UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, DC 20549

FORM 10-Q

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

For the quarterly period ended September 30, 2026

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 offices)
(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)

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 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, a 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 is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of November 13, 2026, there were 13,076,348 shares of registrant's common stock, $0.0001 par value per share, outstanding.

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This report includes our trademarks and registered trademarks, including the LeonaBio logo, and other trademarks or service marks of LeonaBio. Each other trademark, trade name or service mark appearing in this report belongs to its holder.

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 brelgometon 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 brelgometon, 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 brelgometon 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.
- 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.
- 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 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—FINANCIAL INFORMATION

Item 1. Financial Statements (Unaudited)

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

	September 30, 2026	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	$ 16,336	$ 69,276
Short-term investments	15,974	19,055
Prepaid expenses and other current assets	4,023	1,127
Total current assets	36,333	89,458
Restricted cash	631	631
Property and equipment, net	800	1,472
Operating lease right-of-use asset	308	535
Other long-term assets	64	55
Total assets	$ 38,136	$ 92,151
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 788	$ 4,587
Accrued liabilities	6,080	4,963
Sermonix pre-funded warrant	—	37,488
Current operating lease liability	460	465
Total current liabilities	7,328	47,503
Operating lease liability, less current portion	—	338
Milestone liability	13,951	15,116
Other long-term liabilities	840	1,404
Total liabilities	$ 22,119	$ 64,361
Stockholders' equity:		
Common stock, $0.0001 par value; 400,000,000 and 90,000,000 shares authorized at September 30, 2026 and December 31, 2025, respectively; 9,926,878 and 9,335,913 shares issued and outstanding at September 30, 2026 and December 31, 2025, respectively	1	1
Additional paid-in capital	600,902	539,548
Accumulated other comprehensive (loss) income	(12)	(4)
Accumulated deficit	(584,874)	(511,755)
Total stockholders' equity	16,017	27,790
Total liabilities and stockholders' equity	$ 38,136	$ 92,151

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Operating expenses:				
Research and development	$ 15,015	$ 2,825	$ 38,979	$ 10,788
Milestone liability change in fair value	(411)	—	(1,165)	—
General and administrative	6,863	4,049	20,320	12,913
Total operating expenses	21,467	6,874	58,134	23,701
Loss from operations	(21,467)	(6,874)	(58,134)	(23,701)
Other income, net	303	263	1,335	981
Sermonix pre-funded warrant change in fair value	—	—	(16,320)	—
Net loss	$ (21,164)	$ (6,611)	$ (73,119)	$ (22,720)
Unrealized (loss) gain on available-for-sale securities	(3)	1	(8)	(3)
Comprehensive loss attributable to common stockholders	$ (21,167)	$ (6,610)	$ (73,127)	$ (22,723)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.89)	$ (1.68)	$ (3.30)	$ (5.80)
Weighted-average shares used in computing net loss per share attributable to common stockholders, basic and diluted	23,740,719	3,943,887	22,181,938	3,919,258

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

LeonaBio, Inc.
Condensed Consolidated Statements of Stockholders' Equity
For the Three and Nine Months Ended September 30, 2026 and 2025
(in thousands, except share amounts)
(unaudited)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance as of January 1, 2026	9,335,913	$ 1	$ 539,548	$ (4)	$ (511,755)	$ 27,790
Issuance of common stock upon vesting of restricted stock units	57,601	—	—	—	—	—
Reclassification of Sermonix Pre-funded warrant to equity	—	—	53,808	—	—	53,808
Stock-based compensation	—	—	662	—	—	662
Unrealized (loss) gain on available-for-sale securities	—	—	—	(9)	—	(9)
Net loss	—	—	—	—	(32,948)	(32,948)
Balance as of March 31, 2026	9,393,514	$ 1	$ 594,018	$ (13)	$ (544,703)	$ 49,303
Issuance of common stock under employee stock purchase plan	28,149	—	114	—	—	114
Stock-based compensation	—	—	3,181	—	—	3,181
Unrealized (loss) gain on available-for-sale securities	—	—	—	4	—	4
Net loss	—	—	—	—	(19,007)	(19,007)
Balance as of June 30, 2026	9,421,663	$ 1	$ 597,313	$ (9)	$ (563,710)	$ 33,595
Issuance of common stock upon net exercise of pre-funded warrants	505,215	—	—	—	—	—
Stock-based compensation	—	—	3,589	—	—	3,589
Unrealized (loss) gain on available-for-sale securities	—	—	—	(3)	—	(3)
Net loss	—	—	—	—	(21,164)	(21,164)
Balance as of September 30, 2026	9,926,878	$ 1	$ 600,902	$ (12)	$ (584,874)	$ 16,017

	Common Stock (1)		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance as of January 1, 2025	3,904,049	$ —	$ 450,986	$ 1	$ (406,146)	$ 44,841
Issuance of common stock upon vesting of restricted stock units	150	—	—	—	—	—
Stock-based compensation	—	—	1,637	—	—	1,637
Unrealized (loss) gain on available-for-sale securities	—	—	—	(5)	—	(5)
Net loss	—	—	—	—	(9,143)	(9,143)
Balance as of March 31, 2025	3,904,199	$ —	$ 452,623	$ (4)	$ (415,289)	$ 37,330
Issuance of common stock upon vesting of restricted stock units	29,688	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	10,000	—	24	—	—	24
Stock-based compensation	—	—	1,597	—	—	1,597
Unrealized (loss) gain on available-for-sale securities	—	—	—	1	—	1
Net loss	—	—	—	—	(6,966)	(6,966)
Balance as of June 30, 2025	3,943,887	$ —	$ 454,244	$ (3)	$ (422,255)	$ 31,986
Stock-based compensation	—	—	1,410	—	—	1,410
Unrealized (loss) gain on available-for-sale securities	—	—	—	1	—	1
Net loss	—	—	—	—	(6,611)	(6,611)
Balance as of September 30, 2025	3,943,887	$ —	$ 455,654	$ (2)	$ (428,866)	$ 26,786

The accompanying notes are an integral part of these unaudited condensed 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 Quarterly Report on Form 10-Q have been restated to reflect the reverse stock split on a retroactive basis in all periods presented.

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

	Nine Months Ended September 30,	
	2026	**2025**
Operating activities		
Net loss	$ (73,119)	$ (22,720)
Adjustments to reconcile net loss to net cash used in operating activities:		
Milestone liability change in fair value	(1,165)	—
Change in fair value of Sermonix pre-funded warrant	16,320	—
Stock-based compensation	7,432	4,644
Depreciation expense	678	729
Non-cash lease expense	227	201
Amortization of premiums and accretion of discounts on available-for-sale securities, net	(634)	(262)
Changes in operating assets and liabilities:		
Prepaid expenses and other current and long-term assets, net	(2,905)	1,767
Accounts payable and accrued liabilities	(2,682)	(10,386)
Operating lease liability	(343)	(305)
Other long-term liabilities	(564)	—
Net cash used in operating activities	(56,755)	(26,332)
Investing activities		
Purchases of available-for-sale securities	(38,811)	(19,605)
Maturities of available-for-sale securities	42,518	18,349
Purchases of property and equipment	(6)	—
Net cash (used in) provided by investing activities	3,701	(1,256)
Financing activities		
Proceeds from issuance of common stock under employee stock purchase plan	114	24
Net cash provided by financing activities	114	24
Net increase (decrease) in cash, cash equivalents and restricted cash	(52,940)	(27,564)
Cash, cash equivalents and restricted cash, beginning of period	69,907	49,069
Cash, cash equivalents and restricted cash, end of period	$ 16,967	$ 21,505

The accompanying notes are an integral part of these unaudited condensed 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, Capital Resources and Going Concern

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, pre-funded common stock warrants, and convertible notes, and to a lesser extent from grant income and stock option exercises. From the Company's inception through September 30, 2026, 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, pre-funded 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. Refer to Note 8 for more information.

As of September 30, 2026, the Company had cash, cash equivalents and investments of $32.3 million. The Company's net loss for the nine months ended September 30, 2026 was $73.1 million and cash used in operations for the nine months ended September 30, 2026 was $56.8 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 $32.3 million of cash, cash equivalents and investments at September 30, 2026 will not be sufficient to fund its operating expenses and capital expenditure requirements under its current operating plan for at least the next 12 months, which raises substantial doubt as to the Company's ability to continue as a going concern following the date of this Quarterly Report on Form 10-Q. 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.

Additionally, 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 unaudited condensed consolidated financial statements have been prepared in conformity with U.S. generally accepted accounting principles, or U.S. GAAP. The unaudited condensed 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, or 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 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 stock from 1,000,000,000 to 190,000,000. On March 18, 2026, the Company held a special meeting of stockholders in which the stockholders approved the amendment of the Company's amended and restated certificate of incorporation to increase the number of authorized shares to 500,000,000, of which 400,000,000 shares are Common Stock, $0.0001 par value per share, and 100,000,000 shares are Preferred Stock, $0.0001 par value per share. All share amounts and per share amounts disclosed in this Quarterly Report on Form 10-Q have been restated to reflect the reverse stock split on a retroactive basis in all periods presented.

Unaudited Interim Condensed Consolidated Financial Statements

The accompanying condensed consolidated balance sheet as of September 30, 2026, and condensed consolidated statements of operations and comprehensive loss, cash flows, and stockholders' equity for the three and nine months ended September 30, 2026 and 2025, are unaudited. The balance sheet as of December 31, 2025 was derived from the audited consolidated financial statements as of and for the year ended December 31, 2025. The unaudited interim condensed consolidated financial statements have been prepared on a basis consistent with the audited annual financial statements as of and for the year ended December 31, 2025, and, in the opinion of management, reflect all adjustments, consisting solely of normal recurring adjustments, necessary for the fair presentation of the Company's financial position as of September 30, 2026, and the unaudited condensed consolidated results of its operations and its cash flows for the three and nine months ended September 30, 2026 and 2025. The financial data and other information disclosed in these notes related to the three and nine months ended September 30, 2026 and 2025 are also unaudited. The unaudited condensed consolidated results of operations for the three and nine months ended September 30, 2026 are not necessarily indicative of the results to be expected for the full year ending December 31, 2026 or any other period. These unaudited interim condensed consolidated financial statements should be read in conjunction with the Company's audited consolidated financial statements as of and for the year ended December 31, 2025 included in its Annual Report on Form 10-K filed with the Securities and Exchange Commission, or SEC, on March 31, 2026.

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.

	September 30, 2026	December 31, 2025
Cash and cash equivalents	$ 16,336	$ 69,276
Restricted cash	631	631
Cash, cash equivalents and restricted cash	$ 16,967	$ 69,907

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 reassessed 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 until such time that the warrants are exercised, expire or otherwise qualify for equity classification. 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.

If the Company determines that a warrant subsequently meets the criteria for equity classification, the warrant is remeasured at fair value immediately prior to reclassification, with the resulting change in fair value recorded as a non-cash gain or loss on the consolidated statements of operations and comprehensive loss. The warrant is then reclassified from a liability to additional paid-in capital at its fair value on the reclassification date and is not subsequently remeasured.

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.

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 to be 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, is considered to be a Level 3 fair value measurement and is 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 research and development expenses in the consolidated statements of operations and 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 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.

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 the same as basic net loss per share attributable to common stockholders since the effect of potentially dilutive securities is anti-dilutive given the net loss of the Company. Basic and diluted net loss per share also includes contingently issuable shares once all conditions are satisfied.

3. 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):

	September 30, 2026			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market fund	$ 20	$ —	$ —	$ 20
Commercial paper	—	8,129	—	8,129
Total cash equivalents	20	8,129	—	8,149
Short-term investments:				
Commercial paper	—	3,953	—	3,953
U.S. government debt and agency securities	—	12,021	—	12,021
Total short-term investments	$ —	15,974	$ —	$ 15,974
Total assets subject to fair value measurements on a recurring basis	$ 20	$ 24,103	$ —	$ 24,123
Liabilities:				
Milestone liability	—	—	13,951	13,951
Total liabilities subject to fair value measurements on a recurring basis	$ —	$ —	$ 13,951	$ 13,951

	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

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.

Due to a contingent redemption right exercisable if the Company failed to obtain stockholder approval permitting the exercise of the Sermonix Pharmaceuticals, Inc. ("Sermonix") Pre-Funded Warrant under Nasdaq Rule 5635(a) within one year of issuance, the warrant was classified as a liability pursuant to ASC 480 as of December 31, 2025. As of December 31, 2025, the Company determined the fair value of the Sermonix Pre-Funded Warrant based on the fair value of the Company's common stock, adjusted for a discount for lack of marketability, and classified it as Level 3 due to the use of unobservable market data for identical or similar liabilities. Following receipt of stockholder approval on March 18, 2026, the redemption right lapsed and the Sermonix Pre-Funded Warrant met the criteria for equity classification. Accordingly, the Company remeasured the warrant to fair value immediately prior to reclassification and reclassified the warrant to equity as of March 18, 2026. Following reclassification, the Sermonix Pre-Funded Warrant is no longer subject to recurring fair value measurement.

The key inputs into the fair value of the Sermonix Pre-Funded Warrant at December 31, 2025 were as follows:

	Sermonix Pre-Funded Warrant
	December 31, 2025
Fair Value of Common Stock	$ 7.57
Volatility	97.0%
Risk-free rate	3.7%
Dividend Yield	0.0%
Holding period years	0.20

As of December 31, 2025 and September 30, 2026, Level 3 liabilities include the milestone payable to Sermonix pursuant to the Company's license agreement executed on December 18, 2025 (the "Milestone Liability"). The Milestone Liability is classified as a liability under ASC 480 as the Company is contingently obligated to settle the milestone through the transfer of assets or issuance of a variable number of equity shares with a value equal to a fixed monetary amount.

The Company determines the fair value of the Milestone Liability based on the probability weighted present value of the net milestone payment and classifies it 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 Milestone Liability at December 31, 2025 and at September 30, 2026 were as follows:

	Milestone Liability	
	September 30, 2026	December 31, 2025
Weighted Average Cost of Capital	13.0%	11.0%
Probability	43.9%	43.9%

Below is a summary of the change in fair value of the Sermonix Pre-Funded Warrant and the Milestone Liability from December 31, 2025 to September 30, 2026:

	Sermonix Pre-Funded Warrant	Milestone Liability
December 31, 2025	$ 37,488	$ 15,116
Change in fair value	16,320	(929)
Reclassified to equity	(53,808)	—
March 31, 2026	$ —	$ 14,187
Change in fair value	—	175
June 30, 2026	$ —	$ 14,362
Change in fair value	—	(411)
September 30, 2026	$ —	$ 13,951

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):

		September 30, 2026			
	Level	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
Cash equivalents:					
Money market fund	1	$ 20	$ —	$ —	$ 20
Commercial paper	2	8,131	—	(2)	8,129
Total cash equivalents		$ 8,151	$ —	$ (2)	$ 8,149
Short-term investments:					
Commercial paper	2	3,956	—	(3)	3,953
U.S. government debt and agency securities	2	12,028	—	(7)	12,021
Total short-term investments		$ 15,984	$ —	$ (10)	$ 15,974

		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

All the commercial paper, U.S. government debt and agency securities, U.S. treasury bills, and corporate bonds 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 September 30, 2026, 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 September 30, 2026 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.

4. Property and Equipment, Net

Property and equipment consisted of the following (in thousands):

	September 30, 2026	December 31, 2025
Lab equipment	$ 711	$ 705
Office furniture, fixtures, and computer equipment	712	712
Leasehold improvement	4,322	4,322
Property and equipment, at cost	5,745	5,739
Less: accumulated depreciation	(4,945)	(4,267)
Property and equipment, net	$ 800	$ 1,472

Depreciation expense was $0.2 million and $0.7 million for the three and nine months ended September 30, 2026, respectively. Depreciation expense was $0.2 million and $0.7 million for the three and nine months ended September 30, 2025, respectively.

5. Accrued Liabilities

Accrued liabilities consisted of the following (in thousands):

	September 30, 2026	December 31, 2025
Research and development	$ 1,447	$ 2,012
Employee compensation and benefits	2,872	1,948
Professional services and other	1,761	1,003
Total accrued liabilities	$ 6,080	$ 4,963

6. 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):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Research and development expenses:				
Lasofoxifene	$ 10,536	$ —	$ 28,274	$ —
Fosgonimeton (ATH-1017)	20	317	209	2,180
Brelgometon (ATH-1105)	280	673	817	2,834
ATH-1020	—	—	—	5
Preclinical programs and other costs	734	268	1,553	928
Milestone liability change in fair value	(411)	—	(1,165)	—
Personnel-related costs, excluding stock-based compensation	2,346	895	5,701	2,811
Total research and development expenses	13,505	2,153	35,389	8,758
General and administrative expenses	4,153	3,067	14,635	9,569
Other segment expenses[(a)]	3,809	1,654	8,110	5,374
Total operating expenses	21,467	6,874	58,134	23,701
Loss from operations	(21,467)	(6,874)	(58,134)	(23,701)
Other income, net	303	263	1,335	981
Sermonix pre-funded warrant change in fair value	—	—	(16,320)	—
Net loss	$ (21,164)	$ (6,611)	$ (73,119)	$ (22,720)

[(a)] Other segment expenses includes stock-based compensation and depreciation expenses.

7. 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 September 30, 2026.

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 September 30, 2026, 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):

	September 30, 2026
2026	$ 130
2027	346
Total undiscounted lease payments	476
Present value adjustment for minimum lease commitments	(16)
Net lease liability	$ 460

The weighted average remaining lease term and the weighted average discount rate used to determine the operating lease liability were as follows:

	September 30, 2026
Weighted average remaining lease term (years)	0.9
Weighted average discount rate	8.1%

Operating lease expense and variable lease expense consisted of the following (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Operating lease expense	$ 89	$ 89	$ 265	$ 265
Variable lease expense	46	45	120	131

8. 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:

	September 30, 2026	December 31, 2025
Shares issuable upon the exercise of outstanding common stock options and the vesting of outstanding common restricted stock units granted	6,255,770	1,262,430
Shares available for future grant under the 2020 Equity Incentive Plan	—	63,723
Shares available for future grant under the 2026 Equity Incentive Plan	710,437	—
Shares available for future grant under the Employee Stock Purchase Plan	193,205	156,754
Shares available for future grant under the 2024 Inducement Equity Incentive Plan	5,000	35,000
PIPE Pre-Funded Warrants	8,311,404	8,816,684
Series A Common Warrants	23,031,494	23,031,494
Series B Common Warrants	21,259,842	21,259,842
Sermonix Pre-Funded Warrant	5,502,402	5,502,402
Total	65,269,554	60,128,329

2025 PIPE Financing

In December 2025, the Company entered into a securities purchase agreement (the “PIPE 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 were $90.0 million. Issuance costs of $7.6 million are reflected as a 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 had an exercise price of $6.35 per share and became exercisable after the earlier of (1) the latest of (a) June 30, 2026, (b) the date on which the Company publicly announced the enrollment of the 500th subject or the last subject, whichever was earlier, in the ELAINE-3 trial, and (c) the date on which the FDA approved or issued 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”). By their terms, the Series A Common Warrants remained exercisable only until the 30th day following the Series A Common Warrant Initial Exercise Date (the “Series A Common Warrant Termination Date”). The Series A Common Warrant Initial Exercise Date occurred on September 18, 2026. No Series A Common Warrants were exercised, and the Series A Common Warrants expired and terminated in accordance with their terms on October 19, 2026. Accordingly, no Series A Common Warrants remain outstanding as of the date of this report.

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 met the criteria to be classified as equity instruments.

None of the PIPE Pre-Funded 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 B Common Warrants, an investor would exceed the PIPE Maximum Percentage, then the 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 the Company; provided, however, that such percentage may in no event exceed (x) 19.99% prior to a vote of the Company's stockholders approving the removal of such exercise limitation or (y) with respect to certain investors, 4.99%.

Sermonix Pre-Funded Warrant

As consideration for the rights granted to the Company under the Sermonix License Agreement entered into between the Company and Sermonix 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 is 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”), 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 the Company’s common stock would be aggregated with Sermonix for purposes of Section 13(d) of the Exchange Act, would beneficially own more than 4.99% of the total number of issued and outstanding shares of common stock of the Company following such exercise, or the Sermonix Maximum Percentage. The Sermonix Maximum Percentage may be increased or decreased by Sermonix with 61 days’ written notice to the Company; provided, however, that such percentage may in no event exceed 19.99% prior to receipt of the Sermonix Stockholder Approval. If the Sermonix Stockholder Approval was not obtained by the first anniversary of the original issuance of the Sermonix Pre-Funded Warrant, Sermonix 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 was exercising the Sermonix Redemption Right.

As a result of the Sermonix Redemption Right, the Company initially 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 did not meet the criteria for equity classification.

On March 18, 2026, at the Company’s special meeting of its stockholders, the Company’s stockholders approved the proposal to permit the exercise of the Sermonix Pre-Funded Warrant under Nasdaq Rule 5635(a). As a result, the Sermonix Redemption Right lapsed and the Company determined the Sermonix Pre-Funded Warrant met the criteria for equity classification. As a result, the Company remeasured the warrant to fair value immediately prior to reclassification, with the change in estimated fair value recorded as a non-cash gain or loss on the consolidated statements of operations and comprehensive loss for the nine months ended September 30, 2026. Subsequently, the Company reclassified the Sermonix Pre-Funded Warrant to equity on March 18, 2026.

Following receipt of the Sermonix Stockholder Approval, Sermonix became eligible to exercise that portion of the Sermonix Pre-Funded Warrant that equaled 4.99% of outstanding Company common stock (which limitation applied to Sermonix and any affiliates on an aggregate basis). On August 24, 2026, Sermonix notified the Company that it was increasing the Sermonix Maximum Percentage to 49.99%, effective on October 26, 2026. On October 26, 2026, Sermonix effected an assignment to transfer a portion of the Sermonix Pre-Funded Warrant to purchase 2,352,932 shares of common stock of the Company to Perceptive Xontogeny Venture Fund II, LP, or Perceptive Xontogeny, as a distribution-in-kind to a stockholder of Sermonix. Perceptive Xontogeny and its affiliates are greater than five percent holders of the Company's common stock and directors Joseph Edelman and Fred Callori are affiliates of Perceptive Xontogeny and member of the board of directors of the Company. On October 26, 2026, Sermonix exercised the Sermonix Pre-Funded Warrant with respect to 3,149,470 shares of Company common stock and following such exercise is a greater than five percent holder of the Company's common stock.

Equity Incentive Plans

The Company’s board of directors (the “Board”) previously approved, subject to stockholder approval, the Company’s 2026 Equity Incentive Plan (the “2026 Plan”). At the Special Meeting, held on March 18, 2026, the Company’s stockholders approved

the 2026 Plan. There will be no further grants under the Company's prior company-wide equity plan, the 2020 Equity Incentive Plan (the "2020 Plan"), which terminated for future use upon stockholder approval of the 2026 Plan. Termination of the 2020 Plan did not affect outstanding awards previously issued pursuant to the 2020 Plan. A total of 5,700,000 shares of the Compnay's common stock were reserved for issuance pursuant to the 2026 Plan. In addition, the shares reserved for issuance under the 2026 Plan will include any shares subject to awards granted under the 2020 Plan that, on or after the stockholder approval date of the 2026 Plan, are used to pay for the tax withholding related to the award or the exercise price with the award or, with respect to any options and stock appreciation rights, expire or become unexercisable without having been exercised in full, or with respect to any other types of awards under the 2020 Plan, are forfeited to or repurchased by the Company due to failure to vest, or are not issued under an award due to the award being paid out in cash rather than shares of common stock (provided that the maximum number of shares of common stock that may be added to the 2026 Plan pursuant to this sentence is 1,300,000 shares). The 2026 Plan provides for annual increases in the number of shares that may be issued under the 2026 Plan on January 1, 2027 and each subsequent January 1 thereafter by a number of shares equal to the lesser of (1) 5% of the aggregate of outstanding shares of common stock and outstanding pre-funded warrants to purchase shares of common stock on the immediately preceding December 31, and (2) an amount determined by the Company's board of directors.

The Company's 2020 Plan provided 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 2020 Plan terminated on March 18, 2026, the date the Company's stockholders approved the 2026 Plan.

The Company's 2020 Employee Stock Purchase Plan, or 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 Company's 2024 Inducement Equity Incentive Plan, or 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.

9. Stock-based Compensation

Stock-based compensation expense recognized was as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Research and development	$ 1,011	$ 574	$ 2,154	$ 1,737
General and administrative	2,578	836	5,278	2,907
Total stock-based compensation expense	$ 3,589	$ 1,410	$ 7,432	$ 4,644

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 requires 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:

	Nine Months Ended September 30,	
	2026	2025
Risk-free interest rate	4.03%	3.98%
Expected volatility	105.85%	100.54%
Expected term (in years)	5.98	5.97
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 nine months ended September 30, 2026 and 2025 was $38.0 million and $0.4 million, respectively. The fair value of RSUs granted during the nine months ended September 30, 2026 and 2025 was $1.6 million and $0.2 million, respectively.

Stock Option Activity

Changes in shares available for grant under the 2026 Plan, the 2020 Plan, and the 2024 Inducement Plan during the nine months ended September 30, 2026 were as follows:

	Shares Available for Grant
Shares available for grant at December 31, 2025	98,723
2020 Plan reserve increase on January 1, 2026	323,000
Options and restricted stock units granted	(5,098,454)
Options and restricted stock units forfeited, cancelled, or expired	47,513
Termination of future use of 2020 Plan	(355,345)
Shares reserved for issuance under 2026 Plan	5,700,000
Shares available for grant at September 30, 2026	715,437

A summary of stock option activity under the 2026 Plan, the 2020 Plan, and the 2024 Inducement Plan for the nine months ended September 30, 2026 is 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, 2025	1,204,829	$ 37.44	8.14	$ 2,030
Granted	4,933,454	9.30	—	—
Exercised	—	—	—	—
Forfeited/expired	(47,513)	27.90	—	—
Balance at September 30, 2026	6,090,770	$ 14.72	9.14	$ 5
Expected to vest	5,061,217	$ 9.20	9.47	—
Options exercisable	1,029,553	$ 41.89	7.51	$ 5

The total fair value of options granted that vested during the nine months ended September 30, 2026 and 2025 was $4.3 million and $5.9 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 September 30, 2026. There were no options exercised during the nine months ended September 30, 2026 and 2025. As of September 30, 2026, there was $36.1 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 3.2 years. The Company utilizes newly issued shares to satisfy option exercises.

Stock options outstanding and exercisable under the 2026 Plan, the 2020 Plan, and the 2024 Inducement Plan consisted of the following at September 30, 2026.

Exercise Price ($)	Options Outstanding	Options Exercisable
3.13 to 7.14	628,261	138,363
7.47 to 13.50	4,849,621	363,375
20.40 to 41.10	406,844	321,771
89.30 to 294.10	206,044	206,044
Total	6,090,770	1,029,553

Restricted Stock Unit Activity

A summary of restricted stock unit ("RSU") activity under the 2026 Plan and the 2020 Plan for the nine months ended September 30, 2026 is as follows:

	Share Equivalent	Weighted-Average Grant Date Fair Value
Non-vested at December 31, 2025	57,601	$ 3.76
Granted	165,000	9.54
Cancelled	—	—
Vested	(57,601)	3.76
Non-vested at September 30, 2026	165,000	$ 9.54

As of September 30, 2026, there was $0.6 million of total unrecognized compensation costs related to non-vested RSUs. The Company expects to recognize this cost over a remaining weighted-average period of 0.16 years.

10. 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:

	Nine Months Ended September 30,	
	2026	2025
Stock options to purchase common stock	6,090,770	1,212,908
Non-vested Restricted Stock Units	165,000	86,949
Employee stock purchase plan	3,219	2,330
Series A Common Warrants	23,031,494	—
Series B Common Warrants	21,259,842	—
Total	50,550,325	1,302,187

Item 2. 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 unaudited condensed consolidated financial statements and notes thereto included in Part I, Item 1 of this report and with our audited financial statements and related notes thereto and Management's Discussion and Analysis of Financial Condition and Results of Operations included in our Annual Report on Form 10-K, filed with the Securities and Exchange Commission, or the SEC, on March 31, 2026.

Special Note Regarding Forward-Looking Statements

This report 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 unaudited condensed consolidated financial statements and related notes included in Part I, Item 1 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, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or 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 a Phase 2 brelgometon clinical trial and any subsequent clinical trials to show the beneficial characteristics, safety and efficacy of brelgometon;
- 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 II, 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 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 rights 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.

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.

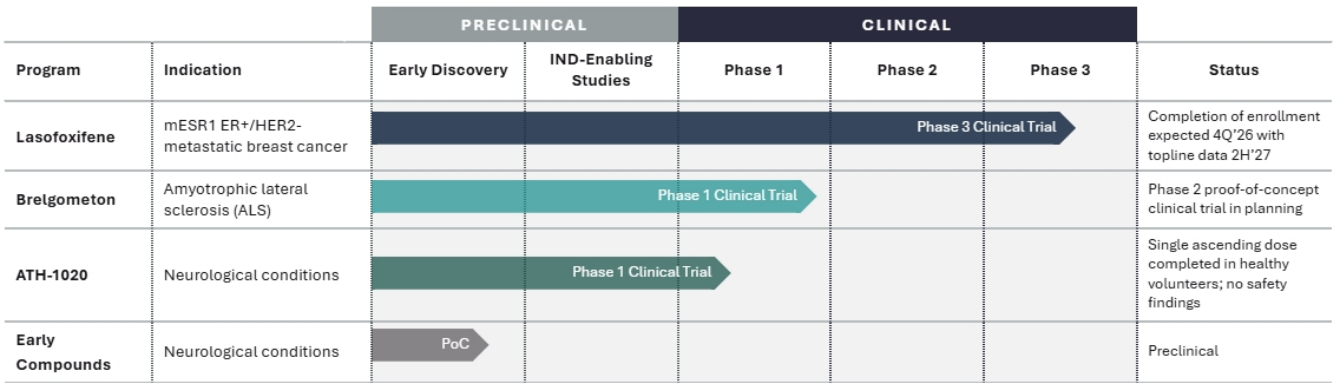
In December 2025, we acquired rights to a promising late-stage asset, lasofoxifene, for the potential treatment of ESR1-mutated (“mESR1”) metastatic breast cancer. This late-stage program has generated promising clinical data and we believe it represents a unique and exciting opportunity to diversify our pipeline. We believe this program fits well with our later-stage clinical development experience, and complements our in-house asset, brelgometon (formerly known as 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 brelgometon, 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

The following figure illustrates the current development stage of our proprietary drug candidates and early discovery and development programs, of which only lasofoxifene and brelgometon 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. Brelgometon, 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.



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 commercial manufacturing organizations, or CMOs, to manufacture and supply our preclinical and clinical materials during the development of our drug candidates. We believe the synthesis of lasofoxifene and brelgometon 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 September 30, 2026, 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 $73.1 million and $22.7 million for the nine months ended September 30, 2026 and 2025, respectively. As of September 30, 2026, we had an accumulated deficit of $584.9 million and cash, cash equivalents and investments of $32.3 million.

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

- continue to advance lasofoxifene, brelgometon 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 not be sufficient to fund our operating expenses and capital expenditure requirements under our current operating plan through at least the next 12 months following the date of this report.

Recent Developments

We expect to complete enrollment in our ELAINE-3 clinical trial for lasofoxifene in the fourth quarter of 2026 and to have topline data in the second half of 2027 for the ELAINE-3 trial. We expect to provide an update on the timing of our proposed Phase 2 proof-of-concept clinical trial for brelgometon following completion of enrollment of the ELAINE-3 clinical trial as we continue to focus on our lasofoxifene program.

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 brelgometon. 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 brelgometon. Drug candidates in later stages of development generally have higher development costs than those in earlier stages. Subject to our ability to obtain additional funding, 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 brelgometon, 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 brelgometon;
- 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, subject to our ability to obtain additional funding, as we 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 Three Months Ended September 30, 2026 and 2025

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

	Three Months Ended September 30,			
	2026	2025	Dollar Change	% Change
		(in thousands)		
Operating expenses:				
Research and development	$ 15,015	$ 2,825	$ 12,190	432%
Milestone liability change in fair value	(411)	—	(411)	*
General and administrative	6,863	4,049	2,814	69%
Total operating expenses	21,467	6,874	14,593	212%
Loss from operations	(21,467)	(6,874)	(14,593)	212%
Other income, net	303	263	40	15%
Net loss	$ (21,164)	$ (6,611)	$ (14,553)	220%

*Not meaningful

Research and Development Expenses

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

	Three Months Ended September 30,			
	2026	2025	Dollar Change	% Change
		(in thousands)		
Direct costs:				
Lasofoxifene	$ 10,536	$ —	$ 10,536	*
Fosgonimeton (ATH-1017)	20	317	(297)	-94%
Brelgometon (ATH-1105)	280	673	(393)	-58%
Preclinical programs and other direct costs	253	61	192	315%
Total direct costs	11,089	1,051	10,038	955%
Indirect costs:				
Milestone liability change in fair value	(411)	—	(411)	*
Personnel-related costs, including stock-based compensation	3,357	1,469	1,888	129%
Facilities and other costs	569	305	264	87%
Total research and development expenses	$ 14,604	$ 2,825	$ 11,779	417%

*Not meaningful

Research and development expenses increased by $11.8 million, from $2.8 million for the three months ended September 30, 2025 to $14.6 million for the three months ended September 30, 2026. The increase was driven primarily by increases in lasofoxifene program costs of $10.5 million and personnel-related costs, including stock-based compensation of $1.9 million. These were offset by decreases in fosgonimeton program costs of $0.3 million and brelgometon program costs of $0.4 million. The $10.5 million increase in lasofoxifene program costs was driven by increases in contract research organization, clinical site visit and other third-party vendor costs of $9.3 million and an increase in contract manufacturing costs of $1.2 million. The $1.9 million increase in personnel-related costs, including stock-based compensation was driven by an increase in headcount to support the ELAINE-3 trial for lasofoxifene. The decrease in fosgonimeton program costs of $0.3 million was driven by decreases in contract research organization, other third-party vendor and clinical site visit costs of $0.3 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. The decrease in brelgometon program costs of $0.4 million was driven by a decrease in contract research organization and other third-party vendor costs of $0.4 million. Refer to Note 3 to our unaudited condensed consolidated financial statements included elsewhere in this report for discussion regarding the milestone liability change in fair value.

General and Administrative Expenses

General and administrative expenses increased by approximately $2.8 million, from $4.0 million for the three months ended September 30, 2025 to $6.9 million for the three months ended September 30, 2026. The increase was primarily due to an increase in personnel-related expenses, including stock-based compensation of $2.7 million.

Other Income, Net

Other income, net, did not meaningfully change for the three months ended September 30, 2026 compared to the three months ended September 30, 2025.

Comparison of the Nine Months Ended September 30, 2026 and 2025

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

	Nine Months Ended September 30,			
	2026	2025	Dollar Change	% Change
	(in thousands)			
Operating expenses:				
Research and development	$ 38,979	$ 10,788	$ 28,191	261%
Milestone liability change in fair value	(1,165)	—	(1,165)	*
General and administrative	20,320	12,913	7,407	57%
Total operating expenses	58,134	23,701	34,433	145%
Loss from operations	(58,134)	(23,701)	(34,433)	145%
Other income, net	1,335	981	354	36%
Sermonix pre-funded warrant change in fair value	(16,320)	—	(16,320)	*
Net loss	$ (73,119)	$ (22,720)	$ (50,399)	222%

*Not meaningful

Research and Development Expenses

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

	Nine Months Ended September 30,			
	2026	2025	Dollar Change	% Change
	(in thousands)			
Direct costs:				
Lasofoxifene	$ 28,274	$ —	$ 28,274	*
Fosgonimeton (ATH-1017)	209	2,180	(1,971)	-90%
Brelgometon (ATH-1105)	817	2,834	(2,017)	-71%
ATH-1020	—	5	(5)	*
Preclinical programs and other direct costs	427	266	161	61%
Total direct costs	29,727	5,285	24,442	462%
Indirect costs:				
Milestone liability change in fair value	(1,165)	—	(1,165)	*
Personnel-related costs, including stock-based compensation	7,855	4,549	3,306	73%
Facilities and other costs	1,397	954	443	46%
Total research and development expenses	$ 37,814	$ 10,788	$ 27,026	251%

*Not meaningful

Research and development expenses increased by $27.0 million, from $10.8 million for the nine months ended September 30, 2025 to $37.8 million for the nine months ended September 30, 2026. The increase was driven primarily by increases in lasofoxifene program costs of $28.3 million and personnel-related costs, including stock-based compensation of $3.3 million. These were offset by decreases in fosgonimeton program costs of $2.0 million and brelgometon program costs of $2.0 million. The $28.3 million increase in lasofoxifene program costs was driven by increases in contract research organization, clinical site visit and other third-party vendor costs of $22.2 million and an increase in contract manufacturing costs of $6.0 million. The $3.3 million increase in personnel-related costs, including stock-based compensation was driven by an increase in headcount to support the ELAINE-3 trial for lasofoxifene. The decrease in fosgonimeton program costs of $2.0 million was driven by decreases in contract research organization, other third-party vendor and clinical site visit costs of $1.7 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. The decrease in brelgometon program costs of $2.0 million was driven by a decrease in contract research organization and other third-party vendor costs of $0.8 million and a decrease in research costs of $0.9 million. Refer to Note 3 to our unaudited condensed consolidated financial statements included elsewhere in this report for discussion regarding the milestone liability change in fair value.

General and Administrative Expenses

General and administrative expenses increased by approximately $7.4 million, from $12.9 million for the nine months ended September 30, 2025 to $20.3 million for the nine months ended September 30, 2026. The increase was primarily due to an increase in personnel-related expenses, including stock-based compensation of $4.5 million and an increase in professional service expenses of $2.4 million.

Other Income, Net

Other income, net, increased by $0.3 million, from $1.0 million for the nine months ended September 30, 2025 to $1.3 million for the nine months ended September 30, 2026 due to higher income from accretion of discounts on debt securities purchased below par value and held to maturity and higher interest income earned on our available-for-sale securities. These increases resulted primarily from higher balances of available-for-sale securities held during the nine months ended September 30, 2026 compared to the nine months ended September 30, 2025.

Sermonix Pre-Funded Warrant Change in Fair Value

Sermonix Pre-Funded Warrant change in fair value increased by $16.3 million, from $0 for the nine months ended September 30, 2025 to $16.3 million for the nine months ended September 30, 2026. Refer to Note 3 to our unaudited condensed 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, pre-funded common stock warrants and convertible notes, and to a lesser extent from grant income and stock option exercises. From our inception through September 30, 2026, we have raised aggregate net cash proceeds of $489.8 million primarily from the issuance of our common stock (excluding option exercises), convertible preferred stock, common stock warrants, pre-funded common stock warrants and convertible notes.

On December 18, 2025, we entered into the PIPE Securities Purchase Agreement with a select group of investors, including Commodore Capital LP, or Commodore, TCGX Crossover Management LLC, or 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 were required to prepare and file a registration statement with the SEC to register the resale of the PIPE Shares, to have the registration statement declared effective and to maintain effectiveness of the registration statement, subject to certain exceptions. The registration statement was declared effective in April 2026. 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 Series A Common Warrants had an exercise price of $6.35 per share and became exercisable after the earlier of (1) the latest of (a) June 30, 2026, (b) the date on which the Company publicly announced the enrollment of the 500th subject or the last subject, whichever was earlier, in the ELAINE-3 trial, and (c) the date on which the FDA approved or issued 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"). By their terms, the Series A Common Warrants remained exercisable only until the 30th day following the Series A Common Warrant Initial Exercise Date (the "Series A Common Warrant Termination Date"). The Series A Common Warrant Initial Exercise Date occurred on September 18, 2026. No Series A Common Warrants were exercised, and the Series A Common Warrants expired and terminated in accordance with their terms on October 19, 2026. Accordingly, no Series A Common Warrants remain outstanding as of the date of this report. 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 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 B Common Warrants, a PIPE Purchaser would exceed the PIPE Maximum Percentage, then the 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.

As of September 30, 2026, we had $32.3 million in cash, cash equivalents and investments and have not generated positive cash flows from operations. 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 September 30, 2026, we had lease payment obligations of $0.5 million, with $0.5 million payable within 12 months. For additional information regarding our lease commitments, refer to Note 7 to our unaudited condensed 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. These purchase obligations and open purchase orders are generally cancellable in full or in part through the contractual provisions. Subject to our ability to obtain additional financing, 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 brelgometon, 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, subject to our ability to obtain additional funding, as we support our continued research activities and development of our programs.

Based upon our current operating plan, we estimate that our $32.3 million of cash, cash equivalents and investments at September 30, 2026 will not be sufficient to fund our operating expenses and capital expenditure requirements under our current operating plan for at least the next 12 months which raises substantial doubt as to our ability to continue as a going concern 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. The Series A Common Warrants issued in connection with the Private Placement became exercisable on the Series A Common Warrant Initial Exercise Date and, by their terms, remained exercisable only until the 30th day thereafter. No Series A Common Warrants were exercised, and they expired and terminated in accordance with their terms on October 19, 2026. As a result, the Series A Common Warrants did not provide, and will not provide, any additional capital to us. See Note 8 to our unaudited condensed consolidated financial statements included elsewhere in this report. If we are unsuccessful in raising additional capital, or we do not raise sufficient capital to fund our current operating plan, we will need to consider steps to extend our cash runway. These steps may include reductions in force, reduced expenditures on our current clinical development programs, including with respect to commercialization efforts for lasofoxifene, or a delay or pause of additional clinical or pre-clinical development efforts. We are actively pursuing financing alternatives to raise additional capital for operations and the continued advancement of lasofoxifene and our other product candidates. In connection with these financing efforts, we will engage in negotiations with investors regarding the economic terms of any investment, which may include equity issuances at a discount to the prevailing market price, tranched investment structures with subsequent investments tied to the satisfaction of certain clinical development milestones, and the issuance of additional warrants with varied exercisability triggers. Even if we are successful in raising additional capital to fund our current operating plan, we may consider implementing additional changes to our operations following such capital raising transaction in support of our corporate strategy, including changes to the prioritization of clinical and pre-clinical development efforts outside of lasofoxifene, personnel changes, including with respect to members of our management team, cost-containment measures, and other measures.

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; however, we are currently unable to offer and sell shares under this facility due to our ineligibility to use a Registration Statement on Form S-3 through December 2026. 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 brelgometon 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 brelgometon 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:

	Nine Months Ended September 30,	
	2026	2025
	(in thousands)	
Net cash (used in) provided by:		
Operating activities	$ (56,755)	$ (26,332)
Investing activities	3,701	(1,256)
Financing activities	114	24
Net increase (decrease) in cash, cash equivalents and restricted cash	$ (52,940)	$ (27,564)

Operating Activities

During the nine months ended September 30, 2026, net cash used in operating activities was $56.8 million. This consisted primarily of a net loss of $73.1 million, partially offset by non-cash charges of $22.9 million, and an increase in our net operating assets of $6.5 million. The non-cash charges primarily consisted of a change in fair value of our Sermonix pre-funded warrant and milestone liability, stock-based compensation expense, depreciation expense, non-cash lease expense, and amortization of premiums and accretion of discounts on available-for-sale securities. The increase in our net operating assets was primarily due to a decrease in accounts payable and accrued liabilities, an increase in prepaid expenses and other assets, and decreases in operating lease liability and long-term liabilities.

During the nine months ended September 30, 2025, net cash used in operating activities was $26.3 million. This consisted primarily of a net loss of $22.7 million, partially offset by non-cash charges of $5.3 million, and an increase in our net operating assets of $8.9 million. The non-cash charges primarily consisted of stock-based compensation expense, depreciation expense, non-cash lease expense, and amortization of premiums and accretion of discounts on available-for-sale securities. The increase in our net operating assets was primarily due to a decrease in accrued expenses, partially offset by an increase in accounts payable and a decrease in prepaid expenses and other assets.

Investing Activities

During the nine months ended September 30, 2026, net cash provided by investing activities was $3.7 million. This consisted of maturities of available-for-sale securities of $42.5 million, partially offset by purchases of available-for-sale securities of $38.8 million.

During the nine months ended September 30, 2025, net cash used in investing activities was $1.3 million. This consisted of purchases of available-for-sale securities of $19.6 million, partially offset by maturities of available-for-sale securities of $18.3 million.

Financing Activities

During the nine months ended September 30, 2026, net cash provided by financing activities was $0.1 million, consisting of proceeds received from the issuance of common stock under our employee stock purchase plan.

During the nine months ended September 30, 2025, net cash provided by financing activities was $0.02 million, consisting of proceeds received from the issuance of common stock under our employee stock purchase plan.

Critical Accounting Policies, Significant Judgments and Use of Estimates

Our financial statements have been prepared in accordance with U.S. generally accepted accounting principles, or GAAP. 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.

Critical accounting policies and significant judgments and estimates are those that we consider the most important to the portrayal of our financial condition and results of operations because they require our most difficult, subjective or complex judgments, often as a result of the need to make estimates about the effect of matters that are inherently uncertain.

During the nine months ended September 30, 2026, there were no material changes to our critical accounting policies. Our critical accounting policies are described under the heading “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Critical Accounting Policies, Significant Judgments and Use of Estimates” in our Annual Report on Form 10-K filed with the SEC on March 31, 2026 and Note 2 to our unaudited condensed consolidated financial statements included elsewhere in this report. We believe that of our critical accounting policies, the following accounting policies involve the most judgment and complexity:

- research and development costs;
- stock-based compensation; and
- income taxes.

Item 3. 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 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

We maintain "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, or the Exchange Act, that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is (1) recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms and (2) accumulated and communicated to our management, including our principal executive and principal financial officer, as appropriate to allow timely decisions regarding required disclosure. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our disclosure controls and procedures are designed to provide reasonable assurance of achieving their control objectives.

Our management, with the participation of our principal executive officer and principal financial and accounting officer, evaluated the effectiveness of our disclosure controls and procedures at the end of the period covered by this Quarterly Report on Form 10-Q. Based upon such evaluation, our principal executive officer and principal financial and accounting officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of such date.

Changes in Internal Control Over Financial Reporting

There have been no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the quarter ended September 30, 2026, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

From time to time, we may be subject to various legal proceedings or claims that arise in the ordinary course of business. As of September 30, 2026, 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 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 unaudited condensed 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, brelgometon, 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 novel 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. 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 brelgometon 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 brelgometon, 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 central nervous system. Our small molecule drug candidates are designed to penetrate the blood brain barrier 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 brelgometon, 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 brelgometon in November 2024. The results of the Phase 1 clinical trial showed that brelgometon 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; 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 brelgometon and other small molecules in our drug product pipeline. We or a future partner may ultimately determine that brelgometon, 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 brelgometon may never lead to marketable products.

We are developing lasofoxifene and brelgometon 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 brelgometon is approved, educating medical personnel regarding the potential efficacy and safety benefits, as well as the challenges, of incorporating lasofoxifene or brelgometon 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 brelgometon. 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 brelgometon as a potential treatment for ALS and other neurodegenerative diseases. Like fosgonimeton, brelgometon is a small molecule designed to positively modulate the HGF system. Although we are encouraged about the potential for brelgometon 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, brelgometon 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 in-house 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 focused our research and development efforts on addressing central nervous system, or CNS and peripheral nervous system, or 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 blood brain barrier 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 Alzheimer’s disease, 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. In addition, the protocol for our Phase 3 ELAINE-3 clinical trial for lasofoxifene currently contemplates a pre-specified futility analysis after fifty percent (50%) of expected

progression-free survival events per RECIST 1.1 criteria are observed by blinded independent central review, which is currently anticipated to occur sometime after completion of enrollment. While the purpose of this futility analysis will be solely to determine whether or not the trial should continue, and is thus not an assessment of efficacy, there can be no assurance regarding the results of the analysis, how long it will take to reach the number of events needed for the pre-specified futility analysis, or whether the clinical trial will continue.

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 risk evaluation and mitigation strategy (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 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.

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, brelgometon 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, brelgometon, 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 subsection titled "Competition" under the section titled "Business" in our Annual Report on Form 10-K filed with the SEC on March 31, 2026.

We are developing lasofoxifene in combination with abemaciclib, and we may in the future develop other drug candidates in combination with other therapies, which could expose us to additional risks.

We are currently developing 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 combination with other targeted therapies such as mTOR or PI3K pathway inhibitors. We may develop other drug candidates in combination with one or more other approved or unapproved therapies. Even if lasofoxifene or any other drug candidate we develop were to receive marketing approval or be commercialized for use in combination with other marketed 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 marketed 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 to support the use of our product candidates with other approved therapies. The occurrence of any of these risks could have a material adverse effect on the approval of our candidates for use with different combination therapies and the commercial success of our approved drug products.

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.

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 brelgometon. Developing lasofoxifene and brelgometon and conducting clinical trials for the potential treatment of breast cancer and ALS, 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 September 30, 2026, we had cash, cash equivalents and investments of $32.3 million. Based upon our current operating plan, we estimate that our existing cash, cash equivalents and investments will not 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 depend 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 brelgometon 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 brelgometon 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 are 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 & 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; however, we are currently unable to offer and sell shares under this facility due to our ineligibility to use a Registration Statement on Form S-3 through December 2026. 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 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.

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 maintain effectiveness of registration statements in accordance with the terms of registration rights agreements related to our December 2025 private placement and Sermonix Pre-Funded Warrant issuance.

In connection with our December 2025 private placement and Sermonix Pre-Funded Warrant 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, Sermonix and their successors and permitted assigns, 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 Pre-Funded Warrant, 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 the registration statements became effective in April 2026, we may be unsuccessful in maintaining

effectiveness of the resale registration statements in the future 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 $73.1 million and $22.7 million for the nine months ended September 30, 2026 and 2025, respectively, and an accumulated deficit of $584.9 million as of September 30, 2026.

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, or 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.

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, changes in the agency leadership and staff departures, government shutdown, lapse of U.S. government appropriations, and layoffs 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, the White House 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 Food and Drug Omnibus Reform Act of 2022 ("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 reviewed 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 Patient Protection and Affordable Care Act (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 was eliminated. Elimination of this cap may require pharmaceutical manufacturers to pay more in rebates than they receive 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, have initiated lawsuits against the federal government asserting that the price negotiation provisions of IRA are unconstitutional. In January 2026, CMS launched the GENEROUS Model (GENErating cost Reductions fOr U.S. Medicaid Model), which aims to ensure fair and reasonable drug prices for Medicaid through CMS-led negotiations with drug manufacturers. Under the GENEROUS Model, manufacturers will provide supplemental rebates to participating states for drugs included in the model to align Medicaid net prices with what certain other countries pay. The GENEROUS Model is voluntary for manufacturers and 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. In October 2026, CMS published the Global Benchmark for Efficient Drug Pricing (GLOBE) Model Final Rule, which will implement a mandatory Medicare Part B five-year demonstration payment model that will require manufacturers of certain high-spend Part B drugs and biologicals to pay rebates to CMS tied to international drug prices for those products. The final rule added exclusions for orphan-only drugs, cellular and gene therapy products, and plasma-derived products, and delayed the start of the first performance year to April 1, 2027. CMS also noted that it intends to waive GLOBE's mandatory participation requirements for manufacturers in any calendar quarter during which they are participating in the GENEROUS Model. The GLOBE Model final rule is the subject of ongoing litigation, which can impact its implementation. 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.

Failure to comply with anti-bribery and anti-corruption laws and anti-money laundering laws, and similar laws, could subject us to penalties and other adverse consequences.

We are subject to the U.S. Foreign Corrupt Practices Act of 1977 (the "FCPA"), the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the United Kingdom Bribery Act 2010, and possibly other anti-bribery and anti-corruption laws and anti-money laundering laws in countries outside of the United States where we conduct our activities. Anti-corruption and anti-bribery laws have been enforced aggressively in recent years and are interpreted broadly to generally prohibit companies, their employees, agents, representatives, business partners, and third-party intermediaries from authorizing, offering, or providing, directly or indirectly, improper payments or benefits to recipients in the public or private sector.

We have interactions with officials and employees of government agencies or government-affiliated hospitals, universities, and other organizations. We expect to leverage third parties to sell our products, assist with clinical trials, assist with obtaining necessary permits, licenses, patent registrations and other regulatory approvals and otherwise conduct our business abroad. We, our employees, agents, representatives, business partners and third-party intermediaries may have direct or indirect interactions with officials and employees of government agencies, government-affiliated hospitals, universities or state-owned or affiliated entities and we may be held liable for the corrupt or other illegal activities of these employees, agents, representatives, business partners or third-party intermediaries even if we do not explicitly authorize such activities. We cannot assure you that all of our employees, agents, representatives, business partners or third-party intermediaries will not take actions in violation of applicable law for which we may be ultimately held responsible. As we increase our international business or commence international sales, our risks under these laws may increase.

These laws also require that we keep accurate books and records and maintain internal controls and compliance procedures designed to prevent any such actions. While we have policies and procedures to address compliance with such laws, we cannot assure you that none of our employees, agents, representatives, business partners or third-party intermediaries will take actions in violation of our policies and applicable law, for which we may be ultimately held responsible.

Any allegations or violation of the FCPA or other applicable anti-bribery and anti-corruption laws and anti-money laundering laws could result in whistleblower complaints, sanctions, settlements, prosecution, enforcement actions, fines, damages, adverse media coverage, investigations, severe criminal or civil sanctions, or suspension or debarment from government contracts, all of which may have an adverse effect on our reputation, business, results of operations, and prospects. Responding to any investigation or action will likely result in a materially significant diversion of management's attention and resources and significant defense costs and other professional fees.

We are subject to U.S. and certain foreign export and import controls, sanctions, embargoes, data export, and foreign investment laws and regulations. Compliance with these legal standards could impair our ability to compete in domestic and international markets. We can face criminal and/or civil liability and other serious consequences for violations, which can harm our business.

Our product candidates, as well as any related technology, may be subject to export control and import laws and regulations, including the Export Administration Regulations administered by the U.S. Department of Commerce, Bureau of Industry and Security ("BIS"), customs regulations administered by U.S. Customs and Border Protection, and various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Control. We may also be subject to other state and national import, sanctions, export control, and foreign investment laws in countries in which we conduct activities.

As such, licenses or notices may be required to import products, technologies, and services from, or export, re-export, or transfer (in-country) products, technologies, and services to, certain countries, end users, or for certain end uses. For example, the U.S. government continues to add additional entities in China and elsewhere to restricted party lists impacting the ability of U.S. companies to provide products, technology, and services to and in some cases receive products, technologies, and services from, these entities. The process for obtaining necessary licenses and making required notices may be time-consuming or unsuccessful, potentially causing delays in sales or losses of sales opportunities. Trade controls are complex and dynamic regimes and monitoring and ensuring compliance can be challenging. Any failure to comply with these regimes could subject us to both civil and criminal penalties, including substantial fines, possible incarceration of responsible individuals for willful violations, possible loss of our export or import privileges, and reputational harm. In addition, investigating or defending against any such allegations, actions, or investigations will likely result in a materially significant diversion of management's attention and resources and significant defense costs and other professional fees. We expect our non-U.S. activities to increase in time, and plans to engage counterparties engaged in international business activities could increase our exposure to these laws and regulations, even if we have limited control over the activities of these counterparties.

There is currently significant uncertainty about the future relationship between the U.S. and various other countries with respect to trade restrictions, treaties, foreign investment laws, data transfer restrictions, and other limitations on cross-border operations. 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. For example, the National Defense Authorization Act of 2026, Section 851, imposes a "prohibition on contracting with certain biotechnology providers" (the BIOSECURE Act), which restricts federal government contracts, grants, and loans from being issued to companies that use biotechnology equipment or services from any designated "biotechnology company of concern," as part of such companies' performance of those agreements with the U.S. government. Once fully implemented through issuance of regulations, the BIOSECURE Act may ultimately limit certain U.S. biotechnology companies from using equipment or services produced or provided by Chinese biotechnology companies that meet the designation criteria of the new law, or certain affiliated entities. In addition, even if we do not seek any covered federal government contracts, grants, or loans, commercial partners, government agencies, or other third parties may view our business less favorably if we contract with entities that ultimately become biotechnology companies of concern. Other regulations could affect the transfer of certain types of data abroad, including to China; for example, the Department of Justice issued a final rule which took effect in April 2025 that places limitations, and in some cases prohibitions, on certain transfers of sensitive personal data to business partners located in China and other designated countries, or with other specified links to China and other designated countries. These rules also may broadly require us to extract promises from other third-party service providers that they will not transfer data we share with them onward to parties linked to countries of concern. Moreover, in the event we seek to grow through acquisitions of companies with foreign operations, or we ourselves may become an acquisition target, such transactions could trigger foreign direct investment screening under one or more national security screening regimes — including review by the Committee on Foreign Investment in the United States ("CFIUS") if we are acquired by a foreign person, and/or review by the national security screening mechanisms adopted by various other jurisdictions in which we or an acquisition target have operations. These regimes vary by jurisdiction, continue to evolve, and may subject us or a transaction to mandatory notification, extended review, mitigation conditions, or prohibition, any of which could delay, restructure, or prevent an acquisition our board and stockholders might otherwise favor, and could adversely affect our growth strategy.

We cannot predict what further actions may ultimately be taken with respect to trade relations between the U.S. and other countries, what products and services may be subject to such actions or what actions may be taken by the other countries in retaliation. If we are unable to obtain or use services from existing service providers or become unable to export or sell our products to any of our customers, our business, liquidity, financial condition, and/or results of operations would be materially and adversely affected.

Recent and further changes in the tariff and trade policies of the U.S. or of other countries could increase manufacturing and sourcing costs, decrease demand for our offerings, disrupt supply chains, or otherwise adversely affect our business and financial condition.

There is currently significant uncertainty about the future relationship between the U.S and its trading partners with respect to trade policies, tariffs, taxes, and similar policies affecting cross-border operations. For example, the U.S. government has made and continues to make significant additional changes in U.S. trade policy, specifically tariffs, and may continue to take future actions that could negatively impact our business. Since 2025, the U.S. government has from time to time significantly escalated tariffs on the import of goods from most U.S. trading partners, including in amounts generally between 10% and 125% through "reciprocal" and "fentanyl-related" tariffs imposed under the International Emergency Economic Powers Act ("IEEPA"); through the use of trade authorities provided under Section 232 of the Trade Expansion Act of 1962, including on certain pharmaceuticals and

pharmaceutical products (beginning for imports of certain companies' patented and branded medicines beginning in July 2026, and expanding more broadly in September 2026); under Section 301 of the Trade Act of 1974, most prominently on items from China; and under Section 122 of the Trade Act of 1974 as a 10% "temporary import surcharge" on imports from most U.S. trading partners. In addition, the U.S. Department of Commerce has initiated Section 232 investigations into additional products, and the U.S. Trade Representative has initiated Section 301 investigations into the trade policies and actions of additional countries and proposed the imposition of new tariffs of 10-12.5% on imports from various U.S. trading partners. When these investigations are complete, the U.S. government may decide to levy additional tariffs on these or other products and/or countries.

In response to these and other U.S. trade measures, China, Canada, and other affected countries have taken or have threatened retaliatory actions to respond, with measures that include the imposition of retaliatory tariffs on imports of products of U.S. origin, the imposition of export controls on a wide array of products (including rare earth metals and other critical minerals), and other actions. The adoption of retaliatory actions by affected countries has prompted and could prompt the U.S. to further increase its tariff measures, and continued escalation of tariffs and trade measures could result in the outbreak of a trade war.

The trade and tariff policies of the U.S. and other countries are therefore currently fluid and subject to further changes. The U.S. government may also impose new tariffs under new or additional authorities, the amount, scope, and duration of which are currently uncertain. The U.S. government also continues to negotiate trade matters with U.S. trading partners, which may result in further changes with respect to tariffs, export controls, and other related matters. While we will make efforts to mitigate the impacts of escalated tariffs on our business, we may be unsuccessful in fully mitigating these effects, or unable to do so at reasonable cost. Recent increases in the tariffs imposed by the U.S. or other countries may (i) have an uncertain effect on the manufacture of our products, including, without limitation, affecting the availability of our products, or of materials used in our products; (ii) affect the prices at which our products, or materials used in our products, may be obtained; (iii) result in customers delaying orders pending further clarity in the tariff landscape; and (iv) have other effects beyond our position or ability to control. In addition, these tariff actions may also indirectly affect our business through impacts on our customers, vendors, suppliers, and other counterparties, who may themselves be directly affected by some or all of these tariff actions, or indirectly affected by macroeconomic effects resulting from these or other tariff related actions, including potential risks associated with inflation or economic recession. Should the trade relationships between the U.S. and its trading partners remain strained or worsen, our business, liquidity, financial condition, and/or results of operations may therefore be materially and adversely affected.

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 brelgometon. 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 its 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, clinical research organizations ("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 that product candidate 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 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 IEEPA to authorize certain recent tariff actions, and these tariffs were rescinded on February 24, 2026. Though tariff refunds have been issued for some subject entries on which these IEEPA tariffs were collected, exact details regarding the availability, timing, and amount of additional refunds remain uncertain and subject to further litigation and legal, regulatory, and administrative actions. The U.S. government also continues to impose, implement, adjust, and refine additional tariffs under various legal authorities. Further tariffs may also be forthcoming following the initiation and completion of additional Section 232 and 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 candidates 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 drug candidates 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 drug candidates 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 brelgometon) 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 from 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 geopolitical 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 ("USPTO") or 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 issue 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"), 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. For example, we are aware of patents that claim methods of treating cancer that could be alleged to cover our methods of treatment, although our methods of treatment are indicated for a different patient population. While we believe that we would have valid defenses to any claims of patent infringement and that these patents would be difficult to enforce, we cannot be certain that we would prevail in any dispute, and we cannot be certain how an adverse determination would affect our business. 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 a 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 patent 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 patents 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 Unified 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 a 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 the 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 titled "Part I, Item 1C—Cybersecurity", in our Annual Report on Form 10-K filed with the SEC on March 31, 2026, 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 August 2025, CRO Inotiv, Inc. 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 and 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. Increasing use of artificial intelligence by threat actors, including to identify and exploit vulnerabilities and to craft attacks, also may increase the risks of security breaches and incidents and may increase the scope and impact of security breaches and incidents. 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, increases 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 the 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 of 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.

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 £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 or 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, while effective, 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 pre-funded 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 our 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 brelgometon, 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 or BTIG, could result in substantial dilution to our existing stockholders and could cause our stock price to decline.

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 brelgometon. The loss of one or more of our key employees, or our inability to hire additional key personnel when needed, could have a material adverse effect on our business and prospects.

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 2. Unregistered Sales of Equity Securities and Use of Proceeds.

Recent Sales of Unregistered Equity Securities

None.

Item 3. Defaults Upon Senior Securities.

Not applicable.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

Rule 10b5-1 trading arrangement

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 6. 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 of the Company	8-K	001-39503	3.2	January 9, 2026
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.				
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File as its XBRL tags are embedded within the Inline XBRL document				
101.SCH	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents				
104	Cover Page Interactive Data File (formatted in Inline XBRL and included in Exhibit 101)				

* 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.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

LeonaBio, Inc.

Date: November 16, 2026

By: /s/ Mark Litton
Mark Litton
President and Chief Executive Officer (*Principal Executive Officer*)

Date: November 16, 2026

By: /s/ Robert Renninger
Robert Renninger
Chief Financial Officer
(*Principal Financial and Accounting Officer*)

Exhibit 31.1

CERTIFICATION OF THE PRINCIPAL EXECUTIVE OFFICER PURSUANT TO EXCHANGE ACT RULE 13a-14(a)/15d-14(a) AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Mark Litton, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of LeonaBio, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d–15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

    a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

    c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 16, 2026

/s/ Mark Litton

Mark Litton

President and Chief Executive Officer

(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF THE PRINCIPAL FINANCIAL OFFICER PURSUANT TO EXCHANGE ACT RULE 13a-14(a)/15d-14(a) AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Robert Renninger, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of LeonaBio, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d–15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

    a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

    c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 16, 2026

/s/ Robert Renninger

Robert Renninger

Chief Financial Officer

(Principal Financial and Accounting Officer)

Exhibit 32.1

LEONABIO, INC.
CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of LeonaBio, Inc. (the "Company") for the quarter ended September 30, 2026, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Mark Litton, President and Chief Executive Officer *(Principal Executive Officer)* of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d), as applicable, of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 16, 2026

/s/ Mark Litton
Mark Litton
President and Chief Executive Officer
(Principal Executive Officer)

This certification accompanies the Report to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of LeonaBio, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Report), irrespective of any general incorporation language contained in such filing.

Exhibit 32.2

LEONABIO, INC.
CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of LeonaBio, Inc. (the "Company") for the quarter ended September 30, 2026, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Robert Renninger, Chief Financial Officer *(Principal Financial and Accounting Officer)* of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d), as applicable, of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 16, 2026

/s/ Robert Renninger
Robert Renninger
Chief Financial Officer
(Principal Financial and Accounting Officer)

This certification accompanies the Report to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of LeonaBio, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Report), irrespective of any general incorporation language contained in such filing.