The Company generally invests its excess cash in investment grade short- to intermediate-term fixed income securities. Such investments are included in cash and cash equivalents, and short-term investments on the consolidated balance sheets, classified as available-for-sale, and reported at fair value with unrealized gains and losses included in accumulated other comprehensive income (loss). Amortization and accretion are included in other income, net. Realized gains and losses on the sale of these securities are recognized in other income, net.

The Company periodically evaluates whether declines in fair values of its investments below their book value are due to expected credit losses, as well as the Company's ability and intent to hold the investment until a forecasted recovery occurs. Expected credit losses are recorded as an allowance through other income, net.

Warrants

The Company accounts for warrants as either equity-classified or liability-classified instruments based on an assessment of the warrant's specific terms and applicable authoritative guidance in Accounting Standards Codification ("ASC") 480 and ASC 815. This assessment is conducted at the time the warrants are issued and as of each subsequent reporting period while the warrants are outstanding.

For warrants that meet all criteria for equity classification, the warrants are required to be recorded at their initial fair value at the time of issuance, as a component of additional paid-in capital, on the consolidated statement of stockholders' equity. For warrants that do not meet all the criteria for equity classification, the warrants are required to be recorded at their initial fair value on the date of issuance, and on each balance sheet date thereafter. Changes in the estimated fair value of the warrants are recorded as a non-cash gain or loss on the consolidated statements of operations and comprehensive loss.

Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. Estimates include those used for fair value of assets and liabilities, fair value of warrant liabilities, fair value of milestone liability, accrued liabilities, valuation allowance for deferred tax assets, and stock-based compensation. Management evaluates related assumptions on an ongoing basis using historical experience and other factors and adjusts those estimates and assumptions when facts and circumstances dictate. Actual results could differ from those estimates.

Research and Development Expenses

Research and development expenses consist primarily of direct and indirect costs incurred for research activities, including development of the pipeline from the Company's drug discovery efforts and the development of its drug candidates. Direct costs include laboratory materials and supplies, contracted research and manufacturing, clinical trial costs, consulting fees, and other expenses incurred to sustain the Company's research and development program. Indirect costs include personnel-related expenses,

consisting of employee salaries, related benefits, and stock-based compensation expense for employees engaged in research and development activities, and facilities and other expenses consisting of direct and allocated expenses for rent and depreciation and lab consumables.

Research and development costs are expensed as incurred. In-licensing fees and other costs to acquire technologies used in research and development that have not yet received regulatory approval and that are not expected to have an alternative future use are expensed when incurred. Non-refundable advance payments for goods and services that will be used over time for research and development are capitalized and recognized as goods are delivered or as the related services are performed. The Company estimates the period over which such services will be performed and the level of effort to be expended in each period. If actual timing of performance or the level of effort varies from the estimate, the Company adjusts the amounts recorded accordingly. The Company has not experienced any material differences between accrued or prepaid costs and actual costs since inception.

Acquired in-process research and development costs represent amounts incurred to acquire externally developed in-process research and development ("IPR&D") projects in transactions other than business combinations when the acquired assets have no alternative future use. These costs are expensed as incurred. Amounts included in acquired in-process research and development consist of upfront cash payments, equity instruments issued as consideration, contingent consideration that meets the criteria for recognition, and liabilities incurred to acquire the rights to the underlying IPR&D projects.

Asset acquisitions

The Company measures and recognizes asset acquisitions that are not deemed to be business combinations based on the cost to acquire the assets, which includes transaction costs, and the consideration is allocated to the items acquired based on a relative fair value methodology. Goodwill is not recognized in asset acquisitions. In an asset acquisition, the cost allocated to acquire in-process research and development with no alternative future use is charged to acquired in-process research and development at the acquisition date.

Contingent consideration arrangements

In connection with certain asset acquisitions, the Company may agree to make certain milestone payments to the owners of licensed technology acquired upon the achievement of certain development, clinical and commercial milestones. These obligations may be settled in cash or in the Company's equity shares.

Contingent consideration payable in cash is first evaluated under ASC 815 to determine whether the arrangement meets the definition of a derivative. If the contingent payment arrangement does not qualify as a derivative under ASC 815, the Company applies the guidance in ASC 450, *Contingencies*. Under this model, contingent consideration is not included in the cost of the assets acquired until the contingency is resolved. A liability is recognized only when the contingent payment is probable and reasonably estimable.

Contingent consideration that is required or may be settled in the Company's equity shares is evaluated under ASC 480, *Distinguishing Liabilities from Equity*, to determine proper classification. Obligations that require or may require the Company to issue a variable number of shares with a monetary value that is fixed, indexed to something other than the Company's own shares, or based on an obligation to repurchase shares are classified as liabilities under ASC 480.

Contingent consideration arrangements classified as liabilities under ASC 480 are carried at fair value. The Company estimates the fair value by applying a probability-based model that utilizes inputs primarily based upon the achievement and related timing of certain development, clinical and commercial milestones that were unobservable in the market. The estimated fair value of contingent consideration liabilities, initially measured and recorded on the acquisition date, are considered to be a Level 3 fair value measurement and are reviewed quarterly, or whenever events or circumstances occur that indicate a change in fair value. The contingent consideration liabilities are recorded at fair value at the end of each

reporting period with changes in estimated fair values recorded in other income (expense) in the consolidated statements of operations and other comprehensive loss.

Significant changes in any of the probabilities of success or in the probabilities as to the periods in which milestones would be achieved could result in a significantly higher or lower fair value measurement. The Company will continue to adjust the liabilities for changes in fair value until the earlier of the achievement or expiration of the obligations.

General and Administrative Expenses

General and administrative expenses consist primarily of personnel-related costs, consisting of employee salaries, related benefits, and stock-based compensation expense for employees in the executive, legal, finance and accounting, human resources, and other administrative functions. General and administrative expenses also include third-party costs such as legal costs, insurance costs, accounting, auditing and tax related fees, consulting fees and facilities and other expenses not otherwise included as research and development expenses. General and administrative costs are expensed as incurred.

Leases

The Company adopted Accounting Standards Codification, ("ASC"), *Topic 842 – Leases* effective January 1, 2020. The Company determines if an arrangement contains a lease at inception. The Company performed an evaluation of contracts in accordance with ASC 842 and has determined it has an operating lease agreement for the laboratory and office facilities that the Company occupies. Operating lease right-of-use ("ROU") assets and operating lease liabilities are recognized at the date the underlying asset becomes available for the Company's use. Operating lease liabilities are based on the present value of the future minimum lease payments over the lease term. ROU assets are measured at the amount of the lease liability, adjusted for any initial direct costs incurred and any lease payments made at or before the lease commencement date, less lease incentives received. As the Company's leases generally do not provide an implicit interest rate, the present value of the future minimum lease payments is determined using the Company's incremental borrowing rate. This rate is an estimate of the collateralized borrowing rate the Company would incur on its future lease payments over a similar term and is based on the information available to the Company at the lease commencement date.

The Company's leases contain options to extend the leases; lease terms are adjusted for these options only when it is reasonably certain the Company will exercise these options. The Company's lease agreements do not contain residual value guarantees or covenants.

The Company has made a policy election regarding its real estate leases not to separate non-lease components from lease components, to the extent they are fixed. Non-lease components that are not fixed are expensed as incurred as variable lease expense. The Company's leases include variable non-lease components, such as common-area maintenance costs. The Company has elected not to record on the balance sheet a lease that has a lease term of 12 months or less and does not contain a purchase option that the Company is reasonably certain to exercise. The Company accounts for leases with initial terms of 12 months or less as operating expenses on a straight-line basis over the lease term.

Lease expense is recognized within operating expenses on a straight-line basis over the terms of the leases. Incentives granted under the Company's facilities lease, including rent holidays, are recognized as adjustments to lease expense on a straight-line basis over the term of the lease.

Stock-based Compensation

The Company measures compensation expense for all stock-based payments to employees, officers and directors based on the estimated fair value of the award at the grant date. For stock options, the Company estimates the grant date fair value of each option award using the Black-Scholes option-pricing model. The grant date fair value of restricted stock units ("RSUs") is based upon the fair market

value of the Company's common stock based on its closing price as reported on the date of grant on the Nasdaq Capital Market. Compensation expense is recognized over the requisite service period on a straight-line basis. Forfeitures are recognized as they occur.

The Company records compensation expense for stock option and RSU grants subject to performance-based milestone vesting over the remaining implicit service period when management determines that achievement of the milestone is probable. Management evaluates when the achievement of a performance-based milestone is probable based on the relative satisfaction of the performance conditions as of the reporting date.

Property and Equipment

Property and equipment consist of computer equipment, computer software, laboratory equipment, leasehold improvements and furniture and office equipment. Property and equipment, excluding leasehold improvements, are recorded at cost and depreciation is recognized using the straight-line method based on estimated useful life, generally three to five years. Leasehold improvements are amortized over the shorter of their useful life or the remaining lease term. Maintenance and repairs are charged to expense as incurred, and costs of improvements are capitalized.

The Company reviews long-lived assets for impairment whenever events or circumstances indicate the carrying amount of an asset group may not be recoverable. Should an impairment exist, the impairment loss would be measured based on the excess of the asset's carrying amount over its fair value. Gains and losses from asset disposals and impairment losses are classified within the consolidated statements of operations and comprehensive loss in accordance with the use of the asset. There were no impairment losses in the years ended December 31, 2025 and 2024 as there have been no events warranting an impairment analysis.

Income Taxes

Income taxes are accounted for under the asset and liability method. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carry forwards. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that includes the enactment date.

In assessing the Company's ability to realize deferred tax assets, management considers whether it is more likely than not that some portion or all of the deferred tax assets will not be realized. The ultimate realization of deferred tax assets is dependent upon the generation of future taxable income during the periods in which those temporary differences are deductible. Management considers the scheduled reversal of deferred tax liabilities, projected future income, tax planning strategies in making this assessment.

The Company recognizes the effect of income tax positions only if those positions are more likely than not of being sustained. Recognized income tax positions are measured at the largest amount that is greater than 50% likely of being realized. Changes in recognition or measurement are reflected in the period in which the change in judgment occurs. The Company accrues interest and penalties related to unrecognized tax benefits in its provision for incomes taxes.

Comprehensive Loss Attributable to Common Stockholders

Comprehensive loss attributable to common stockholders consists of net loss and other gains and losses affecting stockholders' equity that, under U.S. GAAP, are excluded from net loss. The Company's comprehensive loss attributable to common stockholders is comprised of net loss and unrealized gains and losses on available-for-sale securities.

Net Loss Per Share Attributable to Common Stockholders

Basic net loss per share attributable to common stockholders is calculated by dividing the net loss attributable to common stockholders by the weighted-average number of shares of common stock outstanding for the period, without consideration of potentially dilutive securities. Diluted net loss per share attributable to common stockholders is calculated using the more dilutive of the two-class method or treasury method. Diluted net loss per share is the same as basic net loss per share attributable to common stockholders since the effect of potentially dilutive securities is anti-dilutive.

Foreign Currency Transaction Remeasurement Adjustments

Monetary assets and liabilities denominated in foreign currencies were translated into U.S. dollars, the reporting currency, at the exchange rate prevailing at the balance sheet date. Income and expenses denominated in foreign currencies were translated into U.S. dollars at the average exchange rate for the period and the transaction remeasurement adjustments are reported within other income, net in the consolidated statement of operations and comprehensive loss. The functional currency of the Company's Australian subsidiary is the U.S. dollar.

Recent Accounting Pronouncements

In December 2023, the FASB issued ASU 2023-09, Income Taxes (Topic 740) - Improvements to Income Tax Disclosures. The ASU requires that an entity disclose specific categories in the effective tax rate reconciliation as well as provide additional information for reconciling items that meet a quantitative threshold. Further, the ASU requires certain disclosures of state versus federal income tax expense and taxes paid. The amendments in this ASU are required to be adopted for fiscal years beginning after December 15, 2024. Early adoption is permitted and the amendments should be applied on a prospective basis. The Company adopted ASU 2023-09, Income Taxes (Topic 740): Improvements to Income Tax Disclosures for the year ended December 31, 2025 on a prospective basis.

In September 2025, the FASB issued ASU 2025-07, Derivatives and Hedging (Topic 815) and Revenue from Contracts with Customers (Topic 606). Among other things, this ASU refines the scope of Topic 815 to clarify which contracts are subject to derivative accounting. The Company has elected to early adopt this standard prospectively for its annual period ending on December 31, 2025, with no material impact upon adoption.

3. Asset and License Acquisition

On December 18, 2025 (the "Effective Date"), the Company entered into agreements with Sermonix Pharmaceuticals, Inc. ("Sermonix") and Ligand Pharmaceuticals Incorporated ("Ligand"), granting the Company exclusive licenses and rights to develop, manufacture and commercialize oral forms of the selective estrogen-receptor modulator known as lasofoxifene in all countries and territories of the world except for Asia and certain countries in the Middle East (the "Retained Territory").

Sermonix License

Under the Company's license agreement with Sermonix (the "Sermonix License"), the Company received from Sermonix an exclusive (even as to Sermonix), sublicensable license, under relevant patents and know-how owned or in-licensed by Sermonix, to develop, manufacture, commercialize and

otherwise exploit products containing lasofoxifene (the “Licensed Products”) in all countries outside the Retained Territory (the “Licensed Territory”). Such license does not include a sublicense under the patents and know-how that Sermonix previously in-licensed from Ligand because the Company and Ligand entered into a direct license agreement (the “Ligand Agreement”) to replace Sermonix’s license from Ligand with respect to Licensed Products in the Licensed Territory, described in further detail below.

Under the Sermonix License, the Company has the sole right to conduct all development (including regulatory activities), manufacturing and commercialization of Licensed Products in the Licensed Territory, at the Company’s sole cost and expense. The Company is obligated to use commercially reasonable efforts to develop and obtain and maintain regulatory approval for at least one Licensed Product in the Licensed Territory and, following receipt of regulatory approval, to use commercially reasonable efforts to commercialize the applicable Licensed Product in the country of approval. The Company assumed certain of Sermonix’s agreements with third parties that are conducting or supporting the ongoing ELAINE-3 clinical trial of lasofoxifene in the Licensed Territory and certain agreements with third-party contract manufacturers that have been supplying drug substance and drug product for the ELAINE-3 trial. All of Sermonix’s existing inventory of Licensed Product drug substance and drug product was transferred to the Company at no additional cost and the Company agreed to provide a portion of such inventory to Shanghai Henlius Biotech, Inc. ("Henlius") for use in the ELAINE-3 trial in the Retained Territory.

The consideration for the rights granted to the Company under the Sermonix License consists of the following:

(i) Pre-funded warrants to purchase 5,502,402 shares of the Company’s common stock at an exercise price of $0.001 per share (the “Sermonix Pre-Funded Warrant”), issued to Sermonix on the Effective Date pursuant to the securities purchase agreement executed between the Company and Sermonix on December 18, 2025 (the “Sermonix Securities Purchase Agreement”),

(ii) Assumption of liabilities totaling approximately $16.8 million to certain of Sermonix’s third-party service providers for services rendered prior to the Effective Date,

(iii) Deferred monthly advances payable to Sermonix of $75,000 per month, subject to adjustment from time to time upon mutual agreement of the parties. The Company may credit the amount of such monthly payments against all milestone, royalty, and net proceed payments that become due to Sermonix under the Sermonix License, and the Company’s obligation to make such monthly payments will end upon Sermonix’s receipt of the first such milestone, royalty or net proceeds payment.

(iv) If the Company or its affiliates achieve certain commercialization or annual net sales milestones with respect to the Licensed Products, the Company will make milestone payments to Sermonix up to a maximum aggregate total of $100.0 million. Solely with respect to the first milestone payment of $50.0 million due upon the first U.S. commercial sale, the Company may settle such milestone payment in cash or shares of the Company's common stock at the Company’s discretion, with final terms to be negotiated between the parties.

(v) Royalty payments to Sermonix based on the Company’s and its affiliates’ annual net sales of the Licensed Products in the Licensed Territory, with the applicable royalty rates ranging from sub-single digit to low-single digit percentages, subject to customary reductions. Such royalty obligation will expire on a Licensed Product-by-Licensed Product and country-by-country basis on the latest of (i) the expiration of the last valid claim of a patent licensed to the Company by Sermonix that covers such Licensed Product in such country, (ii) the expiration of regulatory

exclusivity of such Licensed Product in such country; and (iii) 10 years after the first commercial sale of such Licensed Product in such country.

(vi) If the Company sublicenses or divests its rights to the Licensed Products to a third party, then in lieu of the milestone and royalty payments described above, the Company will pay to Sermonix a percentage of the net proceeds from such sublicensing or divestiture transaction. The applicable percentage is based on the type of payment received by the Company from the third party as well as status of development of the Licensed Products at the time that the sublicense or divestiture transaction is entered into, with rates starting in the high-double digit and decreasing to low-double digit percentages.

(vii) As consideration for the Company's payment and other obligations under the Sermonix License, the Company will be eligible to receive from Henlius all milestone and royalty payments that Henlius owes to Sermonix under Sermonix's license agreement with Henlius, other than royalties that Sermonix owes to Ligand on account of Sermonix's license agreement with Ligand, and with the further exception that, if Henlius and Sermonix later agree to share the costs of developing the Licensed Products in Japan, then the Company will not be entitled to receive Sermonix's share of income arising from the sublicensing of Japanese rights to the Licensed Product.

The Sermonix License will remain in effect until the Company, its affiliates and its sublicensees are no longer developing or commercializing any Licensed Product in the Licensed Territory. Each party may terminate the Sermonix License in its entirety for the uncured material breach by or insolvency of the other party. The Company may terminate the Sermonix License in its entirety for safety reasons or for convenience at any time after the topline data readout of the ELAINE-3 trial. Upon termination, all rights and licenses granted to Company will revert to Sermonix.

Ligand Agreement

Under the Ligand Agreement, the Company received from Ligand an exclusive (even as to Ligand), sublicensable license, under relevant know-how and certain patents owned or in-licensed by Ligand, to develop, manufacture and sell Licensed Products for oral use in the Licensed Territory. Ligand does not have any restrictions on its ability to develop, manufacture or sell products (other than the Licensed Products) that selectively modulate the estrogen receptor. The Company granted to Ligand a non-exclusive, sublicensable, worldwide license, under the Company's improvements to Ligand's licensed technology, to develop, manufacture and sell products containing lasofoxifene for topical and other non-oral uses.

Under the Ligand Agreement, the Company has the sole right to conduct all development (including regulatory activities), manufacturing and commercialization of Licensed Products for oral use in the Licensed Territory, at the Company's sole cost and expense. The Company is obligated to diligently develop, manufacture and sell oral Licensed Products in the Licensed Territory, to use commercially reasonable efforts to develop oral Licensed Products for treatment of metastatic breast cancer and develop markets for oral Licensed Products in the Licensed Territory, and to obtain all necessary regulatory approvals for its manufacture, use, importation and sale of oral Licensed Products in the Licensed Territory.

The consideration for the rights granted to the Company under the Ligand Agreement consists of the following:

(i) If the Company or its affiliate or sublicensee achieves certain regulatory approval and commercialization milestones with respect to any oral Licensed Product in the Licensed Territory, the Company will make milestone payments to Ligand up to a maximum aggregate total of $4.25 million in regulatory milestones and $10.5 million in commercialization milestones per Licensed Product.

(ii) Royalty payments to Ligand based on the Company's and its affiliates' and sublicensees' annual net sales of oral Licensed Products in the Licensed Territory, with the applicable royalty rates ranging from mid-single digit to low-double digit percentages, subject to reductions for generic product sales and amounts paid to third parties for certain intellectual property licenses. Such royalty obligation will expire on a Licensed Product-by-Licensed Product and country-by-country basis on the latest of (i) the expiration of the last valid claim of a patent licensed to the Company by Ligand in such country, (ii) the expiration of regulatory exclusivity of such Licensed Product in such country, and (iii) 15 years after the first commercial sale of such Licensed Product in such country.

(iii) If the Company sublicenses its rights to the oral Licensed Products to a third party, then in addition to the milestone and royalty payments described above, the Company will pay to Ligand a mid-teen percentage of certain amounts received by the Company from such third-party sublicensee.

The Ligand Agreement will remain in effect so long as the Company, its affiliates and its sublicensees are developing, manufacturing, using or commercializing any Licensed Product in the Licensed Territory. Each party may terminate the Ligand Agreement in its entirety for the uncured material breach by or insolvency of the other party. The Company may terminate the Ligand Agreement in its entirety or on a product-by-product or country-by-country basis, prior to regulatory approval, for safety reasons or failure to achieve the primary efficacy endpoint in any clinical trial of a Licensed Product for oral use. Ligand may terminate the Ligand Agreement if the Company or its affiliate or sublicensee challenges the validity or enforceability of any patent licensed to the Company by Ligand. Upon termination, all rights and licenses granted to Company will revert to Ligand and the Company is obligated to transfer to Ligand the Company's data, regulatory approvals, domain names and trademarks for the oral Licensed Products in the Licensed Territory.

Accounting Treatment

The Company accounted for the transactions as an asset acquisition under ASC 805-50 as substantially all of the fair value of the gross assets acquired was concentrated in a single identifiable IPR&D asset. The assets acquired in the transaction were measured based on the estimated fair value of the aggregate consideration of $68.1 million. The initial consideration recognized and the fair value of the asset acquired is as follows (in thousands):

Sermonix Pre-Funded Warrant[(1)]	$	32,909
Assumed liabilities[(2)]		16,816
Milestone liability[(3)]		15,116
Advance liability[(4)]		2,818
Transaction costs		429
Consideration paid	$	68,088
Assets acquired:		
Acquired in-process research and development[(5)]	$	68,088
Total assets acquired	$	68,088

(1) Reflects the fair value of the Sermonix Pre-Funded Warrant on the Effective Date. See Note 4 for fair value considerations.
(2) Represents approximately $16.8 million payable in cash to satisfy certain of Sermonix's outstanding liabilities owed to third-party service providers for services rendered in connection with the ELAINE-3 trial prior to the Effective Date.
(3) Of the total milestone payments required under the agreements, the Company is contingently obligated to settle the first milestone due to Sermonix by transferring assets or issuing a variable number of equity shares with a value equal to a fixed monetary amount. As such, the first milestone payment is classified a liability under ASC 480 and the initial fair value on the Effective Date of $15.1 million is recognized as part of the initial consideration for the asset acquired. See Note 4 for fair value considerations.
(4) Reflects the initial fair value of the deferred monthly advance of $75,000 to be made by the Company to Sermonix, which advances are creditable against all milestone, royalty, and net proceed payments that become due to Sermonix under the Sermonix License.
(5) The cost attributable to the IPR&D was expensed on the Effective Date as the IPR&D has no alternative future use, and is presented as acquired in-process research and development in the Company's consolidated statements of operations and comprehensive loss for the year ended December 31, 2025.

The remaining cash-settled milestone obligations under the Sermonix and Ligand agreements were not recognized as of the Effective Date or as of December 31, 2025 because (i) the arrangements do not qualify as derivatives under ASC 815, and (ii) achievement of the related milestones is not considered probable. Consistent with ASC 450, the Company will recognize the milestone obligations if and when any such milestones become probable and reasonably estimable or when the triggering events occur. Future royalty payments based on annual net sales are exempt from derivative accounting under the ASC 815 scope exception for specified volumes of sales or service revenues.

As consideration for the rights granted to the Company under the Sermonix License on December 18, 2025, the Company issued to Sermonix the Sermonix Pre-Funded Warrant to purchase 5,502,402 shares of the Company's common stock pursuant to the Sermonix Securities Purchase Agreement.

The Sermonix Pre-Funded Warrant has an exercise price of $0.001 per share and are exercisable at any time after the date of approval from the Company's stockholders as required by Nasdaq, including for purposes of Nasdaq Rule 5635(a), such that the Sermonix Pre-Funded Warrant can be exercised at any time without restriction or additional stockholder approval (the "Sermonix Stockholder Approval"). If the Sermonix Stockholder Approval has not been obtained by the first anniversary of the original issuance of the Sermonix Pre-Funded Warrant, Sermonix shall have the right (the "Sermonix Redemption Right") at any time and from time to time prior to such time that the Sermonix Stockholder Approval is obtained thereafter, to cause the Company to pay, at the option of Sermonix, an amount up to (a) $6.35 (the "Sermonix Redemption Price") multiplied by (b) the number of shares of common stock with respect to which Sermonix is exercising the Sermonix Redemption Right. The Sermonix Redemption Right shall terminate on the earlier of (1) such time as an aggregate of $7.5 million in aggregate Sermonix Redemption Price has been paid by the Company to Sermonix in connection with one or more exercises of the Sermonix Redemption Right; and (2) immediately upon receipt of the Sermonix Stockholder Approval.

As a result of the Sermonix Redemption Right, the Company classified the Sermonix Pre-Funded Warrant as a liability in accordance with ASC 480. Because the redemption feature could require the Company to settle the warrant by transferring assets, the Sermonix Pre-Funded Warrant does not meet the criteria for equity classification.

The Company initially recognized the Sermonix Pre-Funded Warrant at its estimated fair value of approximately $32.9 million on the Effective Date of the Sermonix License (See Note 4 for fair value considerations). The Sermonix Pre-Funded Warrant will be remeasured to its fair value each reporting period, with changes in its estimated fair value of the warrants recorded as a non-cash gain or loss on the consolidated statements of operations and comprehensive loss. The Company will reassess the classification of the Sermonix Pre-Funded Warrant at each reporting date.

As of December 31, 2025, the Sermonix Pre-Funded Warrant has not been exercised in full or in part and remains outstanding.

4. Fair Value

The following table summarizes the Company's assets and liabilities measured at fair value on a recurring basis and their respective input levels based on the fair value hierarchy (in thousands):

	December 31, 2025			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market fund	$ 17,299	$ —	$ —	$ 17,299
U.S. government debt and agency securities	—	2,541	—	2,541
Commercial paper	—	32,314	—	32,314
Total cash equivalents	17,299	34,855	—	52,154
Short-term investments:				
Commercial paper	—	5,209	—	5,209
U.S. government debt and agency securities	—	13,846	—	13,846
Total short-term investments	—	19,055	—	19,055
Total assets subject to fair value measurements on a recurring basis	$ 17,299	$ 53,910	$ —	$ 71,209
Liabilities:				
Sermonix Pre-Funded Warrant	$ —	$ —	$ 37,488	37,488
Milestone Liability	—	—	15,116	15,116
Total liabilities subject to fair value measurements on a recurring basis	$ —	$ —	$ 52,604	$ 52,604

	December 31, 2024			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market fund	$ 30	$ —	$ —	$ 30
U.S. government debt and agency securities		10,299	—	10,299
Commercial paper		19,393	—	19,393
Total cash equivalents	30	29,692	—	29,722
Short-term investments:				
Commercial paper	—	1,445	—	1,445
U.S. government debt and agency securities	—	1,392	—	1,392
Total short-term investments	$ —	2,837	$ —	$ 2,837
Total assets subject to fair value measurements on a recurring basis	$ 30	$ 32,529	$ —	$ 32,559

U.S government debt and agency securities and commercial paper are classified as Level 2 as they were valued based upon quoted market prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets that are not active, and model-based valuation techniques for which all significant inputs are observable in the market or can be corroborated by observable market data for substantially the full term of the assets.

The fair value of the Sermonix Pre-Funded Warrant (see Note 3) was determined based on the fair value of the Company's common stock, adjusted for a discount for lack of marketability, and was classified as Level 3 due to the use of unobservable market data for identical or similar liabilities. The fair value of the Milestone Liability was determined based on the probability weighted present value of the net milestone payment and was classified as Level 3 due to the use of unobservable market data for identical or similar liabilities. The key inputs into the fair value of the Sermonix Pre-Funded Warrant and Milestone Liability at issuance and at December 31, 2025 were as follows:

	Sermonix Pre-Funded Warrant	
	December 31, 2025	December 18, 2025
Fair Value of Common Stock	$ 7.57	$ 6.72
Volatility	97.0%	97.0%
Risk-free rate	3.7%	3.6%
Dividend Yield	0.0%	0.0%
Holding period years	0.20	0.30

	Milestone Liability	
	December 31, 2025	December 18, 2025
Weighted Average Cost of Capital	11.0%	11.00%
Probability	43.9%	43.9%

There were no transfers of financial instruments between Level 1, Level 2, and Level 3.

The following table sets forth a summary of the changes in the fair value of the Sermonix Pre-Funded Warrant and Milestone Liability for the year ended December 31, 2025 (in thousands):

	Sermonix Pre-Funded Warrant	Milestone Liability
December 31, 2024	$ —	$ —
Issuance	32,909	15,116
Change in fair value	4,579	—
December 31, 2025	37,488	15,116

The fair value of the Level 3 liabilities may change significantly as additional data is obtained. In evaluating this information, considerable judgment is required to interpret the data used to develop the assumptions and estimates. Accordingly, the use of different market assumptions and/or different valuation techniques may have a material effect on the estimated fair value amounts, and such changes could materially impact the Company's results of operations in future periods.

The following tables reflect the Company's financial asset balances measured at fair value on a recurring basis (in thousands):

		December 31, 2025			
	Level	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Cash equivalents:					
Money market fund	1	$ 17,299	$ —	$ —	$ 17,299
U.S. government debt and agency securities	2	2,541	—	—	2,541
Commercial paper	2	32,319	—	(5)	32,314
Total cash equivalents		$ 52,159	$ —	$ (5)	$ 52,154
Short-term investments:					
Commercial paper	2	5,210	—	(1)	5,209
U.S. government debt and agency securities	2	13,844	2	—	13,846
Total short-term investments		$ 19,054	$ 2	$ (1)	$ 19,055

		December 31, 2024			
	Level	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Cash equivalents:					
Money market fund	1	$ 30	$ —	$ —	$ 30
U.S. government debt and agency securities	2	10,297	2	—	10,299
Commercial paper	2	19,394	—	(1)	19,393
Total cash equivalents		$ 29,721	$ 2	$ (1)	$ 29,722
Short-term investments:					
Commercial paper	2	1,445	—	—	1,445
U.S. government debt and agency securities	2	1,392	—	—	1,392
Total short-term investments		$ 2,837	$ —	$ —	$ 2,837

All the commercial paper and U.S. government debt and agency securities designated as short-term investments have an effective maturity date that is equal to or less than one year from the respective balance sheet date.

As of December 31, 2025, the Company does not intend to sell any securities in unrealized loss positions, and it is not more-likely-than-not that the Company will be required to sell such securities prior to the recovery of the amortized cost basis. Based on the Company's assessment, the Company concluded all impairments as of December 31, 2025 to be due to factors other than credit loss, such as changes in interest rates. A credit loss allowance was not recognized and the unrealized losses for available-for-sale securities were recorded in other comprehensive loss

5. Property and Equipment, Net

Property and equipment consisted of the following (in thousands):

	December 31, 2025	December 31, 2024
Lab equipment	$ 705	$ 705
Office furniture, fixtures, and computer equipment	712	712
Leasehold improvement	4,322	4,322
Property and equipment, at cost	5,739	5,739
Less: accumulated depreciation	(4,267)	(3,295)
Property and equipment, net	$ 1,472	$ 2,444

Depreciation expense was $1 million and $1 million for the years ended December 31, 2025 and 2024, respectively.

6. Accrued Liabilities

Accrued liabilities consisted of the following (in thousands):

	December 31, 2025	December 31, 2024
Research and development	$ 2,012	$ 3,424
Employee compensation and benefits	1,948	2,990
Legal expense	—	4,127
Professional services and other	1,003	1,861
Total accrued liabilities	$ 4,963	$ 12,402

7. Segment Reporting

The Company operates as a single operating segment, which is the business of developing and commercializing therapeutics. The Company’s chief operating decision maker ("CODM"), its chief executive officer, reviews financial information on an aggregate basis for the purpose of allocating resources and assessing performance. When deciding how to allocate resources, the CODM reviews the financial results of the Company's drug candidate programs. The measure of segment assets is reported on the consolidated balance sheet as total assets.

The table below is a summary of the segment profit or loss, including significant segment expenses (in thousands):

	Year Ended December 31,	
	2025	2024
Research and development expenses:		
Acquired in-process research and development	$ 68,088	$ —
Lasofoxifene	3,568	—
Fosgonimeton (ATH-1017)	2,335	41,510
ATH-1105	3,415	8,567
ATH-1020	5	495
Preclinical programs and other costs	1,251	3,390
Personnel-related costs, excluding stock-based compensation	4,221	12,289
Total research and development expenses	82,883	66,251
General and administrative expenses	12,309	22,631
Other segment expenses[(a)]	7,074	12,020
Total operating expenses	102,266	100,902
Loss from operations	(102,266)	(100,902)
Other income, net	1,236	3,962
Sermonix pre-funded warrant change in fair value	(4,579)	—
Net loss	$ (105,609)	$ (96,940)

[(a)]Other segment expenses includes stock-based compensation and depreciation expenses.

8. Commitments and Contingencies

Legal Proceedings

From time to time, the Company may be subject to various legal proceedings or claims that arise in the ordinary course of business. The Company accrues a liability when the Company's management believes that it is both probable that a liability has been incurred and the amount of loss can be reasonably estimated. There were no material litigation-related accruals recorded as of December 31, 2025.

Operating Leases

The Company has operating leases for laboratory and office facilities in Bothell, Washington that expire in August 2027. The initial terms of the leases range from 6.3 to 7 years and the Company has options to extend the leases for an additional five years that it is not reasonably certain to exercise. As of December 31, 2025, the Company was not party to any finance leases.

The following table reconciles the Company's undiscounted operating lease cash flows to its operating lease liability (in thousands):

	December 31, 2025
2026	$ 509
2027	$ 346
Total undiscounted lease payments	$ 855
Present value adjustment for minimum lease commitments	$ (52)
Net lease liability	$ 803

The weighted average remaining lease term and the weighted average discount rate used to determine the operating lease liability were as follows:

	December 31, 2025
Weighted average remaining lease term (years)	$ 1.9
Weighted average discount rate	8.1%

Operating lease expense and variable lease expense consisted of the following (in thousands):

	Year Ended December 31,	
	2025	2024
Operating lease expense	$ 353	$ 353
Variable lease expense	177	149

Indemnifications

In the ordinary course of business, the Company enters into agreements that may include indemnification provisions. Pursuant to such agreements, the Company may indemnify, hold harmless and defend an indemnified party for losses suffered or incurred by the indemnified party. Some of the provisions will limit losses to those arising from third party actions. In some cases, the indemnification will continue after the termination of the agreement. The maximum potential amount of future payments the Company could be required to make under these provisions is not determinable. To date the Company has not incurred material costs to defend lawsuits or settle claims related to these indemnification provisions. The Company enters into indemnification agreements with its directors and officers that may require the Company to indemnify its directors and officers against liabilities that may arise by reason of their status or service as directors or officers to the fullest extent permitted by Delaware corporate law. The Company currently has directors' and officers' insurance coverage that reduces its exposure and enables the Company to recover a portion of any future amounts paid.

9. Common Stock

Each share of common stock has the right to one vote. The holders of common stock are also entitled to receive dividends whenever funds are legally available and if declared by the Company's board of directors, subject to the prior rights of holders of all classes of stock outstanding having priority rights as to dividends. No cash dividends have been declared by the board of directors from inception.

The Company has reserved the following shares of common stock for future issuance, on an as-converted basis, as follows:

	December 31, 2025	December 31, 2024
Shares issuable upon the exercise of outstanding common stock options and the vesting of outstanding common restricted stock units granted	1,262,430	1,023,497
Shares available for future grant under the 2020 Equity Incentive Plan	63,723	166,638
Shares available for future grant under the Employee Stock Purchase Plan	156,754	133,844
Shares available for future grant under the 2024 Inducement Equity Incentive Plan	35,000	35,000
PIPE Pre-Funded Warrants	8,816,684	—
Series A Common Warrants	23,031,494	—
Series B Common Warrants	21,259,842	—
Sermonix Pre-Funded Warrant	5,502,402	—
Total	60,128,329	1,358,979

2025 PIPE Financing

In December 2025, the Company entered into a securities purchase agreement with investors, pursuant to which the Company issued, by way of a private placement (the "Private Placement") an aggregate of (i) 5,356,547 shares of the Company's common stock, (ii) pre-funded warrants (the "PIPE Pre-Funded Warrants") to purchase 8,816,684 shares of common stock, (iii) warrants (the "Series A Common Warrants") to purchase 23,031,494 shares of common stock and/or shares underlying pre-funded warrants and (iv) warrants (the "Series B Common Warrants") to purchase 21,259,842 shares of common stock and/or shares underlying pre-funded warrants. The aggregate gross proceeds from the Private Placement was $90.0 million. Issuance costs of $7.6 million are reflected as deduction from additional paid-in capital.

The PIPE Pre-Funded Warrants are exercisable at an exercise price of $0.001 per share, subject to customary adjustments and are exercisable at any time on or after issuance subject to the restriction discussed below.

The Series A Common Warrants have an exercise price of $6.35 per share and are exercisable after the earlier of (1) the latest of (a) June 30, 2026, (b) the date on which the Company publicly announces the enrollment of the 500th subject or the last subject, whichever is earlier, in the ELAINE-3 trial; and (c) the date on which the FDA approves or issues a complete response letter to Eli Lilly & Co.'s marketing application, including a supplement to a new drug application, for imlunestrant in combination with abemaciclib in breast cancer, and (2) October 31, 2026 (the "Series A Common Warrant Initial Exercise Date"). The Series A Common Warrants will remain exercisable until the 30th day following the Series A Common Warrant Initial Exercise Date (the "Series A Common Warrant Termination Date"), except that if the Series A Common Warrant Initial Exercise Date has not occurred by December 23, 2030, then December 23, 2030 will be the Series A Common Warrant Termination Date.

The Series B Common Warrants have an exercise price of $7.62 per share and will be exercisable after the later of (1) June 30, 2026 and (2) the date of the completion of the public readout of topline results of the ELAINE-3 trial (the later of (1) and (2), the "Series B Common Warrant Initial Exercise Date"). The Series B Common Warrants will remain exercisable until the 30th day following the Series B Common Warrant Initial Exercise Date (the "Series B Common Warrant Termination Date"), except that if the Series B Common Warrant Initial Exercise Date has not occurred by December 23, 2030, then December 23, 2030 will be the Series B Common Warrant Termination Date. At issuance and at

December 31, 2025, the PIPE Pre-Funded Warrants, Series A Common Warrants and Series B Common Warrants meet the criteria to be classified as equity instruments.

None of the PIPE Pre-Funded Warrants, the Series A Common Warrants or the Series B Common Warrants can be exercised if, immediately prior to or following such exercise, the applicable investor, together with its affiliates and any other persons whose beneficial ownership of shares of common stock would be aggregated with such investor for purposes of Section 13(d) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), would beneficially own more than 4.99%, 9.99% or 19.99%, at the election of such investor (in each case, the “PIPE Maximum Percentage”) of the total number of issued and outstanding shares of common stock following such exercise. If, upon exercise of the Series A Common Warrants or Series B Common Warrants, an investor would exceed the PIPE Maximum Percentage, then the Series A Common Warrants and Series B Common Warrants may be exercised for pre-funded warrants to purchase shares of common stock, which pre-funded warrants will have terms substantially the same as the PIPE Pre-Funded Warrants. The PIPE Maximum Percentage may be increased or decreased by an investor with 61 days’ written notice to us; provided, however, that such percentage may in no event exceed (x) 19.99% prior to a vote of our stockholders approving the removal of such exercise limitation or (y) with respect to certain investors, 4.99%

As of December 31, 2025, no PIPE Pre-Funded Warrants, PIPE Series A Common Warrants or PIPE Series B Common Warrants have been exercised.

Equity Incentive Plans

The Company’s 2020 Equity Incentive Plan (the "2020 Plan") provides for annual increases in the number of shares that may be issued under the 2020 Plan on January 1, 2021 and each subsequent January 1 thereafter by a number of shares equal to the least of (1) 323,000 shares, (2) 5% of the number of shares of common stock issued and outstanding on the immediately preceding December 31, and (3) an amount determined by the Company’s board of directors.

The Company’s 2020 Employee Stock Purchase Plan (the "ESPP") provides for annual increases in the number of shares that may be issued under the ESPP on January 1, 2021 and each subsequent January 1 thereafter by a number of shares equal to the least of (1) 64,600 shares, (2) 1% of the number of shares of common stock issued and outstanding on the immediately preceding December 31, and (3) an amount determined by the Company’s board of directors.

In February 2024, the board of directors adopted the LeonaBio, Inc. 2024 Inducement Equity Incentive Plan (the "2024 Inducement Plan"), and, subject to the adjustment provisions of the 2024 Inducement Plan, reserved 75,000 shares of the Company's common stock for issuance pursuant to equity awards granted under the 2024 Inducement Plan.

Effective January 1, 2026, the Company’s 2020 Plan and ESPP reserves increased by 323,000 shares and 64,600 shares, respectively.

10. Stock-based Compensation

Stock-based compensation expense recognized was as follows (in thousands):

	Year Ended December 31,	
	2025	2024
Research and development	$ 2,316	$ 4,043
General and administrative	3,786	7,006
Total stock-based compensation expense	$ 6,102	$ 11,049

Valuation Assumptions

The fair value of stock options was determined using the Black-Scholes option-pricing model and the assumptions below. Each of these inputs is subjective and generally required significant judgment.

- *Fair Value of Common Stock*—The fair value of each share of common stock is based on the closing price of the Company's common stock on the date of grant, or other relevant determination date, as reported on The Nasdaq Capital Market.
- *Risk-Free Interest Rate*—The risk-free interest rate is based on the U.S. Treasury zero coupon issues in effect at the time of grant for periods corresponding with the expected term of the option.
- *Expected Volatility*—Because the Company was previously privately held and did not have any trading history for its common stock, the expected volatility was estimated based on the average volatility for comparable publicly traded life sciences companies over a period equal to the expected term of the stock option grants. The comparable companies were chosen based on the similar size, stage in life cycle or area of specialty. The Company will continue to apply this process until a sufficient amount of historical information regarding the volatility of its own stock price becomes available.
- *Expected Term*—The expected term represents the period that the stock-based awards are expected to be outstanding and is determined using the simplified method (based on the midpoint between the vesting date and the end of the contractual term) as the Company has limited history of relevant stock option exercise activity.
- *Expected Dividend Yield*—The Company has never paid dividends on its common stock and has no plans to pay dividends going forward. Therefore, it used an expected dividend yield of zero.

The fair value of each stock option was estimated using the Black-Scholes option-pricing model with the following weighted-average assumptions:

	Year Ended December 31,	
	2025	2024
Risk-free interest rate	3.83%	4.13%
Expected volatility	102.99%	97.21%
Expected term (in years)	6.00	5.88
Expected dividend yield	—	—

The grant date fair value of RSUs is based upon the fair market value of the Company's common stock based on its closing price as reported on the date of grant on the Nasdaq Capital Market.

The fair value of options granted during the years ended December 31, 2025 and 2024 was $1.6 million and $10.6 million, respectively. The fair value of RSUs granted during the year ended December 31, 2025 and 2024 was $0.2 million and $0.4 million, respectively.

Stock Option Activity

Changes in shares available for grant under the 2020 Plan and the 2024 Inducement Plan during the year ended December 31, 2025 were as follows:

	Shares Available for Grant
Shares available for grant at December 31, 2024	201,638
2020 Plan reserve increase on January 1, 2025	195,277
Options and restricted stock units granted	(551,384)
Options and restricted stock units forfeited, cancelled, or expired	253,192
Shares available for grant at December 31, 2025	98,723

A summary of stock option activity under the 2020 Plan and the 2024 Inducement Plan for the year ended December 31, 2025 was as follows:

	Shares	Weighted-Average Exercise price per Share	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Balance at December 31, 2024	961,156	$ 60.83	8.08	$ 130
Granted	490,488	3.90		
Exercised	—	—	—	—
Forfeited/expired	(246,815)	61.86		
Balance at December 31, 2025	1,204,829	$ 37.44	8.14	$ 2,030
Expected to vest	603,476	$ 10.47	9.26	$ 1,704
Options exercisable	601,353	$ 64.51	7.02	$ 326

The total fair value of options granted that vested during the years ended December 31, 2025 and 2024 was $7.3 million and $11.0 million, respectively.

The aggregate intrinsic value in the table above is calculated as the difference between the exercise price of the underlying options and the estimated fair value of the Company's common stock underlying all options that were in-the-money at December 31, 2025. The aggregate intrinsic value of options exercised was $0 million and $0.2 million during the year ended December 31, 2025 and 2024, respectively, determined as of the date of option exercise. As of December 31, 2025, there was $4.5 million of total unrecognized compensation cost related to non-vested stock options. The Company expects to recognize this cost over a remaining weighted-average period of 2.49 years. The Company utilizes newly issued shares to satisfy option exercises.

Stock options outstanding and exercisable under the 2020 Plan and the 2024 Inducement Plan consisted of the following at December 31, 2025:

Exercise Price ($)	Options Outstanding	Options Exercisable
3.13 to 7.14	568,973	103,161
10.40 to 42.20	424,264	289,359
89.3 to 199.40	185,933	183,174
200.55 to 294.10	25,659	25,659
Total	1,204,829	601,353

Restricted Stock Unit Activity

A summary of RSU, activity for the year ended December 31, 2025 is as follows:

	Share Equivalent	Weighted-Average Grant Date Fair Value
Non-vested at December 31, 2024	62,259	$ 4.57
Granted	60,896	$ 3.76
Cancelled	(6,377)	$ 4.16
Vested	(59,177)	$ 4.51
Non-vested at December 31, 2025	57,601	$ 3.76

As of December 31, 2025, there was $0.4 million of total unrecognized compensation cost related to non-vested RSUs. The Company expects to recognize this cost over a remaining weighted-average period of 0.17 years.

Employee Stock Purchase Plan

Under the ESPP, eligible employees can authorize payroll deductions for amounts up to the lesser of 15% of their qualifying wages or the statutory limit under the U.S. Internal Revenue Code. The ESPP provides for offering periods of six months in duration with one purchase period per offering period beginning May 18 and November 18 of each year. Participants in an offering period will be granted the right to purchase shares of the Company's common stock at a price per share that is 85% of the lesser of the fair market value of the shares at (1) the first day of the offering period or (2) the end of each purchase period within the offering period. A maximum of 10,000 shares of common stock may be purchased by each participant at the purchase date during the offering period. The fair market value of the ESPP options granted is determined using the Black-Scholes model and is amortized on a straight-line basis. Stock-based compensation expense recognized during the years ended December 31, 2025 and 2024 associated with the ESPP was $0 million and $0.1 million, respectively. During the year ended December 31, 2025, the Company issued 16,131 shares of common stock to employees under the ESPP.

11. Income Taxes

Components of Income and Income Tax

The Company did not record a provision (benefit) for income taxes for the years ended December 31, 2025 and 2024. Net loss is attributable to the following tax jurisdictions (in thousands):

	Year Ended December 31,	
	2025	2024
United States	$ (105,610)	$ (96,910)
Foreign	1	(30)
Net Loss	$ (105,609)	$ (96,940)

The provision for income taxes differs from the amount expected by applying the federal statutory rates to the net loss before taxes as follows:

	Year Ended December 31, 2024
Federal statutory income tax rate	21.0%
State taxes	—
Stock-based compensation	(1.8)
Non-deductible expenses and others	(0.9)
Tax credits	3.1
Change in valuation allowance	(21.4)
Effective income tax rate	—%

The Company adopted ASU 2023-09, Income Taxes (Topic 740): Improvements to Income Tax Disclosures for the year ended December 31, 2025 on a prospective basis (in thousands):

	Year Ended December 31, 2025	
At Statutory Rate	$ (22,178)	21%
State Income Taxes, net of Federal Effect	—	0
Change in Valuation Allowance	20,269	(19.2)
Nontaxable or Nondeductible Items	—	0
Equity Compensation	1,809	(1.7)
Other Nondeductible Items	669	(0.6)
Tax credits	(759)	0.7
Worldwide change in UTB	190	(0.2)
Total	$ —	—%

Deferred Tax Assets and Liabilities

The components of the Company's deferred tax assets and liabilities were as follows (in thousands):

	Year Ended December 31, 2025	2024
Deferred tax assets:		
Net operating loss carryforwards	$ 56,887	$ 43,414
Research and development tax credit carryforwards	12,931	12,362
Accrued liabilities	369	538
Stock-based compensation	2,287	2,867
Operating lease liability	170	256
Property and equipment	352	250
Intangibles	14,944	8
Capitalized research and development	24,924	32,540
Total deferred tax assets	112,864	92,235
Deferred tax liabilities:		
Right of use asset	(113)	(170)
Prepaid expenses and other	(109)	(82)
Investments	(19)	(34)
Total deferred tax liabilities	(241)	(286)
Less valuation allowance	(112,623)	(91,949)
Net deferred tax assets	$ —	$ —

Deferred income taxes reflect temporary differences between the carrying amounts of assets and liabilities for financial reporting and income tax purposes, and operating losses and tax credit carryforwards. The Company considers a number of factors concerning the realizability of its net deferred tax assets, including its history of operating losses, the nature of the deferred tax assets, and the timing, likelihood and amount, if any, of future taxable income during the periods in which those temporary differences and carryforwards become deductible, all of which require significant judgment. As of December 31, 2025, the Company has recorded a full valuation allowance on its net deferred tax assets as the Company has concluded that it is not more likely than not that such losses or credits will be utilized. The valuation allowance increased by $20.7 million during 2025 and 2024.

At December 31, 2025, the Company has federal net operating loss and tax credit carryforwards of $9.5 million and $17.2 million, respectively, which expire over a period of 6 to 12 years. Net operating loss carryforwards of $259.8 million were generated after 2017, and therefore do not expire. As of December 31, 2025, the Company also had state net operating loss carryforwards of $5 million, which expire over a period of 17 to 20 years.

Uncertain Tax Positions

The Company files federal income tax returns. With few exceptions, the Company is no longer subject to income tax examinations by tax authorities for years prior to 2016. However, to the extent allowed by law, the tax authorities may have the right to examine prior periods where net operating losses or tax credits were generated and carried forward and may make adjustments to the amount of the net operating loss or credit carryforward amount. The Company is not currently under examination in any jurisdiction.

A reconciliation of the beginning and ending amount of unrecognized tax benefits for uncertain tax positions were as follows (in thousands):

	Year Ended December 31,	
	2025	2024
Beginning balance	$ 4,121	$ 3,109
Additions for tax positions taken in prior years	—	—
Additions for tax positions taken in the current year	190	1,012
Ending balance	$ 4,311	$ 4,121

If the unrecognized tax benefits for uncertain tax positions as of December 31, 2025 are recognized, there will be no impact to the effective tax rate due to the valuation allowance. The Company recognizes interest and penalties related to unrecognized tax benefits within the income tax expense line in the accompanying consolidated financial statements. At December 31, 2025, there were no material interest and penalties on uncertain tax benefits. The Company does not anticipate any significant changes to its unrecognized tax benefits in the next 12 months.

12. Employee Benefit Plans

The Company has a 401(k) Plan for all of its employees. The 401(k) Plan allows eligible employees to defer, at the employee's discretion, up to 100% of their pretax compensation up to the Internal Revenue Service annual limit. The Company made matching contributions of $0.2 million and $0.5 million during the years ended December 31, 2025 and 2024, respectively.

13. Net Loss Per Share Attributable to Common Stockholders

The following outstanding shares of potentially dilutive securities were excluded from the computation of the diluted net loss per share attributable to common stockholders for the periods presented because their effect would have been anti-dilutive:

	Year Ended December 31,	
	2025	2024
Stock options to purchase common stock	1,204,829	961,156
Non-vested Restricted Stock Units	57,601	62,259
Employee stock purchase plan	1,238	851
Series A Common Warrants	23,031,494	
Series B Common Warrants	21,259,842	
Sermonix Pre-Funded Warrant	5,502,402	
Total	51,057,406	1,024,266

14. Corporate Restructuring

On September 15, 2024, the Company committed to the Restructuring. (see Note 2). In connection with the Restructuring, the Company incurred costs of $2.9 million, consisting primarily of cash severance costs and termination benefits, which the Company recognized during the year ended December 31, 2024. The Company substantially completed the Restructuring by December 31, 2024.

As of December 31, 2024, the accrued liability balance associated with the Restructuring was $0.4 million and was included in the accrued expenses line of the accompanying consolidated balance sheet. Cash paid during the year ended December 31, 2024 associated with the termination benefits was $2.5 million. The remaining $0.4 million accrued as of December 31, 2024 was settled during the twelve months ended December 31, 2025.

15. Subsequent events

The Company evaluated subsequent events and transactions that occurred after the balance sheet date through the date that the consolidated financial statements were issued. Based upon this review, or within these consolidated financial statements, other than as disclosed below, the Company did not identify any subsequent events that would have required recognition or disclosure in the consolidated financial statements.

Special Meeting

On February 23, 2026, the Company filed with the SEC a definitive proxy statement (the "Proxy Statement") with respect to its special meeting of stockholders to be held on March 18, 2026 at 8:00 a.m. Pacific Time (the "Special Meeting") to vote upon the proposals detailed in the Proxy Statement including(1) to approve the issuance of 5,502,402 shares of common stock pursuant to the exercise of the Sermonix Pre-Funded Warrant in accordance with Nasdaq Rule 5635(a)(2), (2) to approve the issuance of shares of common stock to Sermonix pursuant to an exercise of the Sermonix Pre-Funded Warrant if, immediately following such exercise, Sermonix, together with its affiliates and any other persons whose beneficial ownership of shares of common stock would be aggregated with Sermonix for purposes of Section 13(d) of the Exchange Act, would beneficially own in excess of 19.99% of the Company's outstanding common stock, in accordance with Nasdaq Rule 5635(b), (3) to approve the issuance of shares of common stock to Perceptive Life Sciences Master Fund, Ltd and Perceptive Xontogeny Venture Fund II, LP (collectively, "Perceptive") pursuant to the exercise of warrants if, immediately following such exercise, Perceptive, together with any other persons whose beneficial ownership of shares of common stock would be aggregated with Perceptive for purposes of Section 13(d) of the Exchange Act, would beneficially own in excess of 19.99% of the Company's outstanding common stock, in accordance with Nasdaq Rule 5635(b), (4) to approve the LeonaBio, Inc. 2026 Equity Incentive Plan, (5) to amend the Company's amended and restated certificate of incorporation to increase the number of authorized shares of common stock and (6) to adjourn the Special Meeting, if necessary or appropriate in the view of the board of directors, including to solicit additional proxies if there are insufficient votes at the time of the Special Meeting to adopt any of Proposal 1, Proposal 2, Proposal 3, Proposal 4 or Proposal 5.

At the Special Meeting, the Company's stockholders voted on each proposal presented, as described in the Proxy Statement, and approved each proposal. No other items were presented for stockholder approval at the Special Meeting.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure.

None.

Item 9A. Controls and Procedures.

Evaluation of disclosure controls and procedures

Our disclosure controls and procedures are designed to ensure that information we are required to disclose in reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in SEC rules and forms, and that such information is accumulated and communicated to our management, including our chief executive officer and principal financial and accounting officer, as appropriate, to allow timely decisions regarding required disclosure.

Our management, with the participation and supervision of our chief executive officer and our principal financial and accounting officer, have evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) as of the end of the period covered by this Annual Report on Form 10-K. Based on such evaluation, our chief executive officer and principal financial and accounting officer have concluded that as of such date, our disclosure controls and procedures were, in design and operation, effective at a reasonable assurance level.

Management's annual report on internal control over financial reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Exchange Act Rule 13a-15(f) and Rule 15d-15(f). Our management conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2025 based on the criteria established in Internal Control - Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission. As a result of that assessment, our management has concluded that our internal control over financial reporting was effective as of December 31, 2025.

This Annual Report on Form 10-K does not include an attestation report of our independent registered public accounting firm on our internal control over financial reporting due to us qualifying as a non-accelerated filer.

Changes in Internal Control

There were no changes in our internal control over financial reporting identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the period covered by this Annual Report on Form 10-K that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Inherent Limitations on the Effectiveness of Controls

The effectiveness of any system of internal control over financial reporting, including ours, is subject to inherent limitations, including the exercise of judgment in designing, implementing, operating, and evaluating the controls and procedures, and the inability to eliminate misconduct completely. Accordingly, in designing and evaluating the disclosure controls and procedures, management recognizes that any system of internal control over financial reporting, including ours, no matter how well designed and operated, can only provide reasonable, not absolute assurance of achieving the desired control objectives. Moreover, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate. We intend to continue to monitor and upgrade our internal controls as necessary or appropriate for our business but cannot assure you that such improvements will be sufficient to provide us with effective internal control over financial reporting.

Item 9B. Other Information.

During our last fiscal quarter, no director or officer, as defined in Rule 16a-1 (f) of the Exchange Act, adopted or terminated a “Rule 10b5-1 trading arrangement” or any “non-Rule 10b5-1 trading arrangement,” each as defined in Item 408 of Regulation S-K.

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections.

Not applicable.

PART III

Item 10. Directors, Executive Officers and Corporate Governance

Composition of the Board of Directors

Our board of directors currently consists of eight directors, seven of whom are independent under the listing standards of The Nasdaq Stock Market LLC ("Nasdaq"). Our board of directors is divided into three classes with staggered three-year terms. Thus, at each annual meeting of stockholders, a class of directors will be elected for a three-year term to succeed the class whose term is then expiring.

The following table sets forth the names, ages as of March 24, 2026, and certain other information for each of our directors:

Name	Age	Position(s)
Joseph Edelman[1]	70	Director
John M. Fluke, Jr.[2]	83	Director
James A. Johnson[3][4][5]	69	Director
Barbara Kosacz[1][6]	68	Director
Mark Litton, Ph.D.	58	President, Chief Executive Officer, and Director
Michael Panzara, M.D.,M.P.H.[1][7]	58	Director
Grant Pickering[3]	58	Director
Kelly A. Romano[2][8]	64	Chairwoman of the Board of Directors

(1) Member of our nominating and corporate governance committee.

(2) Member of our audit committee.

(3) Member of our compensation committee.

(4) Chairman of our audit committee.

(5) Member of our compliance committee.

(6) Chairwoman of our compensation committee.

(7) Chairman of our compliance committee.

(8) Chairwoman of our nominating and corporate governance committee.

There are no family relationships among any of the directors or executive officers. Pursuant to the terms of the PIPE Securities Purchase Agreement, each of Commodore and TCGX are entitled to designate one board member, subject to certain conditions, and we have agreed to use our reasonable best efforts to cause the resignation of two (2) current members of our board of directors by the six (6) month anniversary of the PIPE Closing Date in connection with such designations. Refer to Item 13 of this report for additional information.

Joseph Edelman

Joseph Edelman has served on our board of directors since May 2020. Mr. Edelman is the founder and chief executive officer of Perceptive Advisors LLC ("Perceptive Advisors"), a hedge fund that specializes in investing in biotechnology stocks, and serves on the board of directors of Perceptive Capital Solutions Corp. He founded Perceptive Advisors in 1999, aiming to support progress in the life sciences industry by identifying opportunities and directing financial resources toward the most promising technologies in modern healthcare. Mr. Edelman earned an M.B.A. from the Leonard N. Stern School of Business at New York University in 1986 and a B.A., magna cum laude, in psychology from the University of California San Diego in 1978. We believe that Mr. Edelman's experience as a board member and investor in many successful biotechnology companies qualifies him to serve on our board of directors

John M. Fluke, Jr.

John M. Fluke, Jr. has served on our board of directors since December 2014. Mr. Fluke is chairman of Fluke Capital Management, L.P., which he founded in 1976, and was chairman and chief executive officer of the John Fluke Manufacturing Co. until 1990. Mr. Fluke previously served on the boards of PACCAR Inc., CellCyte Genetics Corporation, Cell Therapeutics, Primus International, and American Seafoods Group. Mr. Fluke is a current trustee of the Greater Seattle Chamber of Commerce (formerly serving as its chairman), and previously served as chairman of the Washington State China Relations Council and a trustee emeritus of the Museum of Flight. He also previously served as chairman of the Washington Technology Center at the University of Washington, which is an organization responsible for managing technology transfers from public universities in Washington state to the private sector for commercialization. Mr. Fluke has also served as chairman of the trustees of Junior Achievement of Washington and president of the Seattle Council of Boy Scouts of America. Mr. Fluke earned an M.S. in electrical engineering from Stanford University in 1966 and a B.S. in electrical engineering from the University of Washington in 1964. We believe that Mr. Fluke's extensive leadership experience and background as an investor in many successful companies qualifies him to serve on our board of directors.

James A. Johnson

James A. Johnson has served on our board of directors since August 2020. Mr. Johnson previously served as the chief financial officer of Nohla Therapeutics, a cell therapy company, from January 2018 to August 2019. Prior to Nohla, Mr. Johnson served as the chief financial officer of NanoString Technologies, a provider of life science tools for translational research and molecular diagnostics, from October 2012 to December 2017. During his tenure as chief financial officer at NanoString, Mr. Johnson oversaw strategic and corporate finance activities from private stage through the company's initial public offering and additional rounds of financing, marking the third initial public offering in his career as a chief financial officer. Prior to joining NanoString, Mr. Johnson served as chief financial officer of Relypsa, Inc., a clinical-stage biopharmaceutical company. Prior to Relypsa, Mr. Johnson served for nearly 10 years as chief financial officer of ZymoGenetics, Inc., until the company was acquired by Bristol-Myers Squibb in October 2010. Previously, he served for seven years as chief financial officer of Targeted Genetics Corporation (renamed Armata Pharmaceuticals) and as Vice President of Finance at Immunex Corporation during its evolution from product development to commercial operations. Mr. Johnson received a B.A. in business administration from the University of Washington in 1979. We believe that Mr. Johnson's depth of experience in the biopharmaceuticals industry, including as chief financial officer of a number of publicly traded biopharmaceutical companies, qualifies him to serve on our board of directors.

Barbara Kosacz

Barbara Kosacz has served on our board of directors since March 2021. Ms. Kosacz served as chief operating officer and general counsel at Kronos Bio, Inc., a clinical-stage biopharmaceutical company, from July 2020 until February 2024. Prior to joining Kronos Bio, Ms. Kosacz was a partner at Cooley LLP from November 1997 to December 2000, and again from November 2002 until July 2020, where she led the international life sciences practice. Ms. Kosacz has more than 25 years of experience in counseling clients in the life sciences arena, ranging from early stage startups to larger public companies, venture funds, investment banks, and non-profit institutions. She has served as a member of the BIO Emerging Companies' Section Governing Board, the Board of Trustees of the Keck Graduate Institute, the business advisory board of Locust Walk Partners, and as a speaker at multiple life sciences-related conferences, as well as guest lecturer at the University of California, Berkeley, Stanford University, Columbia University, and the University of Pennsylvania about biotechnology law, biotech business models, corporate partnering negotiations and deal structures, and bioethics. Recognized by Best Lawyers in America since 2008 and as Biotechnology Lawyer of the Year in 2018, Ms. Kosacz was listed as a "leading lawyer" for healthcare and life sciences in the 2018 Legal 500, as a "Band 1" attorney in the 2018 edition of Chambers USA: America's Leading Lawyers for Business and recognized as a "highly recommended transactions" lawyer by IAM Patent 1000 for her "nearly three decades advising diverse companies in the industry at a deeply strategic and commercial level and overseeing their most complex and profitable deals." Ms. Kosacz is a member of the board of directors of XOMA Royalty Corporation, a public biotechnology royalty aggregation company, and a member of the board of directors

of the Scripps Research Institute. She also served as a member of the board of directors of Phoenix Biotech Acquisition Corp., a blank check company formed for the purpose of acquiring or merging with one or more businesses, from October 2021 to February 2024. Ms. Kosacz received her B.A. from Stanford University and her J.D. from the University of California, Berkeley School of Law. We believe that Ms. Kosacz's extensive experience as an advisor to life sciences companies qualifies her to serve on our board of directors.

Mark Litton

Mark Litton, Ph.D., has served as our president and chief executive officer and member of our board of directors since October 2021 and previously served as our chief operating officer from July 2019 to October 2021. Prior to joining us, Dr. Litton served as the president and chief operating officer of Alpine Immune Sciences, Inc., a publicly traded biotechnology company, from August 2018 to April 2019. Dr. Litton served as the chief business officer, treasurer, and secretary from 2004 to 2018 of Alder BioPharmaceuticals, Inc., a publicly traded biopharmaceutical company co-founded by Dr. Litton in 2004, which was acquired by Lundbeck A/S in October 2019. From 1999 to 2004, Dr. Litton served as vice president of business development for Celltech Group, where he was responsible for securing, commercializing, and partnering on numerous novel discoveries and therapeutic programs. In 1999, Dr. Litton joined Celltech Group as an employee of Chiroscience Group plc and was later promoted to vice president of business development after Chiroscience's merger with Celltech Group in 1999. From 1997 to 1999, Dr. Litton served as the manager of business development for Ribozyme Pharmaceuticals Inc. (now Sirna Therapeutics, Inc.), a biopharmaceutical company and wholly owned subsidiary of Alnylam Pharmaceuticals, Inc., where he helped form relationships with Eli Lilly and Company, Roche Bioscience and GlaxoWellcome plc (now GlaxoSmithKline plc) a biopharmaceutical company. From 1991 to 1994, Dr. Litton served as a research associate for DNAX Research Institute, a research facility of Schering-Plough (now Merck & Co., a publicly traded pharmaceutical company). Dr. Litton earned a Ph.D. in immunology from Stockholm University in 1997, an M.B.A. from Santa Clara University in 1994 and a B.A. in biochemistry and molecular biology from the University of California Santa Cruz in 1990. We believe that Dr. Litton's experience in the biopharmaceutical industry and the perspective and experience he brings as our chief executive officer qualifies him to serve on our board of directors.

Michael Panzara, M.D., M.P.H.

Michael Panzara, M.D., M.P.H., has served on our board of directors since March 2022. Dr. Panzara served as chief medical officer at Neurvati Neurosciences, Inc., a Blackstone Life Sciences portfolio company, from October 2022 to March 2026. Previously, Dr. Panzara served as chief medical officer and head of therapeutics discovery and development at Wave Life Sciences Ltd., a publicly traded genetic medicines company, since May 2020, where he previously served as chief medical officer from November 2018 to May 2020 and as franchise lead of neurology from July 2016 to November 2018. Prior to joining Wave Life Sciences, Dr. Panzara served in various roles at Sanofi Genzyme, including most recently as head of multiple sclerosis, neurology and ophthalmology therapeutic area for global development. Dr. Panzara has held numerous other positions in the healthcare and biopharmaceutical industries, including vice president and chief medical officer in neurology at Biogen, and instructor in neurology at Harvard Medical School with clinical appointments at Brigham & Women's Hospital and Massachusetts General Hospital. Dr. Panzara earned an M.P.H from Harvard School of Public Health in 2002, an M.D. from Stanford University School of Medicine in 1994, and a B.A. in biology from the University of Pennsylvania in 1989. We believe that Dr. Panzara's extensive experience in the healthcare and biopharmaceutical industries qualifies him to serve on our board of directors.

Grant Pickering

Grant Pickering has served on our board of directors since January 2022. Mr. Pickering co-founded and serves as chief executive officer and as a member of the board of directors at Vaxcyte, Inc., a publicly traded biotechnology company, since November 2013. Mr. Pickering also served as strategic advisor at Atreca, Inc., a publicly traded biotechnology company, from May 2013 to April 2015. Prior to joining Vaxcyte, Mr. Pickering was chief executive officer of Mymetics Corporation, a publicly traded

biotechnology company. Prior to that, Mr. Pickering served as executive-in-residence at Kleiner Perkins, a venture capital firm, while serving as the chief executive officer and as a member of the board of directors at Juvaris BioTherapeutics, Inc., a biopharmaceutical company. Prior to that he served as senior vice president of operations of Dendreon Corporation, a publicly traded biotechnology company. Mr. Pickering earned an M.B.A. from Georgetown University in 1997 with high honors and a B.S. in marketing from Penn State University in 1989. We believe that Mr. Pickering's experience in the healthcare, biopharmaceutical, and biotechnology industries qualifies him to serve on our board of directors.

Kelly A. Romano

Kelly A. Romano has served as chairwoman of our board of directors since August 2021 and as a member of our board of directors since December 2020. Ms. Romano brings over 30 years of executive operating experience with technology companies, with a background in commercial buildings and aerospace. Ms. Romano is the chief executive officer of BlueRipple Capital, LLC, a consultancy firm she founded in May 2018 and the founder and chair of the board of directors of Grizzly MEP since August 2025. Ms. Romano previously served as an operating partner at AE Industrial Partners, a private equity firm focused on aerospace and industrial investments, from August 2020 to July 2023. Ms. Romano also served on the Executive Advisory Board at Gryphon Investors, a middle-market private equity firm, from December 2016 to November 2023. Previously, she spent 32 years working at United Technologies Corp. (UTC) and held a number of senior executive global positions, including president of Intelligent Building Technologies in UTC Building & Industrial Systems and president of Building Systems & Services at Carrier Corporation. Ms. Romano is also a member of several boards of directors, including UGI Corporation, Dorman Products, Inc., and Potter Global Technologies. Ms. Romano earned a B.S. in business administration from the State University of New York at Buffalo, an M.B.A. from Syracuse University, and is a graduate of the Northwestern University Kellogg School of Management's Corporate Board Governance Executive Program, as well as senior executive programs at Darden School of Management, University of Virginia. We believe that Ms. Romano's executive operating experience and aptitude for understanding growing companies qualifies her to serve on our board of directors

Executive Officers

The following table sets forth the names, ages and positions of our executive officers as of March 24, 2026.

Name	Age	Position(s)
Mark Litton, Ph.D.	58	President, Chief Executive Officer and Director
Javier San Martin, M.D.	61	Chief Medical Officer
Kevin Church, Ph.D.	41	Chief Scientific Officer
Mark Worthington	59	General Counsel, Chief Compliance Officer and Corporate Secretary
Robert Renninger	42	Chief Financial Officer

There are no family relationships among any of the directors or executive officers.

The following is biographical information for our executive officers, other than Dr. Litton, whose biographical information is included above.

Javier San Martin

Javier San Martin has served as our Chief Medical Officer since April 2024. Prior to that, he served as Chief Medical Officer at Arrowhead Pharmaceuticals, a biopharmaceutical company, from November 2019 to January 2024, where he guided development teams to advance that company's RNAi-based therapeutics in the metabolic and liver disease area. Prior to Arrowhead, he served as Senior Vice President and Head of Global Clinical Development at Ultragenyx Pharmaceutical, a biopharmaceutical company, from 2013 to 2019, where he led the development of Crysvita® (burosumab-twza), the first drug approved to treat the rare, inherited disease of x-linked hypophosphatemia. Before that, Dr. San Martin served as Senior Vice President of Clinical Development at Alder Biopharmaceuticals, from 2012 to 2013, where he managed medical, regulatory, and clinical operations. Earlier, he led two major development programs as Global Development Leader for Amgen's Bone Therapeutic Area and directed the anti-sclerostin antibody clinical program Eventiy® (romosozumab-aqqg) through the end of Phase 2 and was responsible for development and approval of Prolia® (denosumab) for the treatment of postmenopausal osteoporosis. Prior to Amgen, Dr. San Martin spent seven years at Eli Lilly working on Phase 3b and Phase 4 clinical trials to support the successful launch and medical affairs activities for Evista® and Forteo®. Dr. San Martin received his medical degree from the University of Buenos Aires Medical School and completed his residence in internal medicine at CEMIC University of Buenos Aires.

Kevin Church, Ph.D.

Kevin Church, Ph.D., has served as our chief scientific officer since January 2023. Prior to this, Dr. Church held various roles at the Company, including executive vice president of research from October 2021 to January 2023, vice president of discovery from July 2020 to October 2021, director of discovery from July 2018 to July 2020, senior research scientist from February 2018 to July 2018, and research scientist from July 2016 to February 2018. Dr. Church has research experience in diverse fields of study including neurodegenerative diseases, wound healing, and cancer. Dr. Church earned his Ph.D. in molecular biosciences from Washington State University in 2016, and prior to that earned his B.S. in microbiology from the University of Idaho in 2006. While in graduate school, Dr. Church was recognized for excellence in his graduate teaching assistantships. Dr. Church's graduate work primarily focused on the development of novel therapeutics for the treatment of pancreatic cancer, but also included research relating to the treatment of diabetic ulcers and neurodegenerative diseases such as Parkinson's disease dementia and Alzheimer's disease.

Mark Worthington

Mark Worthington has served as our general counsel since June 2021, after working with us as outside corporate counsel for several years. Prior to joining the Company, Mr. Worthington served as a partner with Summit Law Group in Seattle, Washington from September 1997 to May 2021, where he practiced corporate and securities law, chaired the corporate/securities practice and served as the co-managing partner. Mr. Worthington has advised private and public life sciences and other companies on a wide range of corporate governance, compliance and transactional matters, including public offerings, mergers and acquisitions, and joint ventures, and worked closely with executive management teams on strategic business and legal matters. Mr. Worthington received his J.D. from University of California College of the Law, San Francisco, in 1993 and his B.A. in American Studies from Stanford University in 1988

Robert Renninger

Robert Renninger has served as our Chief Financial Officer since December 2025. From February 2025 to December 2025, he served as senior vice president, finance and accounting, and previously served as our vice president of finance from January 2022 to February 2025, as our senior director of finance from September 2020 to January 2022, and as our director of finance from July 2020 to September 2020. Mr. Renninger served as financial controller of Infobip (formerly OpenMarket), a global communications platform, from July 2019 to July 2020. He also served as technical controller of Baker Hughes, an energy company, from September 2017 to May 2019. Mr. Renninger also served in various roles at Ernst & Young, LLP, a global accounting firm, from September 2007 through September 2017, including most recently as senior manager. Mr. Renninger received his Master of accounting from the University of Michigan in 2007 and his B.A. in accounting from Seattle University in 2006. Mr. Renninger has over 15 years of experience serving in various finance, accounting, and auditing positions for companies in the pharmaceutical, technology, and energy sectors.

Governance

Code of Business Conduct and Ethics

We have adopted a written code of business conduct and ethics that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. A copy of the code is posted on the investor section of our website at https://investors.leonabio.com/corporate-governance/governance-overview. We will post amendments to our code of business conduct and ethics or waivers of our code of business conduct and ethics for directors and executive officers on the same website.

Audit Committee

The members of our audit committee are Messrs. Johnson and Fluke and Ms. Romano, each of whom is a non-employee member of our board of directors. Our audit committee chairman, Mr. Johnson, is our audit committee financial expert, as that term is defined under the SEC rules implementing Section 407 of the Sarbanes-Oxley Act of 2002, and possesses financial sophistication, as defined under the rules of Nasdaq. Our audit committee oversees our corporate accounting and financial reporting process and assists our board of directors in monitoring our financial systems. Our audit committee operates under a written charter that specifies its duties and responsibilities and satisfies the applicable listing standards of Nasdaq. A copy of the charter of our audit committee is available on our website at https://investors.leonabio.com/corporate-governance/governance-overview. Our board of directors has determined that each of Messrs. Johnson and Fluke and Ms. Romano is independent for audit committee purposes, as that term is defined in the rules of the SEC and the applicable Nasdaq rules, and has sufficient knowledge in financial and auditing matters to serve on the audit committee. During our fiscal year ended December 31, 2025, our audit committee held four meetings (including regularly scheduled and special meetings).

Our audit committee:

- selects, retains, compensates, evaluates, oversees and, where appropriate, terminates our independent registered public accounting firm;
- reviews and approves the scope and plans for the audits and the audit fees and approves all non-audit and tax services to be performed by the independent audit;
- evaluates the independence and qualification of the independent registered public accounting firm;
- reviews internal controls and integrity of financial statements;
- reviews financial information presentation, earnings press releases and guidance;
- oversees the design, implementation and performance of our internal audit function, if any;
- sets hiring policies with regard to the hiring of employees and former employees of our independent auditor and oversees compliance with such policies;
- reviews and monitors compliance with our Investment Policy and approves any amendments or deviations;
- reviews, approves and monitors related party transactions;
- develops, approves, reviews and monitors compliance with our code of business conduct and ethics;
- adopts and oversees procedures to address complaints regarding accounting, internal accounting controls or auditing matters;
- reviews and discusses with our management and the independent auditor our compliance with various laws;
- reviews and discusses with management, our independent auditor and our compliance committee, guidelines and policies to identify, monitor, and address enterprise risks, including the risks and exposures associated with cybersecurity, information security and privacy matters, and risks relating to securities laws, anti-corruption compliance and conflicts of interest, and conducts, in conjunction with management and our compliance committee, compliance risk analyses;
- engages independent legal, accounting and other advisors;
- determines appropriate funding for compensation to independent registered accounting firms, advisors and related expenses; and
- reviews the adequacy of the audit committee charter and recommends any proposed changes to our board of directors.

Insider Trading Policy

We have adopted an insider trading policy that governs the purchase, sale, and other dispositions of our securities by our directors, officers, employees and other covered individuals, that we believe is reasonably designed to promote compliance with applicable insider trading laws, rules and regulations and the listing standards of Nasdaq. A copy of our insider trading policy was filed as an exhibit to this Annual Report. In addition, with regard to trading in our own securities, it is our policy to comply with the federal securities laws and the applicable exchange listing requirements. Under our insider trading policy, our employees, including our executive officers, consultants, contractors, advisors, and the members of our board of directors are prohibited from, directly or indirectly, among other things, (1) engaging in short sales, (2) trading in publicly traded options, such as puts and calls, and other derivative securities with respect to our securities (other than stock options, restricted stock units and other compensatory awards issued to such individuals by us), (3) purchasing financial instruments (including prepaid variable forward

contracts, equity swaps, collars and exchange funds), or otherwise engaging in transactions that hedge or offset, or are designed to hedge or offset, any decrease in the market value of equity securities granted to them by us as part of their compensation or held, directly or indirectly, by them, (4) pledging any of our securities as collateral for any loans or as part of any other pledging transaction and (5) holding our securities in a margin account.

Item 11.Executive Compensation

Processes And Procedures for Compensation Decisions

Our executive compensation programs are designed to:

- attract, motivate, incentivize and retain employees at the executive level who contribute to our long-term success;
- provide compensation packages to our executives that are fair and competitive and reward high levels of performance and the achievement of our business objectives; and
- more closely align our executives' interests with those of our stockholders by focusing on long-term equity incentives that correlate with the growth of sustainable long-term value for our stockholders.

Our compensation committee is responsible for making compensation decisions for executive officers other than our chief executive officer, but may, in its discretion, choose to make compensation recommendations to the full board of directors.

Our compensation committee has retained Pearl Meyer & Partners, LLC, a compensation consulting firm, to provide recommendations based on research and analysis of executive compensation in companies in similar industries at a similar size and stage of corporate development, with the goal of ensuring that the compensation we offer to our executives is competitive and fair. Typically, our chief executive officer and principal financial and accounting officer will prepare and present recommendations at compensation committee meetings based on the compensation consultant recommendations, our chief executive officer's own assessment of Company and individual performance and incentive and retention needs, and a representative from the compensation consultant will usually be present in the meetings to respond to committee questions. Our compensation committee considers the recommendations for cash and stock-based compensation and approves such compensation for the executive team, excluding the chief executive officer, and recommends such compensation to the board of directors for the chief executive officer. With regard to incentive compensation, our compensation committee evaluates the achievement of defined goals by the executive team, excluding the chief executive officer, and recommends to the board of directors with respect to the achievement of defined goals for the chief executive officer.

Executive Compensation

This section discusses the material components of the executive compensation program for our named executive officers who are named in the subsection titled "2024 – 2025 Summary Compensation Table" below. For 2025, our "named executive officers" and their positions were as follows:

- Mark Litton, Ph.D., our president and chief executive officer;
- Javier San Martin, M.D., our chief medical officer; and
- Mark Worthington, our general counsel, chief compliance officer and corporate secretary.

All share amounts in this section reflect the Company's 10-for-1 reverse stock split that became effective on September 17, 2025.

2024 – 2025 Summary Compensation Table

The following table represents information regarding the total compensation awarded to, earned by or paid to our named executive officers for the years ended December 31, 2025 and December 31, 2024.

Name and Principal Position	Year	Salary ($)	Bonus ($) (1)	Stock Awards ($) (2)	Option Awards ($) (3)	Non-Equity Incentive Plan Compensation ($) (4)	All Other Compensation ($)	Total ($)
Mark Litton, Ph.D., President and Chief Executive Officer	2025	625,000	171,875	83,720	518,135	—	25,085 (5)	1,423,814
	2024	625,000	—	138,353	1,991,260	395,313	25,085 (5)	3,175,009
Javier San Martin, M.D., Chief Medical Officer	2025	500,000	100,000	27,907	182,143	—	85 (6)	810,134
Mark Worthington, General Counsel, Chief Compliance Officer and Corporate Secretary	2025	450,000	90,000	21,541	140,601	—	29,335 (5)	731,477
	2024	450,000	—	49,489	606,498	207,000	24,523 (5)	1,337,509

(1) In January 2025, the compensation committee, and with respect to Dr. Litton, the board of directors, approved a retention bonus program under the Company's Executive Incentive Compensation Plan, which program was amended in February 2025. As amended, the retention bonus program provided that each employee would be entitled to receive a cash bonus equal to the greater of (i) 50% of the employee's 2025 annual target bonus and (ii) 10% of the employee's 2025 annual base salary, due on the earliest of (i) September 20, 2025, (ii) consummation of a contemplated strategic transaction and (iii) the date the Company wound up its affairs. The bonuses reflected in this column were paid to the executives on September 30, 2025.

(2) In accordance with Securities and Exchange Commission rules, amounts in this column reflect the aggregate grant date fair value of time-based and performance RSU awards granted during 2024 and 2025 computed in accordance with ASC 718, rather than the amounts paid or realized by the named executive officer. The performance RSU award amounts reflect the probable outcome of the performance conditions, in accordance with ASC 718. For a discussion of valuation assumptions, see Note 9 and the sections titled "Stock-based Compensation" to our financial statements included in this Annual Report. The fair value of the performance RSU awards at the grant date has been calculated assuming that the highest level of performance conditions will be achieved for each award.

(3) In accordance with Securities and Exchange Commission rules, amounts in this column reflect the aggregate grant date fair value of stock options granted during 2024 and 2025 computed in accordance with ASC 718, rather than the amounts paid or realized by the named executive officer. For a discussion of valuation assumptions, see Note 9 and the sections titled "Stock-based Compensation" to our financial statements included in this Annual Report.

(4) Represents cash bonuses earned by the named executive officers pursuant to our Executive Incentive Compensation Plan for 2024 performance, paid in 2025.

(5) Represents payments made on the executive's behalf for basic life insurance and contributions to vested and unvested defined contribution plans.

(6) Represents payments made on the executive's behalf for basic life insurance.

Outstanding Equity Awards at December 31, 2025

The following table shows grants of stock options and RSUs to each of our named executive officers outstanding at December 31, 2025.

	Number of Securities Underlying Unexercised Options				Stock Awards (unvested)		
Name	Vesting Commencement Date	Exercisable (#)	Unexercisable (#)	Option Exercise Price ($)	Expiration Date	Number of Shares or Units (#)	Market Value of Shares or Units ($)(4)
Mark Litton, Ph.D.	7/1/2019	10,088	—	13.50	8/14/2029	—	—
	8/26/2020	4,665	—	170.00	9/16/2030	—	—
	1/8/2021	8,499	—	211.50	2/17/2031	—	—
	1/27/2022	39,166	833 (1)	99.10	1/26/2032	—	—
	1/27/2023	41,319	1,180 (2)	41.10	1/26/2033	—	—
	2/15/2024	29,795	35,204 (1)	36.60	2/14/2034	—	—
	10/3/2024	32,500	—	4.26	10/2/2034	—	—
	3/3/2025	6,259	27,121 (1)	3.77	3/2/2035	—	—
	3/3/2025	—	—	—	3/2/2035	22,254 (3)	168,463
	9/25/2025	—	133,526 (5)	3.81	9/24/2035	—	—
Javier San Martin, M.D.	4/15/2024	16,675	23,325 (6)	20.60	4/14/2034	—	—
	10/1/2024	14,000	—	4.50	9/30/2034	—	—
	3/3/2025	2,088	9,039 (1)	3.77	3/2/2035	—	—
	3/3/2025	—	—	—	3/2/2035	7,418 (3)	56,154
	9/24/2025	—	44,508 (5)	4.07	9/23/2035	—	—
Mark Worthington	6/1/2021	14,999	—	153.40	11/2/2031	—	—
	1/18/2022	14,687	312 (1)	106.40	1/17/2032	—	—
	1/19/2023	14,583	416 (2)	33.70	1/18/2033	—	—
	2/14/2024	10,087	11,912 (1)	32.60	2/13/2034	—	—
	10/1/2024	10,999	—	4.50	9/30/2034	—	—
	3/3/2025	1,611	6,977 (1)	3.77	3/2/2035	—	—
	3/3/2025	—	—	—	3/2/2035	5,726 (3)	43,346
	9/24/2025	—	34,358 (5)	4.07	9/23/2035	—	—

(1) Stock option vests over four years, with 1/48 vesting on the monthly anniversary of the vesting commencement date, subject to continued service with us through the applicable vesting date.

(2) Stock option vests over three years, with 1/36 vesting on the monthly anniversary of the vesting commencement date, subject to continued service with us through the applicable vesting date.

(3) 100% of the RSUs will vest on the one-year anniversary of the vesting commencement date.

(4) The market value of RSUs that have not vested is based on the closing price of the Company's common stock on Nasdaq on December 31, 2025, which was $7.57 per share.

(5) Stock option scheduled to vest only upon satisfaction of both a milestone requirement and a service-based requirement. The milestone requirement was satisfied on December 23, 2025. The service-based requirement will be satisfied as to 50% of the total number of shares granted under the option on December 23, 2026 and as to 1/36th of the remaining 50% on each monthly anniversary thereafter.

(6) Stock option vests as to 25% on the first anniversary of the vesting commencement date, with the remaining stock options vesting monthly thereafter.

Executive Employment Arrangements

Each of our named executive officers has executed our standard form of confidential information, invention assignment and arbitration agreement.

Dr. Mark Litton

In September 2020, we entered into a confirmatory employment letter with Dr. Litton, our then chief operating officer and current president and chief executive officer. The confirmatory employment letter has no specific term and provides that Dr. Litton is an at-will employee and superseded all prior employment agreements between Dr. Litton and us.

In February 2025, the board of directors, upon recommendation of our compensation committee, approved a continuation of Dr. Litton's annual base salary at $625,000, effective as of January 1, 2025. In March 2026, the board of directors, upon recommendation of our compensation committee, approved an increase of Dr. Litton's annual base salary to $667,000, effective as of January 1, 2026. Dr. Litton's target annual bonus amount under the Company's bonus plan remains at 55% of his annual base salary.

Javier San Martin

In March 2024, we entered into an offer letter with Dr. San Martin, our chief medical officer. The offer letter has no specific term and provides that Dr. San Martin is an at-will employee. In March 2026, our compensation committee approved an increase of Dr. San Martin's annual base salary to $540,000, effective as of January 1, 2026. Dr. San Martin's target annual bonus amount under the Company's bonus plan remains at 40% of his annual base salary.

Mark Worthington

In May 2021, we entered into an offer letter with Mr. Worthington, our general counsel, chief compliance officer and corporate secretary. The offer letter has no specific term and provides that Mr. Worthington is an at-will employee. In March 2026, our compensation committee approved an increase of Mr. Worthington's annual base salary to $475,000, effective as of January 1, 2026. Mr. Worthington's target annual bonus amount under the Company's bonus plan remains at 40% of his annual base salary.

Change In Control and Severance Agreements

In June 2021 we entered into a change in control and severance agreement with Mr. Worthington, in January 2022 we entered into an amended change in control and severance agreement with Dr. Litton, and in April 2024 we entered into a change in control and severance agreement with Dr. San Martin. These agreements provide for certain severance and change in control benefits as described below.

If the employment of a named executive officer with whom we have entered into a change in control and severance agreement is terminated outside the period beginning one month prior to the date of a change in control and ending 12 months following that change in control (the "Change in Control Period"), either (1) by the Company without "cause" (excluding by reason of death or disability) or (2) by the named executive officer for "good reason" (as such terms are defined in the named executive officer's change in control and severance agreement), the named executive officer will receive the following benefits if such named executive officer timely signs and does not revoke a release of claims in our favor:

- a lump-sum payment equal to 9 months (or 12 months in the case of Dr. Litton) of the named executive officer's annual base salary as in effect immediately prior to such termination (or if such termination is due to a resignation for good reason based on a material reduction in base salary, then as in effect immediately prior to the reduction);
- payment of premiums for coverage under the Consolidated Omnibus Budget Reconciliation Act of 1985, as amended (COBRA), for the named executive officer and his eligible dependents, if any, for up to 9 months (or up to 12 months for Dr. Litton); and
- in the case of Dr. Litton, 25% accelerated vesting and exercisability of the shares subject to the stock option award granted to Dr. Litton on August 15, 2019, that are outstanding and unvested as of the date of such termination.

If, during the Change in Control Period, the employment of a named executive officer with whom we have entered into a change in control and severance agreement is terminated either (1) by the Company without cause (excluding by reason of death or disability) or (2) by the named executive officer for good reason, the named executive officer will receive the following benefits if the named executive officer timely signs and does not revoke a release of claims in our favor:

- a lump-sum payment equal to 12 months (or 18 months in the case of Dr. Litton) of the named executive officer's annual base salary as in effect immediately prior to such termination (or if

such termination is due to a resignation for good reason based on a material reduction in base salary, then as in effect immediately prior to the reduction) or if greater, at the level in effect immediately prior to the change in control;

- a lump-sum payment equal to 100% (or 150% in the case of Dr. Litton) of the named executive officer's target annual bonus as in effect for the fiscal year in which such termination occurs or if greater, at the level in effect immediately prior to the change in control;
- payment of premiums for coverage under COBRA for the named executive officer and the named executive officer's eligible dependents, if any, for up to 12 months (or up to 18 months for Dr. Litton); and
- 100% accelerated vesting and exercisability of all Company equity awards with service-based vesting (but that are not subject to performance-based vesting) that are outstanding and unvested as of the date of the qualifying termination.

In addition, the change in control and severance agreement with Dr. Litton provides for 100% accelerated vesting and exercisability of Company equity awards granted under our 2014 Equity Incentive Plan and held by Dr. Litton to the extent such awards are not assumed or substituted for by the successor corporation in a change in control.

If any of the amounts provided for under these change in control and severance agreements or otherwise payable to the named executive officer would constitute "parachute payments" within the meaning of Section 280G of the Internal Revenue Code and could be subject to the related excise tax, the named executive officer would be entitled to receive either full payment of benefits or such lesser amount which would result in no portion of the benefits being subject to the excise tax, whichever results in the greater amount of after-tax benefits to the named executive officer. The change in control and severance agreements do not require us to provide any tax gross-up payments.

Under the change in control and severance agreement, "cause" generally means the named executive officer's: indictment or conviction of any felony or any crime involving dishonesty or moral turpitude; participation in any fraud against us or other dishonesty which is not the result of an innocent or inadvertent mistake by the named executive officer with respect to us; willful violation of his obligations to us after there has been delivered to the named executive officer a written demand for performance from the board of directors; continued violation or breach of any material written Company policy, agreement with us, or any statutory or fiduciary duty to us after we have delivered to the named executive officer a written notification of such violation or breach; or damaging or misappropriating or attempting to damage or misappropriate any of our property, including intellectual property.

Under the change in control and severance agreement, "good reason" generally means that the named executive officer resigns from the Company within 30 days following the end of our cure period (discussed below) as a result of any of the following that occurs without his consent: a material reduction in the named executive officer's duties or responsibilities that is inconsistent with his position, provided that a mere change of title alone will not constitute such a material reduction; the requirement that the named executive officer change his principal office to a facility that increases his commute by more than 40 miles from his commute to the location at which the named executive officer was employed prior to such change; or a material reduction in base salary or a material reduction in his employee benefits (other than (1) in connection with a general decrease in salary (or employee benefits, as applicable) of all similarly situated employees, and (2) following our change in control, to the extent necessary to make his salary (or employee benefits, as applicable) commensurate with those of our other employees or our successor entity or parent entity who are similarly situated with him). For a resignation to qualify as "good reason," the named executive officer also must provide written notice within 90 days following the initial

existence of the good reason condition, and we must have failed to materially remedy such event within 30 days after receipt of such notice.

Equity Awards

2025 Time-Based Options and RSUs

In February 2025, our compensation committee approved, or recommended that our board of directors approve, grants of stock options under our 2020 Equity Incentive Plan to our named executive officers. Dr. San Martin and Mr. Worthington were awarded stock options equal to 11,127 and 8,589 shares, respectively, which vest monthly over four years, subject to continued service with us through the applicable vesting date. In February 2025, our compensation committee recommended, and our board of directors approved, a grant of stock options under our 2020 Equity Incentive Plan of 33,381 shares to Dr. Litton, which vest monthly over four years, subject to continued service with us through the applicable vesting date. Each of the named executive officer's options are also subject to acceleration pursuant to the terms of his change in control and severance agreement, as described above.

In February 2025, our compensation committee approved, or recommended that our board of directors approve, grants of RSUs under our 2020 Equity Incentive Plan to our named executive officers. Dr. San Martin and Mr. Worthington were awarded 7,418 and 5,726 RSUs, respectively, which vested as to 100% on the one-year anniversary of the applicable vesting commencement date. In February 2025, our compensation committee recommended, and our board of directors approved, a grant of 22,254 RSUs under our 2020 Equity Incentive Plan to Dr. Litton, which vested as to 100% on the one-year anniversary of the applicable vesting commencement date. Each of the named executive officer's RSU awards are also subject to acceleration pursuant to the terms of his change in control and severance agreement, as described above.

Retention Awards

In October 2024, our compensation committee approved, or recommended that our board of directors approve, the grant of retention equity awards under our 2020 Equity Incentive Plan to each of Dr. Litton, Dr. San Martin and Mr. Worthington in the form of stock options and RSUs.

Our compensation committee awarded to Dr. San Martin 14,000 stock options and 14,000 RSUs and Mr. Worthington 11,000 stock options and 11,000 RSUs, and our board of directors awarded to Dr. Litton 32,500 stock options and 32,500 RSUs, which vested in accordance with the following schedule: one-third (1/3rd) of each award vested on each of December 31, 2024, June 30, 2025 and December 31, 2025, subject to continued service with us through the applicable vesting date.

In addition to the accelerated vesting provisions of our 2020 Equity Incentive Plan described below and, once the performance milestones were met, of the applicable named executive officer's change in control and severance agreement, as described above, upon a Qualifying Merger (as defined in the applicable award agreement and generally meaning a transaction with a privately held corporation the primary purpose of which is a go-public transaction for the private company, and where the Company or its successor is publicly traded following the transaction), each of the aforementioned retention equity awards would accelerate vesting in full, subject to the applicable named executive officer's continued service to us through the date of such Qualifying Merger.

In September 2025, the compensation committee approved, or, with respect to Dr. Litton, recommended that the board of directors approve, grants of stock options under our 2020 Equity Incentive Plan to each of Dr. Litton, Dr. San Martin and Mr. Worthington. Our compensation committee awarded to Dr. San Martin and Mr. Worthington 44,508 stock options and 34,358 stock options, respectively, and our board of directors awarded Dr. Litton 133,526 stock options. Such options were scheduled to vest only upon satisfaction of both a milestone requirement and a service-based requirement. In December 2025, the compensation committee determined that the milestone requirement

was met due to the Company then having cash resources of at least $30 million available for Phase 3 clinical development.

The service-based requirement will be satisfied as to fifty percent (50%) of the total number of shares granted under the stock options on the one-year anniversary of the compensation committee's determination that the milestone requirement was met and as to one thirty-sixth (1/36th) of the remaining fifty percent (50%) of the total number of shares granted under the stock options each month thereafter on the same day of the month as the date of the compensation committee's determination (and if there is no corresponding day, the last day of the month), subject to the recipient continuing to be a service provider through the applicable vesting dates.

In addition to the accelerated vesting provisions of our 2020 EIP described below and, once the performance milestones were met, of the applicable named executive officer's change in control and severance agreement, as described above, upon a Qualifying Merger (as defined in the applicable award agreement and generally having the same meaning as described above), each of the September 2025 retention equity awards will accelerate vesting in full, subject to the applicable named executive officer's continued service to us through the date of such Qualifying Merger. In addition, if, after the performance milestones were met, there is a sale or exclusive license or sublicense of a Company asset with respect to which all or a portion of the net proceeds are distributed to Company stockholders, the vesting of the September 2025 retention equity awards accelerate vesting in full, subject to the applicable named executive officer's continued service to us through the date of such asset transaction.

Certain RSU Awards Granted in Prior Years

Our compensation committee approved a grant of 3,000 RSUs to Mr. Worthington, and our board of directors approved a grant of 6,000 RSUs to Dr. Litton, in November 2021, under the Company's 2020 Equity Incentive Plan to provide additional performance incentives aligned with key strategic goals.

In December 2022, our compensation committee revised the vesting schedule of the RSU awards held by Mr. Worthington, and Dr. Litton due to changed circumstances relating to the performance goals under the original vesting schedule. At the time of such amendment, one third (1/3rd) of the number of shares subject to the RSU awards had vested upon the completion of the public readout of topline results of the Company's ACT-AD Phase 2 clinical trial in June 2022. The original vesting schedule provided that an additional one third (1/3rd) of the number of shares subject to the RSU awards would vest at the completion of the public readout of topline results of the Company's LIFT-AD Phase 2/3 clinical trial (the "LIFT-AD Readout"), and the remaining one third (1/3rd) of the number of shares subject to the RSU awards would be scheduled to vest six (6) months after the LIFT-AD Readout, in each case subject to the recipient's continued service with us through the applicable vesting date. The vesting schedule was amended with respect to the remaining two-thirds (2/3rds) of the shares subject to the RSU awards that remained unvested at the time of the amendment, to provide that one third (1/3rd) of the number of shares subject to the RSU awards would vest at the date our compensation committee determined that enrollment of the Company's LIFT-AD Phase 2/3 clinical trial had been completed (which occurred in January 2024), and the remaining one third (1/3rd) of the number of shares subject to the RSU awards would vest at the completion of the LIFT-AD Readout (which occurred in September 2024), in each case subject to the recipient's continued service with us through the applicable vesting date.

The RSU award agreements provided for 100% vesting acceleration in the event that, on or within 12 months following a Change in Control (within the meaning of our 2020 Equity Incentive Plan), the award recipient was terminated by the Company without "cause" (as defined in the recipient's award agreement).

Equity Incentive Plans

Under our 2020 Equity Incentive Plan and our 2024 Inducement Equity Incentive Plan unless otherwise specified in an award agreement for a particular award, all unvested options, RSUs and other equity awards vest in full and, if applicable, become exercisable, and performance-based awards would be deemed achieved at 100% of target, upon a “change in control” (as defined in the applicable plan) of the Company or a merger of the Company with or into another corporation or entity, unless the option or award is assumed or substituted for by the acquiring entity, and to the extent exercisable, would terminate if not exercised within the applicable period.

All awards granted under our 2014 Equity Incentive Plan to our named executive officers are fully vested. Under our 2014 Equity Incentive Plan, in the event of a “corporate transaction” as defined thereunder, these outstanding awards may be assumed, continued or substituted for by the surviving or acquiring corporation, or the awards may be cancelled for no consideration or in exchange for such cash consideration as our board of directors deems appropriate, including that an option may be cancelled for a payment equal to the difference between the value the named executive officer would have received upon exercise of the option and the option exercise price.

Our board of directors or the compensation committee, as administrator of our equity incentive plans, has the authority to provide for the accelerated vesting of any or all outstanding equity awards under the plans.

Clawback Policy

In February 2023, our board of directors adopted an executive compensation recovery ("clawback") policy applicable to our current and future former executive officers. This initial clawback policy provided us the right, within three years following the original filing date of the applicable financial statements, to recover certain compensation from executive officers in the event all or a portion of our financial statements were subject to a material negative restatement as the result of the gross negligence, intentional misconduct or fraud by an executive officer. We amended and restated our clawback policy in November 2023 to reflect the Nasdaq listing standards that became effective in October 2023 and again in September 2024. In accordance with the Nasdaq listing standard requirements, our amended and restated clawback policy provides that if we are required to prepare an accounting restatement due to our material noncompliance with any financial reporting requirement under the securities laws, including any required accounting restatement to correct an error in previously issued financial statements that is material to the previously issued financial statements, or that would result in a material misstatement if the error were corrected in the current period or left uncorrected in the current period, then we must recover from the covered executives any excess compensation covered by the amended and restated clawback policy. The September 2024 amendment and restatement of our clawback policy expanded the clawback policy to apply in the event that we are required to retract or correct scientific results. This expansion provides that we must also recover from the covered executives any excess compensation covered by the amended and restated clawback policy in the event that we are required to retract a scientific publication or correct a material scientific finding in a scientific publication (as determined by our board of directors or compliance committee), where such retraction or correction is due to fraudulent or intentional misconduct, gross negligence, or a material violation of a Company policy or the policy of a scientific publication related to scientific integrity. As described in more detail in our amended and restated clawback policy, excess compensation generally is incentive-based compensation that exceeds the amount the individual otherwise would have received had the compensation been determined based on the restated amounts. Excess compensation generally is covered by the amended and restated clawback policy if it is received by an individual during our three completed fiscal years immediately prior to the date we determine an accounting restatement or a retraction or correction of scientific results is required (or a legally authorized body, such as a court, directs us to prepare an accounting restatement), if the amounts were received after the individual became an executive officer, and if he or she served as an executive officer during the applicable performance period (and only if the amounts were received after October 2, 2023).

Executive Incentive Compensation Plan

Our Executive Incentive Compensation Plan is administered by our board of directors or a committee appointed by our board of directors. Our Executive Incentive Compensation Plan allows us to grant incentive awards, generally payable in cash, to employees selected by the administrator, including our named executive officers, based upon any performance goals that may be established by the administrator.

Under our Executive Incentive Compensation Plan, the administrator will determine any performance goals applicable to an award, which goals may include, without limitation, attainment of research and development milestones; sales bookings; business divestitures and acquisitions; capital raising; cash flow; cash position; contract awards or backlog; corporate transactions; customer renewals; customer retention rates from an acquired company, subsidiary, business unit or division; earnings (which may include any calculation of earnings, including but not limited to earnings before interest and taxes, earnings before taxes, earnings before interest, taxes, depreciation and amortization and net taxes); earnings per share; expenses; financial milestones; gross margin; growth in stockholder value relative to the moving average of the S&P 500 Index or another index; internal rate of return; leadership development or succession planning; license or research collaboration arrangements; market share; net income; net profit; net sales; new product or business development; new product invention or innovation; number of customers; operating cash flow; operating expenses; operating income; operating margin; overhead or other expense reduction; patents; procurement; product defect measures; product release timelines; productivity; profit; regulatory milestones or regulatory-related goals; retained earnings; return on assets; return on capital; return on equity; return on investment; return on sales; revenue; revenue growth; sales results; sales growth; savings; stock price; time to market; total stockholder return; working capital; unadjusted or adjusted actual contract value; unadjusted or adjusted total contract value; and individual objectives such as peer reviews or other subjective or objective criteria. The performance goals may differ from participant to participant and from award to award. The administrator also may determine that a target award or portion of a target award will not have a performance goal associated with it but instead will be granted, if at all, as determined by the administrator.

The administrator of our Executive Incentive Compensation Plan, in its sole discretion and at any time, may increase, reduce or eliminate a participant's actual award, and/or increase, reduce or eliminate the amount allocated to any bonus pool for a particular performance period. The actual award may be below, at or above a participant's target award, in the discretion of the administrator. The administrator may determine the amount of any reduction on the basis of such factors as it deems relevant, and the administrator is not required to establish any allocation or weighting with respect to the factors it considers.

Actual awards generally will be paid in cash (or its equivalent) only after they are earned, and, unless otherwise determined by the administrator, a participant must be employed with us through the date the actual award is paid. The administrator of our Executive Incentive Compensation Plan reserves the right to settle an actual award with a grant of an equity award under our then-current equity compensation plan, which equity award may have such terms and conditions, including vesting, as determined by the administrator. Payment of awards occurs as soon as administratively practicable after they are earned, but no later than the dates set forth in our Executive Incentive Compensation Plan.

Awards under our Executive Incentive Compensation Plan are subject to our amended and restated clawback policy, which we may revise from time to time to comply with applicable laws. The administrator also may impose such other clawback, recovery or recoupment provisions with respect an award under our Executive Incentive Compensation Plan as the administrator determines necessary or appropriate, including for example, reduction, cancellation, forfeiture or recoupment upon a termination of a participant's employment for cause. Certain participants may be required to reimburse us for certain amounts paid under an award under our Executive Incentive Compensation Plan in connection with certain accounting restatements we may be required to prepare due to our material noncompliance with any financial reporting requirements under applicable securities laws, as a result of misconduct.

The administrator of our Executive Incentive Compensation Plan has the authority to amend, alter, suspend or terminate our Executive Incentive Compensation Plan, provided such action does not impair the existing rights of any participant with respect to any earned awards. Our Executive Incentive Compensation Plan will remain in effect until terminated in accordance with its terms.

Retention Bonus Program

In January 2025, the compensation committee, and with respect to Dr. Litton, the board of directors, approved a cash retention program under the Company's Executive Incentive Compensation Plan, under which covered employees are entitled to receive a cash bonus (the "Retention Bonus Program"), upon the satisfaction of retention criteria set forth in the Retention Bonus Program. In February 2025, the compensation committee approved an amendment to the Retention Bonus Program, pursuant to which each applicable employee would be entitled to receive a cash bonus equal to the greater of (i) 50% of such employee's 2025 annual target bonus and (ii) 10% of such employee's 2025 annual base salary, and with such bonus due to be paid, less applicable tax withholdings, on the earliest of (x) September 30, 2025, (y) the consummation of a contemplated strategic transaction and (z) the date the Company winds up its affairs, subject, in each case, to each applicable employee remaining employed through such payment date. The retention bonuses awarded to our named executive officers for 2025 are set forth in the "*2024-2025 Summary Compensation Table*" above.

In September 2025, the compensation committee, and with respect to Dr. Litton, the board of directors, amended the Retention Bonus Program to provide additional retention into 2026. The amendment provides that certain employees will be entitled to receive an additional cash bonus equal to the greater of (i) 50% of such employee's 2025 annual target bonus and (ii) 10% of such employee's 2025 annual base salary, and with such bonus due to be paid, less applicable tax withholdings, on the earliest of (x) January 31, 2026, and (y) the date the Company winds up its affairs, subject, in each case, to (a) each applicable employee remaining employed through such payment date and (b) by no later than December 31, 2025, the Company having cash and cash equivalents of at least $30 million available for Phase 3 clinical development of an in-licensed drug candidate, as determined by the compensation committee or the board of directors (the "Bonus Condition").

In December 2025, our compensation committee determined that the Bonus Condition had been achieved.

Equity Granting Practices

Our board of directors or compensation committee, as applicable, does not grant equity awards on a predetermined schedule. Our grant committee, which consists of certain members of management and which has been delegated prescribed authority to grant equity awards to certain non-executive officer service providers, approves equity awards in advance, and such awards generally become effective on the first trading day of the following month. Awards to our non-employee directors are granted automatically pursuant to our outside director compensation policy. We have not granted, nor do we intend to grant, stock options in anticipation of the release of material, nonpublic information, and we have not taken, nor do we intend to take, material nonpublic information into account when determining the terms of stock options. Similarly, we have not timed, nor do we intend to time, the release of material, nonpublic information for the purpose of affecting the value of executive compensation or for any other purpose.

Director Compensation Table

The following table provides information regarding compensation of our non-employee directors for service as directors for the year ended December 31, 2025.

	Fees Earned or Paid in Cash ($)	Option Awards ($) (1)	Total ($)
Kelly A. Romano (2)	85,500	5,157	90,657
Joseph Edelman (3)	44,000	5,157	49,157
John M. Fluke, Jr. (4)	47,500	5,157	52,657
James A. Johnson (5)	65,000	5,157	70,157
Barbara Kosacz (6)	54,000	5,157	59,157
Michael Panzara, M.D., M.P.H. (7)	54,000	5,157	59,157
Grant Pickering (8)	45,000	5,157	50,157

(1) In accordance with Securities and Exchange Commission rules, the amount in this column reflects the aggregate grant date fair value of stock options granted during 2025 computed in accordance with Accounting Standards Codification (ASC) 718, rather than the amount paid or realized by the director. For a discussion of valuation assumptions, see Note 9 to our financial statements included in this Annual Report.

(2) As of December 31, 2025, Ms. Romano held stock options to purchase 10,431 shares of our common stock.

(3) As of December 31, 2025, Mr. Edelman held stock options to purchase 11,818 shares of our common stock.

(4) As of December 31, 2025, Mr. Fluke held stock options to purchase 11,818 shares of our common stock.

(5) As of December 31, 2025, Mr. Johnson held stock options to purchase 11,818 shares of our common stock.

(6) As of December 31, 2025, Ms. Kosacz held stock options to purchase 10,431 shares of our common stock.

(7) As of December 31, 2025, Dr. Panzara held stock options to purchase 9,275 shares of our common stock.

(8) As of December 31, 2025, Mr. Pickering held stock options to purchase 9,390 shares of our common stock.

In September 2020, based on discussions with and assistance from a third-party compensation consultant then retained by our compensation committee to provide our board of directors and our compensation committee with an analysis of publicly available market data and assistance in determining compensation to be provided to our non-employee directors, our board of directors adopted, and our stockholders approved, an outside director compensation policy providing for certain compensation to our non-employee directors. The outside director compensation policy was subsequently amended and restated in January 2022, January 2023, September 2024, September 2025 and March 2026 in consultation with Pearl Meyer & Partners, LLC, a third-party compensation consultant retained by our compensation committee beginning in October 2021 to provide our board of directors and our compensation committee with an analysis of publicly available market data and assistance in determining any proposed changes in non-employee director compensation.

Cash Compensation

The amended and restated outside director compensation policy provides for the following cash compensation program for our non-employee directors:

- $40,000 per year for service as a non-employee director;
- $30,000 per year for service as chairperson of our board of directors;
- $15,000 per year for service as chairperson of our audit committee;
- $7,500 per year for service as a member of our audit committee;
- $10,000 per year for service as chairperson of our compensation committee (increased to $12,000 per year effective as of January 1, 2026);
- $5,000 per year for service as a member of our compensation committee (increased to $6,000 per year effective as of January 1, 2026);
- $8,000 per year for service as chairperson of our nominating and corporate governance committee;
- $4,000 per year for service as a member of our nominating and corporate governance committee;
- $10,000 per year for service as chairperson of our compliance committee; and
- $5,000 per year for service as a member of our compliance committee.

Each non-employee director who serves as a committee chairperson receives only the cash retainer fee as the chairperson of the committee but not the cash retainer fee as a member of that committee. These fees to our non-employee directors are paid quarterly in arrears on a prorated basis. Under our amended and restated outside director compensation policy, we also reimburse our non-employee directors for reasonable travel expenses to attend meetings of our board of directors and its committees. The above-listed fees for service as chairperson or members of committees are payable in addition to the non-employee director retainer specified above.

The Strategic Transactions Committee was formed in September 2024 and met 50 times through the entry into the Sermonix License and completion of the PIPE Financing. In recognition of their efforts and time, in January 2026, our board of directors approved payments to each of Kelly Romano, James Johnson, Barbara Kosacz and Michael Panzara of $1,000 per meeting attended by each committee member.

Equity Compensation

Initial Award

Pursuant to our amended and restated outside director compensation policy, each person who becomes a non-employee director will receive, on the first trading day on or after the date that the person first becomes a non-employee director, an initial award (the "Initial Award"), of stock options to purchase 4,180 (increased to 56,000 shares of our common stock for non-employee directors joining after the March 2026 amendment and restatement of the outside director compensation policy) shares of our common stock. The Initial Award vests in equal installments as to $1/36^{th}$ of the shares of our common stock subject to the Initial Award on a monthly basis following the Initial Award's grant date, on the same day of the month as the grant date, subject to continued services to us through the applicable vesting dates. If the person was a member of our board of directors and also an employee, then becoming a non-employee director due to termination of employment will not entitle the person to an Initial Award.

Annual Award

Pursuant to our amended and restated outside director compensation policy, each non-employee director automatically receives, on the first trading day immediately after the date of each annual meeting of our stockholders, an annual award, (the "Annual Award") of stock options to purchase 2,090 shares of our common stock (increased to 28,000 shares of our common stock for annual meetings of our stockholders held in 2026 or later) (such shares, the "Annual Award Shares"). If, as of the date of the applicable annual meeting, such director has not been in continuous service as a non-employee director since the date of the most recently preceding annual meeting, their first annual award will be prorated and equal to the product of the Annual Award Shares multiplied by the quotient of (1) the number of whole months of continuous service as a non-employee director completed as of the date of such annual meeting divided by (2) 12, rounded down to the nearest whole share (up to a maximum of 2,090 or 28,000 shares, as applicable). Each Annual Award vests on the earlier of the one-year anniversary of the grant date, or the day immediately before the day of the next annual meeting of our stockholders that occurs after the grant date of the Annual Award, subject to continued service to us through the applicable vesting date.

2026 Award

Pursuant to the amended and restated outside director compensation policy approved in March 2026, in addition to the Annual Award described above, each non-employee director then-serving shall receive an award of stock options to purchase 28,000 shares of our common stock (the “2026 Award”). The 2026 Award will vest in equal installments as to 1/24th of the shares of our common stock subject to the 2026 Award on a monthly basis following the 2026 Award’s grant date, on the same day of the month as the grant date, subject to continued services to us through the applicable vesting dates.

Change in Control

In the event of a “Change in Control” (as defined in our 2026 Equity Incentive Plan), each non-employee director’s then outstanding equity awards covering shares of our common stock will accelerate vesting in full, provided that he or she remains a non-employee director through the date of the Change in Control.

Other Award Terms

Each Initial Award, Annual Award and 2026 Award will be granted under our 2026 Equity Incentive Plan (or its successor plan, as applicable) and form of award agreement under such plan. These awards will have a maximum term to expiration of 10 years from their grant and a per share exercise price equal to 100% of the fair market value of a share of our common stock on the award’s grant date.

Director Compensation Limits

Our amended and restated outside director compensation policy provides that in any fiscal year, a non-employee director may be paid cash compensation and other compensation and granted equity awards with an aggregate value of no more than $500,000 (with the value of equity awards based on their grant date fair value determined in accordance with U.S. Generally Accepted Accounting Principles for purposes of this limit), with such limit increased to $750,000 for the fiscal year of his or her initial service as a non-employee director. Equity awards granted or other compensation provided to a non-employee director while he or she was an employee or consultant of the Company (other than a non-employee director), or granted or provided prior to the effective date of the registration statement relating to our initial public offering, do not count toward this annual limit.

Role Of Board in Risk Oversight Process

Management is responsible for the day-to-day management of risks that the Company faces, in conjunction with our chief compliance officer (who is currently our general counsel) who oversees

compliance with certain corporate policies, and our board of directors has an active role, as a whole and also at the committee level, in overseeing the management of our risks. The board of directors is responsible for general oversight of risks and regular review of information regarding our risks, including credit risks, liquidity risks, environmental, social and governance related risks, cybersecurity risks, and operational risks. Our compensation committee is responsible for overseeing the management of risks relating to our executive compensation plans and arrangements. Our audit committee is responsible for overseeing the management of our risks relating to accounting matters and financial reporting. Our nominating and corporate governance committee is responsible for overseeing the management of our risks associated with the independence of our board of directors. Our compliance committee is responsible for assisting our board of directors in the oversight of our healthcare legal and regulatory compliance and scientific research integrity. While each committee is responsible for evaluating certain risks and overseeing the management of such risks, our entire board of directors is regularly informed through discussions with committee members about such risks. Our board of directors believes its administration of its risk oversight function has not affected the board of directors' leadership structure.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

The following table sets forth the beneficial ownership of our common stock as of March 16, 2026 by:

- each person, or group of affiliated persons, who is known by us to beneficially own more than 5% of our common stock;
- each of our named executive officers;
- each of our directors; and
- all of our executive officers and directors as a group.

The percentage of beneficial ownership shown in the table is based upon 9,393,514 shares of common stock outstanding as of the March 16, 2026.

Information with respect to beneficial ownership has been furnished by each director, officer or beneficial owner of more than 5% of our common stock. We have determined beneficial ownership in accordance with the rules of the Securities and Exchange Commission. These rules generally attribute beneficial ownership of securities to persons who possess sole or shared voting power or investment power with respect to those securities. In addition, the rules take into account shares of common stock issuable pursuant to the exercise or conversion of stock options or warrants or convertible notes that are either immediately exercisable or convertible or exercisable or convertible on or before the 60th day after March 16, 2026. Certain of the options granted to our executive officers may be exercised prior to the vesting of the underlying shares. These shares are deemed to be outstanding and beneficially owned by the person holding those options for the purpose of computing the percentage ownership of that person, but they are not treated as outstanding for the purpose of computing the percentage ownership of any other person. Unless otherwise indicated, the persons or entities identified in this table have sole voting and investment power with respect to all shares shown as beneficially owned by them, subject to applicable community property laws.

Except as otherwise noted below, the address for each person or entity listed in the table is c/o LeonaBio, Inc., 18706 North Creek Parkway, Suite 104, Bothell, Washington 98011.

	Shares Beneficially Owned	
	Number of Shares (#)	Percentage (%)
5% and greater stockholders:		
Anders Hove (1)	596,068	6.35%
Perceptive Entities (2)	1,882,370	19.99%
Named executive officers and directors:		
Mark Litton (3)	244,445	2.55%
Mark Worthington (4)	89,259	*
Javier San Martin (5)	53,976	*
Joseph Edelman (6)	1,882,370	19.99%
John M. Fluke, Jr. (7)	24,589	*
James A. Johnson (8)	10,228	*
Barbara Kosacz (9)	8,341	*
Kelly A. Romano (10)	16,412	*
Grant Pickering (11)	9,878	*
Michael Panzara (12)	7,185	*
All current directors and executive officers as a group (12 persons)	2,473,404	25.12

* Represents beneficial ownership of less than 1% of our outstanding common stock.

(1) Based on the Schedule 13G/A filed with the Securities and Exchange Commission on January 8, 2026. Consists of (i) 250,348 shares of common stock which Acorn Bioventures, L.P. and Acorn Capital Advisors GP, LLC may be deemed to beneficially own and (ii) 345,720 shares of common stock which Acorn Bioventures 2, L.P. and Acorn Capital Advisors GP 2, LLC may be deemed to beneficially own. Acorn Capital Advisors GP, LLC is the General Partner of Acorn Bioventures, L.P. and may be deemed to beneficially own the shares of common stock beneficially owned by Acorn Bioventures, L.P. Acorn Capital Advisors GP 2, LLC is the General Partner of Acorn Bioventures 2, L.P. and may be deemed to beneficially own the shares of common stock beneficially owned by Acorn Bioventures 2, L.P. Mr. Hove, in his capacity as Manager of each of Acorn Capital Advisors GP, LLC and Acorn Capital Advisors GP 2, LLC, may be deemed to beneficially own the shares beneficially owned by each of Acorn Capital Advisors GP, LLC and Acorn Capital Advisors GP 2, LLC. The business address of Acorn Bioventures, L.P. and Acorn Bioventures 2, L.P. is 420 Lexington Avenue, Suite 2626, New York, New York 10170.

(2) Based on the Schedule 13D/A filed with the Securities and Exchange Commission on January 16, 2026. Consists of (i) 1,529,566 shares of common stock and 18,960 shares of common stock issuable upon the partial exercise of a pre-funded warrant that is exercisable within 60 days of March 16, 2026 held directly by Perceptive Life Sciences Master Fund, Ltd. (“Master Fund”), and (ii) 329,756 shares of common stock and 4,088 shares of common stock issuable upon the partial exercise of a pre-funded warrant that is exercisable within 60 days of March 16, 2026 held directly by Perceptive Xontogeny Venture Fund II, LP (“PXV II” and, together with Master Fund, “Perceptive Entities”). Perceptive Advisors LLC (“Perceptive Advisors”) is the investment advisor of Perceptive Life Sciences Master Fund Ltd (“Perceptive Master Fund”) and may be deemed to have beneficial ownership of the shares beneficially owned thereby. Perceptive Venture Advisors, LP (“Perceptive Venture”) is the investment manager to Perceptive Xontogeny Venture Fund II, LP (“PXV II”), and accordingly, may be deemed to beneficially own the securities directly held by PXV II. Joseph Edelman is the controlling person of Perceptive Advisors and Perceptive Venture and accordingly, may be deemed to have beneficial ownership of the shares beneficially owned by the Perceptive Master Fund, PXV II, Perceptive Advisors and Perceptive Venture. Perceptive Advisors, Perceptive Venture, the Perceptive Master Fund, PXV II and Mr. Edelman disclaim beneficial ownership of all such securities except to the extent of its or his pecuniary interest therein. The business address for each of Perceptive Advisors, Perceptive Venture, the Perceptive Master Fund, PXV II and Mr. Edelman is 51 Astor Place, 10th Floor, New York, NY 10003.

(3) Consists of 57,926 shares held of record by Dr. Litton, 656 shares held by Irrevocable Trust of OSL, 656 shares held by Irrevocable Trust of SWL, and 656 shares held by Irrevocable Trust of WGL, each irrevocable trust is for the benefit of Dr. Litton's children, and options to purchase 184,551 shares that are exercisable within 60 days of March 16, 2026.

(4) Consists of 18,376 shares held by Mr. Worthington and options to purchase 70,883 shares that are exercisable within 60 days of March 16, 2026.

(5) Consists of 15,887 shares held by Dr. San Martin and options to purchase 38,089 shares that are exercisable within 60 days of March 16, 2026.

(6) Consists of the shares referenced in footnote (2) above and excludes 9,728 shares issuable upon the exercise of options held by Mr. Edelman, which are not exercisable within 60 days of March 16, 2026. Joseph Edelman is the managing member of Perceptive Advisors LLC and he may be deemed to beneficially own the shares held by Perceptive. Mr. Edelman disclaims beneficial ownership of all such securities except to the extent of its or his pecuniary interest therein.

(7) Consists of 373 shares held of record by Fluke Capital Management, L.P., 14,488 shares held by Mr. Fluke, and options to purchase 9,728 shares that are exercisable within 60 days of March 16, 2026

(8) Consists of 500 shares held by Mr. Johnson and options to purchase 9,728 shares that are exercisable within 60 days of March 16, 2026.

(9) Consists of options held by Ms. Kosacz to purchase 8,341 shares that are exercisable within 60 days of March 16, 2026.

(10) Consists of 8,071 shares held by Ms. Romano and options to purchase 8,341 shares that are exercisable within 60 days of March 16, 2026.

(11) Consists of 2,578 shares held by Mr. Pickering and options to purchase 7,300 shares that are exercisable within 60 days of March 16, 2026.

(12) Consists of options held by Dr. Panzara to purchase 7,185 shares that are exercisable within 60 days of March 16, 2026.

(13) Consists of 2,020,518 shares held by our current directors and executive officers as a group, pre-funded warrants to purchase 23,048 shares that are exercisable within 60 days of March 16, 2026, and options to purchase 429,838 shares that are exercisable within 60 days of March 16, 2026.

Securities Authorized for Issuance Under Equity Compensation Plans

The following table summarizes information about our equity compensation plans as of December 31, 2025. All outstanding awards relate to our common stock.

Plan Category	(a) Number of Securities to be Issued Upon Exercise of Outstanding Options, Warrants and Rights (#)	(b) Weighted Average Exercise Price of Outstanding Options, Warrants and Rights ($)	(c) Number of Securities Remaining Available for Future Issuance Under Equity Compensation Plans (Excluding Securities Reflected in Column (a)) (#) (3)
Equity compensation plans approved by security holders:			
2014 Equity Incentive Plan	13,240	13.06	—
2020 Equity Incentive Plan	1,209,190 (1)	38.31 (2)	63,723
2020 Employee Stock Purchase Plan	—	—	156,754
Equity compensation plans not approved by security holders:			
2024 Inducement Equity Incentive Plan	40,000	20.60	35,000
Total	1,262,430		255,477

(1) Includes both 1,151,589 outstanding options and 57,601 outstanding, unvested time-based and performance restricted stock units.

(2) Represents the outstanding options' weighted-average exercise price and does not take into account the shares issuable upon vesting of outstanding time-based and performance restricted stock units, which do not have an exercise price.

(3) Our 2020 Equity Incentive Plan includes provisions providing for an annual increase in the number of securities available for future issuance on the first day of each fiscal year, equal to the least of: (1) 323,000 shares; (2) 5% of the outstanding shares of common stock as of the last day of the immediately preceding fiscal year; and (3) such lesser number of shares determined by the board of directors. Our 2020 Employee Stock Purchase Plan includes provisions providing for an annual increase in the number of securities available for future issuance on the first day of each fiscal year, equal to the least of: (1) 1% of the outstanding shares of common stock as of the last day of the immediately preceding fiscal year; (2) 64,600 shares; and (3) such lesser number of shares determined by the board of directors.

In February 2024, our board of directors adopted the Inducement Plan, and, subject to the adjustment provisions of the Inducement Plan, reserved 75,000 shares of our common stock for issuance pursuant to equity awards granted under the Inducement Plan. The Inducement Plan was adopted without stockholder approval pursuant to Nasdaq Rule 5635(c)(4). The Inducement Plan provides for the grant of equity-based awards, including nonstatutory stock options, stock appreciation rights, restricted stock, restricted stock units, and performance awards, and its terms are substantially similar to our 2020 Equity Incentive Plan, including with respect to treatment of equity awards in the event of a "Change in Control" (as defined under the Inducement Plan) of the Company or a merger of the Company with or into another corporation or entity, but with such other terms and conditions intended to comply with the Nasdaq inducement award exception. However, the 2020 Equity Incentive Plan permits certain exchange programs (including repricings) without stockholder approval, while our Inducement Plan does not permit such exchange programs.

Item 13.Certain Relationships and Related Transactions and Director Independence

The following is a summary of transactions since January 1, 2024 to which we have been a party in which the amount involved exceeded $120,000 and in which any of our executive officers, directors, promoters or beneficial holders of more than 5% of our capital stock had or will have a direct or indirect material interest, other than compensation arrangements which are described under the section of this Annual Report titled "Executive Compensation."

Related-Person Transactions Policy

We have a formal, written policy that our executive officers, directors (including director nominees), holders of more than 5% of any class of our voting securities and any member of the immediate family of or any entities affiliated with any of the foregoing persons, are not permitted to enter into a related-person transaction with us without the prior approval or, in the case of pending or ongoing related-person transactions, ratification of our audit committee. For purposes of our policy, a related-person transaction is a transaction, arrangement or relationship where we were, are or will be involved and in which a related-person had, has or will have a direct or indirect material interest.

Certain transactions with related persons, however, are exempted from pre-approval including, but not limited to:

- compensation of our executive officers and directors that is otherwise disclosed in our public filings with the SEC;
- compensation, benefits and other transactions available to all of our employees generally;
- transactions where a related-person's interest derives solely from his or her service as a director of another entity that is a party to the transaction;
- transactions where a related-person's interest derives solely from his or her ownership of less than 10% of the equity interest in another entity that is a party to the transaction; and
- transactions where a related-person's interest derives solely from his or her ownership of a class of our equity securities and all holders of that class received the same benefit on a pro rata basis.
- No member of the audit committee may participate in any review, consideration or approval of any related-person transaction where such member or any of his or her immediate family members is the related-person. In approving or rejecting the proposed agreement or transaction, our audit committee shall consider the relevant facts and circumstances available and deemed relevant to the audit committee, including, but not limited to:
- the benefits and perceived benefits to us;
- the materiality and character of the related-person's direct and indirect interest;
- the availability of other sources for comparable products or services;
- the terms of the transaction; and
- the terms available to unrelated third parties under the same or similar circumstances.

Private Placement Financing

On December 18, 2025, we completed a private placement of an aggregate of 5,356,547 shares of common stock and, in lieu of common stock, pre-funded warrants to purchase 8,816,684 shares of common stock, and accompanying Series A Warrants to purchase 23,031,494 shares of common stock and Series B warrants to purchase 21,259,842 shares of common stock with total gross proceeds of approximately $90 million (the “PIPE Transaction”). Affiliates of Perceptive Advisors LLC ("Perceptive Advisors") participated in the transaction. Perceptive Life Sciences Master Fund Ltd. purchased an aggregate of 989,270 shares of our common stock, a pre-funded warrant to purchase 1,372,935 shares of our common stock, and an accompanying Series A Common Warrant to purchase 3,838,583 shares of our common stock and an accompanying Series B Common Warrant to purchase 3,543,307 shares of our common stock. Perceptive Xontogeny Venture Fund II, LP purchased 329,756 shares of common stock, a pre-funded warrant to purchase 457,645 shares of our common stock and an accompanying Series A Common Warrant to purchase 1,279,526 shares of our common stock and an accompanying Series B Common Warrant to purchase 1,181,101 shares of our common stock. The per share purchase price was $6.35 for the common stock and accompanying Series A Common Warrants and Series B Common Warrants, $6.349 for the pre-funded warrants and accompanying Series A Common Warrants and Series B Common Warrants, for an aggregate purchase price for Perceptive of $20.0 million. In connection with the private placement, we entered into a registration rights agreement with the purchasers in the PIPE Transaction, including Perceptive, requiring us to register the resale of the shares of our common stock and shares of common issuable upon exercise of the pre-funded warrants, Series A Common Warrants and Series B Common Warrants. Joseph Edelman, a member of our board of directors, is a managing member of Perceptive Advisors, the investment manager of Perceptive. Prior to the PIPE Transaction, Perceptive Life Sciences Master Fund Ltd. was a greater than 5% holder of our common stock. Other than Perceptive, none of the investors in the PIPE Transaction were greater than 5% holders of our common stock prior to the PIPE Transaction. Certain of the investors in the PIPE Transaction may, from time to time, be holders of 5% or more of our common stock either through the exercise of warrants and pre-funded warrants issued to such investors in the PIPE Transaction or otherwise, and such investors are parties to the registration rights agreement described above.

Additionally, pursuant to the terms of the PIPE Securities Purchase Agreement, for so long as funds affiliated with each of Commodore and TCGX, respectively, beneficially own 5% or more in the aggregate of the issued and outstanding shares of our common stock (including any shares of common stock issuable upon the exercise of any outstanding PIPE Warrants whose initial exercise date has occurred), each of Commodore and TCGX will be entitled to designate one member of the board of directors, and we will take all actions reasonably necessary to have such designee promptly appointed to our board of directors, including, but not limited to, increasing the size of our board of directors to accommodate the appointment of such designee, subject to specified conditions. In addition, we have agreed to use our reasonable best efforts to cause the resignation of two (2) current members of our board of directors by the six (6) month anniversary of the PIPE Closing Date.

Sermonix Transaction

On December 18, 2025, we entered into the Sermonix License with Sermonix granting us an exclusive license and rights to develop, manufacture and commercialize oral forms of the selective estrogen-receptor modulator known as lasofoxifene in all countries and territories of the world except for Asia and certain countries in the Middle East. Under the terms of the Sermonix License, we assuming responsibility, in such countries and territories, for the ELAINE-3 trial and will coordinate with Sermonix and Henlius, Sermonix's exclusive licensee for the Retained Territory, with respect to Henlius’ conduct of the ELAINE-3 trial in the Retained Territory. As consideration for the rights granted to us under the Sermonix license, we issued to Sermonix the Sermonix Pre-Funded Warrant, agreed to make payments to certain of Sermonix’s third-party service providers that total approximately $16.8 million, and make payments to Sermonix of $75,000 per month, subject to adjustment from time to time. Sermonix is also eligible to receive certain milestone payments and royalties pursuant to the terms of the Sermonix License.

In connection with entry into the Sermonix License, on December 18, 2025, we also entered into the Sermonix Securities Purchase Agreement, pursuant to which we issued to the Sermonix Pre-Funded Warrant. The Sermonix Pre-Funded Warrant has an exercise price of $0.001 per share, and was issued as partial consideration for Sermonix's entry into the Sermonix License. We also entered into a registration rights agreement with Sermonix, pursuant to which we were required to file a registration statement to register the resale of shares issuable pursuant to the Sermonix Pre-Funded Warrant. In addition, we agreed to, no later than in connection with our 2026 annual meeting of stockholders (the "Annual Meeting"), provide notice of, and solicit proxies from our stockholders to obtain the Sermonix Stockholder Approval. If the Sermonix Stockholder Approval was not obtained at or before the Annual Meeting, we agreed to cause an additional meeting (special or general) of the Company's stockholders to be held every 90 days thereafter for the purpose of obtaining the Sermonix Stockholder Approval until the Sermonix Stockholder Approval was obtained. If the Sermonix Stockholder Approval was not obtained by the first anniversary of the original issuance of the Sermonix Pre-Funded Warrant, Sermonix would have had the right (the "Sermonix Redemption Right") at any time and from time to time prior to such time that the Sermonix Stockholder Approval is obtained thereafter, to cause the Company to pay, at the option of Sermonix, an amount up to (a) $6.35 (the "Sermonix Redemption Price") multiplied by (b) the number of shares of common stock with respect to which Sermonix is exercising the Sermonix Redemption Right. The Sermonix Redemption Right would have terminated on the earlier of (1) such time as we have paid an aggregate of $7.5 million in aggregate Sermonix Redemption Price to Sermonix in connection with one or more exercises of the Sermonix Redemption Right; and (2) immediately upon receipt of the Sermonix Stockholder Approval. At our March 2026 special meeting of stockholders, the Sermonix Stockholder Approval was obtained and the Sermonix Redemption Right terminated.

An affiliate of Perceptive Advisors held approximately 29% of the outstanding capital stock of Sermonix (excluding securities convertible into shares of capital stock) at the time of entry into the Sermonix License and Sermonix Securities Purchase Agreement. As of the date of this Annual Report on Form 10-K, an affiliate of Perceptive Advisors holds approximately 49.8% of the outstanding capital stock of Sermonix.

For additional information please see the Sermonix Transaction section of Item 1. Business.

Other Transactions

We have entered into employment offer letters and change in control and severance agreements with our executive officers. For a description of these agreements with our named executive officers, see the section of this Annual Report titled "Executive Compensation – Executive Employment Arrangements."

We have granted stock options and/or restricted stock units, and issued common stock in connection therewith as applicable, to our executive officers and our non-employee directors. For a description of these grants and issuances, see the sections of this Annual Report titled "Director Compensation" and "Executive Compensation."

We have entered into indemnification agreements with our directors and executive officers which provide for the indemnification for certain expenses and liabilities incurred in connection with any action, suit, proceeding or alternative dispute resolution mechanism, or hearing, inquiry or investigation that may lead to the foregoing, to which they are a party, or are threatened to be made a party, by reason of the fact that they are or were a director, officer, employee, agent or fiduciary of us, or any of our subsidiaries, by reason of any action or inaction by them while serving as a director, officer, employee, agent or fiduciary, or by reason of the fact that they were serving at our request as a director, officer, employee, agent or fiduciary of another entity. In the case of an action or proceeding by or in the right of us or any of our subsidiaries, no indemnification will be provided for any claim where a court determines that the indemnified party is prohibited from receiving indemnification. We believe that these indemnification agreements are necessary to attract and retain qualified persons as directors and officers. We also maintain directors' and officers' liability insurance.

Director Independence

Our common stock is listed on the Nasdaq Capital Market. Under the rules of Nasdaq, independent directors must comprise a majority of a listed company's board of directors. In addition, the rules of Nasdaq require that, subject to specified exceptions, each member of a listed company's audit, compensation, and nominating and corporate governance committees be independent. Audit committee members must also satisfy the independence criteria set forth in Rule 10A-3 under the Exchange Act. Under the rules of Nasdaq, a director will only qualify as an "independent director" if, in the opinion of that company's board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director.

To be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors or any other board committee: (1) accept, directly or indirectly, any consulting, advisory or other compensatory fee from the listed company or any of its subsidiaries; or (2) be an affiliated person of the listed company or any of its subsidiaries.

Our board of directors has undertaken a review of its composition, the composition of its committees and the independence of our directors and considered whether any director has a material relationship with us that could compromise his or her ability to exercise independent judgment in carrying out his or her responsibilities. Based upon information requested from and provided by each director concerning his or her background, employment and affiliations, including family relationships, our board of directors has determined that, other than Dr. Litton, none of our directors has a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director and that each of these directors is "independent" as that term is defined under the rules of Nasdaq. Our board of directors also determined that Mr. Johnson, Mr. Fluke and Ms. Romano, who comprise our audit committee; Ms. Kosacz, Mr. Johnson and Mr. Pickering, who comprise our compensation committee; Ms. Romano, Ms. Kosacz, Mr. Edelman and Dr. Panzara, who comprise our nominating and corporate governance committee; and Dr. Panzara and Mr. Johnson, who comprise our compliance committee, satisfy applicable independence standards established by applicable SEC rules and the rules of Nasdaq. In making this determination, our board of directors considered the relationships that each non-employee director has with us and all other facts and circumstances our board of directors deemed relevant in determining their independence, including the beneficial ownership of our capital stock by each non-employee director.

Item 14. Principal Accounting Fees and Services

Principal Independent Accountant Fees and Services

The following table presents fees for professional audit services and other services rendered to us by Ernst & Young LLP for our fiscal years ended December 31, 2025 and 2024 (in thousands)

	2025 ($)	2024 ($)
Audit Fees[1]	$ 834	$ 832
Audit Related Fees[2]	—	—
Tax Fees[3]	—	—
All Other Fees[4]	—	—
Total Fees Paid	$ 834	$ 832

(1) Audit fees include fees incurred associated with the annual audit, the reviews of the Company's interim financial information, consents to documents filed with the SEC, and services provided in connection with the preparation and filing of our registration statements.

(2) Audit-related fees include fees for assurance and related services that are reasonably related to the performance of the audit or review of our financial statements. There were no such fees incurred in 2025 or 2024.

(3) Tax fees consist of fees for professional services, including tax compliance services and tax advisory services. There were no such fees incurred in 2025 or 2024.

(4) All other fees include any fees billed that are not audit fees, audit-related fees or tax fees. There were no such fees incurred in 2025 or 2024.

Pre-approval Policies and Procedures

Our audit committee's policy is to pre-approve all audit and permissible non-audit services provided by our independent registered public accounting firm, the scope of services provided by our independent registered public accounting firm and the fees for the services to be performed. These services may include audit services, audit-related services, tax services and other services. Pre-approval is detailed as to the particular service or category of services and is generally subject to a specific budget. Our independent registered public accounting firm and management are required to periodically report to the audit committee regarding the extent of services provided by our independent registered public accounting firm in accordance with this pre- approval, and the fees for the services performed to date. The audit committee may delegate to the chairperson of the audit committee authority to approve in advance permitted services to be performed by the independent auditor or other registered public accounting firms along with any associated fees.

All services related to the fees described in the table above were pre-approved by our audit committee.

PART IV

Item 15. Exhibits, Financial Statement Schedules.

(a) List the following documents filed as a part of the report:

(1) All financial statements;

See Index to Financial Statements in Part II, Item 8 of this Annual Report on Form 10-K.

(2) Financial Statement Schedules

All financial statement schedules have been omitted because the required information was not applicable or was not present in amounts sufficient to require submission of the schedules, or because the information required is included in the financial statements or the accompanying notes.

(3) Exhibits

The exhibits listed in the following Index to Exhibits are filed, furnished or incorporated by reference as part of this Annual Report on Form 10-K.

Index to Exhibits

Exhibit Number	Description	Incorporated by Reference			
		Form	File No.	Exhibit	Filing Date
3.1	Amended and Restated Certificate of Incorporation of the Company	10-Q	001-39503	3.1	November 12, 2020
3.2	Certificate of Amendment to the Amended and Restated Certificate of Incorporation of the Company dated May 23, 2024	8-K	001-39503	3.1	May 29, 2024
3.3	Certificate of Amendment to the Amended and Restated Certificate of Incorporation of the Company dated September 10, 2025	8-K	001-39503	3.1	September 11, 2025
3.4	Certificate of Amendment to the Amended and Restated Certificate of Incorporation of the Company dated January 6, 2026	8-K	001-39503	3.1	January 9, 2026
3.5	Certificate of Amendment to the Amended and Restated Certificate of Incorporation of the Company dated March 18, 2026	8-K	001-39503	3.1	March 19, 2026
3.6	Amended and Restated Bylaws	8-K	001-39503	3.2	January 9, 2026
4.1	Specimen Common Stock Certificate of the Registrant	S-3	333-292826	4.1	January 20, 2026
4.2	Description of Capital Stock				
4.3	Form of PIPE Pre-Funded Warrant	8-K/A	001-39503	4.1	December 18, 2025

4.4	Form of PIPE Series A Common Warrant	8-K/A	001-39503	4.2	December 18, 2025
4.5	Form of PIPE Series B Common Warrant	8-K/A	001-39503	4.3	December 18, 2025
4.6	Form of Sermonix Pre-Funded Warrant	8-K/A	001-39503	4.4	December 18, 2025
4.7	Form of PIPE Registration Rights Agreement	8-K/A	001-39503	4.5	December 18, 2025
4.8	Form of Sermonix Registration Rights Agreement	8-K/A	001-39503	4.6	December 18, 2025
10.1**	Form of Director and Executive Officer Indemnification Agreement				
10.2**	2014 Equity Incentive Plan, as amended				
10.3**	2020 Equity Incentive Plan, as amended				
10.4**	Form of Stock Option Agreement under the 2020 Equity Incentive Plan, as amended				
10.5**	Form of Restricted Stock Award Agreement under the 2020 Equity Incentive Plan, as amended				
10.6**	Form of RSU Agreement under the 2020 Equity Incentive Plan, as amended				
10.7**	2020 Employee Stock Purchase Plan, as amended and Form of Subscription Agreement Thereunder				
10.8**	2026 Equity Incentive Plan	8-K	001-39503	10.1	March 19, 2026
10.9**	Form of Stock Option Agreement under the 2026 Equity Incentive Plan	8-K	001-39503	10.2	March 19, 2026
10.10**	Form of Restricted Stock Award Agreement under the 2026 Equity Incentive Plan	8-K	001-39503	10.3	March 19, 2026
10.11**	Form of RSU Agreement under the 2026 Equity Incentive Plan	8-K	001-39503	10.4	March 19, 2026
10.12	Lease agreement, dated July 20, 2020, by and between the Registrant and North Creek Parkway Center Investors, LP	S-1/A	333-248428	10.11	September 9, 2020

10.13**	Outside Director Compensation Policy, as amended				
10.14**	Executive Incentive Compensation Plan				
10.15**	Confirmatory Employment Letter between the Registrant and Mark Litton, Ph.D.	S-1/A	333-248428	10.15	September 9, 2020
10.16**	Confirmatory Employment Letter between the Registrant and Kevin Church, Ph.D.	S-1/A	333-248428	10.16	September 9, 2020
10.17**	Amended and Restated Change in Control and Severance Agreement between the Registrant and Mark Litton, Ph.D.	8-K	001-39503	10.1	January 31, 2022
10.18**	Employment Offer Letter between the Registrant and Mark Worthington	10-Q	001-39503	10.3	August 16, 2021
10.19**	Change in Control and Severance Agreement between the Registrant and Mark Worthington	10-Q	001-39503	10.4	August 16, 2021
10.20	First Amendment to Lease by and between the Registrant and Nitrogen Propco 2020, L.P., as successor-in-interest to North Creek Parkway Center Investors, L.P., dated June 28, 2021	10-Q	001-39503	10.5	August 16, 2021
10.21**	Change in Control and Severance Agreement between the Registrant and Kevin Church	10-K	001-39503	10.28	March 28, 2022
10.22	Controlled Equity Offering Sales Agreement[SM], dated January 6, 2023, among the Registrant, Cantor Fitzgerald & Co. and BTIG, LLC	8-K	001-39503	1.1	January 6, 2023
10.23**	2024 Inducement Equity Incentive Plan and related form agreements				
10.24**	Employment Offer Letter between the Registrant and Javier San Martin	10-Q/A	001-39503	10.1	August 9, 2024
10.25**	Change in Control and Severance Agreement between the Registrant and Javier San Martin	10-Q/A	001-39503	10.2	August 9, 2024
10.26**	Employment Offer Letter between the Company and Robert Renninger	8-K	001-39503	10.1	September 17, 2024
10.27**	Change in Control and Severance Agreement between the Company and Robert Renninger, dated as of January 8, 2026	8-K	001-39503	10.1	January 9, 2026
10.28***	License Agreement between Sermonix Pharmaceuticals, Inc. and Athira Pharma, Inc. dated December 18, 2025	8-K/A	001-39503	2.1	March 31, 2026
10.29***	License Agreement between Ligand Pharmaceuticals Incorporated and Athira Pharma, Inc. dated December 18, 2025				

10.30	Securities Purchase Agreement dated December 18, 2025, by and among the Company and the PIPE Purchasers	8-K/A	001-39503	10.1	December 18, 2025
10.31	Securities Purchase Agreement dated December 18, 2025, by and between the Company and Sermonix Pharmaceuticals, Inc.	8-K/A	001-39503	10.2	December 18, 2025
19	Insider Trading Policy, as amended				
21.1	List of Subsidiaries of the Registrant	S-1/A	333-24828	21.1	September 9, 2020
23.1	Consent of Independent Registered Public Accounting Firm				
24.1	Power of Attorney (included in signature pages hereto)				
31.1	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002				
31.2	Certification of Principal Accounting and Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002				
32.1*	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002				
32.2*	Certification of Principal Accounting and Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002				
97	Amended and Restated Compensation Recovery Policy				
101.INS	The following materials from the Company's Annual Report on Form 10-K for the year ended December 31, 2025, formatted in Inline XBRL (Inline eXtensible Business Reporting Language): (i) Consolidated Balance Sheets as at December 31, 2025 and 2024, (ii) Consolidated Statements of (Loss) Income and Comprehensive (Loss) Income for the years ended December 31, 2025 and 2024, (iii) Consolidated Statements of Changes in Stockholders' Equity for the years ended December 31, 2025 and 2024, (iv) Consolidated Statements of Cash Flows for the years ended December 31, 2025 and 2024 and (vi) Notes to Consolidated Financial Statements.				

104	Cover Page Interactive Data File (formatted in Inline XBRL and included in Exhibit 101)

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* The certifications filed as Exhibits 32.1 and 32.2 are not deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934 and are not to be incorporated by reference into any filing of the Company under the Securities Act of 1933 or the Securities Exchange Act of 1934, whether made before or after the date hereof irrespective of any general incorporation by reference language contained in any such filing, except to the extent that the registrant specifically incorporates it by reference.

** Indicates a management contract or compensatory plan.

*** Certain confidential information contained in this exhibit, marked by [***], has been omitted because it is both (1) not material and (2) is the type that the Company treats as private and confidential.

Item 16. Form 10-K Summary

Not applicable.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, as amended, the Registrant has duly caused this Report to be signed on its behalf by the undersigned, thereunto duly authorized.

LeonaBio, Inc.

Date: March 31, 2026

By: /s/ Mark Litton
Mark Litton
President and Chief Executive Officer (*Principal Executive Officer*)

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Mark Litton and Robert Renninger, and each one of them, as his or her true and lawful attorneys-in-fact and agents, with full power of substitution and resubstitution, for him or her and in their name, place and stead, in any and all capacities, to sign any amendments to this Annual Report on Form 10-K and to file the same, with Exhibits thereto and other documents in connection therewith with the Securities and Exchange Commission, hereby ratifying and confirming all that each of said attorneys-in-fact, or substitute or substitutes may do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, this Annual Report on Form 10-K has been signed below by the following persons on behalf of the Registrant in the capacities and on the dates indicated.

Signature	Title	Date
/s/ Mark Litton Mark Litton	President, Chief Executive Officer and Director (*Principal Executive Officer*)	March 31, 2026
/s/ Robert Renninger Robert Renninger	Chief Financial Officer (*Principal Financial and Accounting Officer*)	March 31, 2026
/s/ Kelly A. Romano Kelly A. Romano	Chairwoman of the Board of Directors	March 31, 2026
/s/ Joseph Edelman Joseph Edelman	Director	March 31, 2026
/s/ John M. Fluke, Jr. John M. Fluke, Jr.	Director	March 31, 2026
/s/ James A. Johnson James A. Johnson	Director	March 31, 2026
/s/ Barbara Kosacz Barbara Kosacz	Director	March 31, 2026
/s/ Michael Panzara Michael Panzara	Director	March 31, 2026
/s/ Grant Pickering Grant Pickering	Director	March 31, 2026

NASDAQ: LONA

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