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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2025

OR

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

For the transition period from _____ to _____

Commission File Number: 001-38085

Ovid Therapeutics Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware	**46-5270895**
(State or Other Jurisdiction of Incorporation or Organization)	**(I.R.S. Employer Identification Number)**
441 Ninth Avenue, 14th Floor New York, New York	**10001**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (646) 661-7661

(Former name, former address and former fiscal year, if changed since last report)

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, par value $0.001 per share	OVID	The Nasdaq Stock Market LLC

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

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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 the filing requirements for the past 90 days. Yes ☒ No ☐

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

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

Large Accelerated Filer	☐	Accelerated Filer	☐
Non-accelerated Filer	☒	Smaller Reporting Company	☒
Emerging growth company	☐		

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

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

As of November 7, 2025, the registrant had 71,212,353 shares of common stock, $0.001 par value per share, outstanding.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains 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. All statements other than statements of historical fact are "forward-looking statements" for purposes of this Quarterly Report on Form 10-Q. In some cases, you can identify forward-looking statements by terminology such as "aim," "anticipate," "assume," "believe," "contemplate," "continue," "could," "design," "due," "estimate," "expect," "goal," "intend," "may," "objective," "plan," "positioned," "potential," "predict," "project," "should," "target," "will," "would" or the negative or plural of those terms, and similar expressions.

Forward-looking statements include, but are not limited to, statements about:

- our ability to identify additional novel compounds with significant commercial potential to acquire or in-license;
- our ability to successfully acquire or in-license additional drug candidates on reasonable terms;
- the potential use, development and therapeutic potential of the drug candidates in our pipeline;
- our ability to develop medical therapies that deliver preferable safety and tolerability profiles to approved drugs;
- our expectations regarding the duration of our cash runway and the expectation that it will support our operations and development programs;
- our estimates regarding expenses, future revenue including any royalty or milestone payments, capital requirements and needs for additional financing;
- our ability to obtain regulatory approval of our current and future drug candidates;
- our expectations regarding the timing of initiation, completion, and results and data from clinical trials and potential regulatory filings;
- our expectations regarding the potential market size for drug candidates and the rate and degree of market acceptance of such drug candidates;
- our ability to fund our working capital requirements;
- the implementation of our business model and strategic plans for our business and drug candidates, including the programs in our planned pipeline;
- developments or disputes concerning our intellectual property or other proprietary rights;
- our ability to maintain and establish collaborations or obtain additional funding;
- our expectations regarding government and third-party payor coverage and reimbursement;
- our ability to compete in the markets we serve;
- the impact of government laws and regulations;
- developments relating to our competitors and our industry;
- interim topline and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data becomes available and are subject to audit and verification procedures that could result in material changes in the final data;
- the impact of geopolitical tensions (including war or the perception that hostilities may be imminent), adverse global economic conditions, tariffs, the current U.S. government shutdown, funding shortages at governmental and regulatory agencies, supply chain disruptions, terrorism, natural disasters or public health crises on our operations, research and development and clinical trials and potential disruption in the operations and business of third parties and collaborators with whom we conduct business;
- our ability to maintain compliance with Nasdaq listing requirements;
- our ability to continue as a going concern; and
- the factors that may impact our financial results.

Factors that may cause actual results to differ materially from current expectations include, among other things, those set forth in Part II, Item 1A, "Risk Factors," herein and for the reasons described elsewhere in this Quarterly Report

on Form 10-Q. Any forward-looking statement in this Quarterly Report on Form 10-Q reflects our current view with respect to future events and is subject to these and other risks, uncertainties and assumptions relating to our operations, results of operations, industry and future growth. Given these uncertainties, you should not rely on these forward-looking statements as predictions of future events. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity, performance or achievements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

This Quarterly Report on Form 10-Q also contains estimates, projections and other information concerning our industry, our business and the markets for certain drugs and consumer products, including data regarding the estimated size of those markets, their projected growth rates and the incidence of certain medical conditions. Information that is based on estimates, forecasts, projections or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained these industry, business, market and other data from reports, research surveys, studies and similar data prepared by third parties, industry, medical and general publications, government data and similar sources and we have not independently verified the data from third party sources. In some cases, we do not expressly refer to the sources from which these data are derived.

In this Quarterly Report on Form 10-Q, unless otherwise stated or as the context otherwise requires, references to “Ovid,” the “Company,” “we,” “us,” “our” and similar references refer to Ovid Therapeutics Inc. and its wholly owned subsidiaries. This Quarterly Report on Form 10-Q also contains references to our trademarks and to trademarks belonging to other entities. Solely for convenience, trademarks and trade names referred to, including logos, artwork and other visual displays, may appear without the ® or ™ symbols, but such references are not intended to indicate, in any way, that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto. We do not intend our use or display of other companies’ trade names or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other companies.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

OVID THERAPEUTICS INC.
Condensed Consolidated Balance Sheets

(in thousands, except share and per share data)	September 30, 2025	December 31, 2024
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 20,611	$ 26,301
Marketable securities	4,992	26,774
Prepaid expenses and other current assets	3,221	2,865
Total current assets	28,824	55,940
Long-term equity investments	20,908	20,974
Restricted cash	1,931	1,931
Right-of-use asset, net	11,916	12,797
Property and equipment, net	269	433
Other noncurrent assets	—	92
Total assets	$ 63,848	$ 92,167
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,710	$ 3,192
Accrued expenses	2,959	5,994
Current portion, lease liability	1,409	1,336
Deferred revenue	718	—
Total current liabilities	6,796	10,522
Long-term liabilities:		
Lease liability	12,354	13,419
Total liabilities	19,150	23,941
Stockholders' equity:		
Preferred stock, $0.001 par value; 10,000,000 shares authorized; Series A convertible preferred stock, 10,000 shares designated, 1,250 shares issued and outstanding at September 30, 2025 and December 31, 2024	—	—
Common stock, $0.001 par value; 125,000,000 shares authorized; 71,151,981 and 71,009,866 shares issued and outstanding at September 30, 2025 and December 31, 2024, respectively	71	71
Additional paid-in-capital	376,129	372,489
Accumulated other comprehensive loss	(126)	(35)
Accumulated deficit	(331,376)	(304,299)
Total stockholders' equity	44,698	68,226
Total liabilities and stockholders' equity	$ 63,848	$ 92,167

See accompanying notes to these unaudited condensed consolidated financial statements

OVID THERAPEUTICS INC.
Condensed Consolidated Statements of Operations
(unaudited)

(in thousands, except share and per share data)	For The Three Months Ended September 30, 2025	For The Three Months Ended September 30, 2024	For The Nine Months Ended September 30, 2025	For The Nine Months Ended September 30, 2024
Revenue:				
License and other revenue	$ 132	$ 173	$ 6,534	$ 490
Total revenue	132	173	6,534	490
Operating expenses:				
Research and development	5,870	7,855	18,993	30,844
General and administrative	6,785	5,544	17,687	20,809
Total operating expenses	12,655	13,399	36,680	51,653
Loss from operations	(12,523)	(13,226)	(30,146)	(51,163)
Other income (expense), net	365	(780)	3,069	33,983
Loss before provision for income taxes	(12,158)	(14,006)	(27,077)	(17,180)
Provision for income taxes	—	—	—	—
Net loss	$ (12,158)	$ (14,006)	$ (27,077)	$ (17,180)
Net loss per share of Series A preferred stock, basic and diluted	$ (168.01)	$ (193.92)	$ (374.30)	$ (238.21)
Weighted-average Series A preferred stock shares outstanding, basic and diluted	1,250	1,250	1,250	1,250
Net loss per share of common stock, basic and diluted	$ (0.17)	$ (0.20)	$ (0.37)	$ (0.24)
Weighted-average common stock shares outstanding, basic and diluted	71,114,181	70,975,785	71,090,052	70,870,224

See accompanying notes to these unaudited condensed consolidated financial statements

OVID THERAPEUTICS INC.
Condensed Consolidated Statements of Comprehensive Loss
(unaudited)

(in thousands)	For The Three Months Ended September 30, 2025	For The Three Months Ended September 30, 2024	For The Nine Months Ended September 30, 2025	For The Nine Months Ended September 30, 2024
Net loss	$ (12,158)	$ (14,006)	$ (27,077)	$ (17,180)
Other comprehensive (loss) income:				
Cumulative translation adjustment	(60)	—	(92)	—
Unrealized gain on marketable securities	3	48	1	35
Comprehensive loss	$ (12,215)	$ (13,958)	$ (27,168)	$ (17,145)

See accompanying notes to these unaudited condensed consolidated financial statements

OVID THERAPEUTICS INC.

Condensed Consolidated Statements of Stockholders' Equity (unaudited)

(in thousands, except shares)	Series A Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total
	Shares	Amount	Shares	Amount				
Balance, December 31, 2024	1,250	$ —	71,009,866	$ 71	$ 372,489	$ (35)	$ (304,299)	$ 68,226
Issuance of common stock from exercise of stock options and purchases from employee stock purchase plan	—	—	99,648	—	13	—	—	13
Stock-based compensation expense	—	—	—	—	1,284	—	—	1,284
Other comprehensive loss	—	—	—	—	—	(12)	—	(12)
Net loss	—	—	—	—	—	—	(10,235)	(10,235)
Balance, March 31, 2025	1,250	$ —	71,109,514	$ 71	$ 373,786	(47)	$ (314,534)	$ 59,276
Issuance of common stock from exercise of stock options and purchases from employee stock purchase plan	—	—	—	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	1,219	—	—	1,219
Other comprehensive loss	—	—	—	—	—	(22)	—	(22)
Net loss	—	—	—	—	—	—	(4,684)	(4,684)
Balance, June 30, 2025	1,250	$ —	71,109,514	$ 71	$ 375,005	$ (69)	$ (319,218)	$ 55,789
Issuance of common stock from exercise of stock options and purchases from employee stock purchase plan	—	—	42,467	—	15	—	—	15
Stock-based compensation expense	—	—	—	—	1,109	—	—	1,109
Other comprehensive loss	—	—	—	—	—	(57)	—	(57)
Net loss	—	—	—	—	—	—	(12,158)	(12,158)
Balance, September 30, 2025	1,250	$ —	71,151,981	$ 71	$ 376,129	$ (126)	$ (331,376)	$ 44,698

(in thousands, except shares)	Series A Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total
	Shares	Amount	Shares	Amount				
Balance, December 31, 2023	1,250	$ —	70,691,992	$ 71	$ 365,591	$ 1	$ (277,866)	$ 87,797
Issuance of common stock from exercise of stock options and purchases from employee stock purchase plan	—	—	91,969	—	228	—	—	228
Stock-based compensation expense	—	—	—	—	1,968	—	—	1,968
Other comprehensive loss	—	—	—	—	—	(20)	—	(20)
Net loss	—	—	—	—	—	—	(11,694)	(11,694)
Balance, March 31, 2024	1,250	$ —	70,783,961	$ 71	$ 367,787	(19)	$ (289,560)	$ 78,279
Issuance of common stock from exercise of stock options and purchases from employee stock purchase plan	—	—	187,616	—	356	—	—	356
Stock-based compensation expense	—	—	—	—	1,740	—	—	1,740
Other comprehensive income	—	—	—	—	—	7	—	7
Net income	—	—	—	—	—	—	8,521	8,521
Balance, June 30, 2024	1,250	$ —	70,971,577	$ 71	$ 369,883	$ (12)	$ (281,039)	$ 88,903
Issuance of common stock from exercise of stock options and purchases from employee stock purchase plan	—	—	38,289	—	37	—	—	37
Stock-based compensation expense	—	—	—	—	1,307	—	—	1,307
Other comprehensive income	—	—	—	—	—	48	—	48
Net loss	—	—	—	—	—	—	(14,006)	(14,006)
Balance, September 30, 2024	1,250	$ —	71,009,866	$ 71	$ 371,228	$ 36	$ (295,045)	$ 76,290

See accompanying notes to these unaudited condensed consolidated financial statements

OVID THERAPEUTICS INC.
Condensed Consolidated Statements of Cash Flows
(unaudited)

(in thousands)	Nine Months Ended September 30, 2025	Nine Months Ended September 30, 2024
Cash flows from operating activities:		
Net loss	$ (27,077)	$ (17,180)
Adjustments to reconcile net loss to cash used in operating activities:		
Change in fair value of royalty monetization liability	—	(29,028)
Unrealized loss (gain) on equity investments	66	(3,500)
Change in accrued interest and accretion of discount on marketable securities	(433)	(2,301)
Stock-based compensation expense	3,612	5,016
Depreciation and amortization expense	256	453
Noncash operating lease expense	882	815
Change in lease liability	(994)	(926)
Change in operating assets and liabilities:		
Prepaid expenses and other current assets	(356)	649
Accounts payable	(1,480)	(993)
Accrued expenses	(3,120)	1,047
Deferred revenue	718	—
Net cash used in operating activities	(27,926)	(45,948)
Cash flows from investing activities:		
Purchase of marketable securities	(14,792)	(56,533)
Sales/maturities of marketable securities	37,000	90,000
Purchases of property and equipment	—	(49)
Software development and other costs	—	(116)
Net cash provided by investing activities	22,208	33,302
Cash flows from financing activities:		
Proceeds from exercise of options and purchases from employee stock purchase plan	28	622
Net cash provided by financing activities	28	622
Net decrease in cash, cash equivalents and restricted cash	(5,690)	(12,024)
Cash, cash equivalents and restricted cash, at beginning of period	28,232	28,972
Cash, cash equivalents and restricted cash, at end of period	$ 22,542	$ 16,948

See accompanying notes to these unaudited condensed consolidated financial statements

OVID THERAPEUTICS INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(unaudited)

NOTE 1 – NATURE OF OPERATIONS

Ovid Therapeutics Inc. (the "Company") was incorporated under the laws of the state of Delaware and commenced operations on April 1, 2014 and maintains its principal executive office in New York, New York. The Company is a biopharmaceutical company that is dedicated to developing small molecule medicines for brain conditions with significant unmet need.

Liquidity and Going Concern

Since its inception, the Company has devoted substantially all of its efforts to business development, research and development, recruiting management and technical staff, and raising capital, and has financed its operations through the issuance of convertible preferred stock, common stock, other equity instruments, the sale and/or licensing of certain assets and the licensing of certain intellectual property. As of September 30, 2025, the Company had $25.6 million in cash, cash equivalents and marketable securities. Since its founding, the Company has generated its revenue primarily from the Company's royalty, license and termination agreement ("RLT Agreement") with Takeda Pharmaceutical Company Limited ("Takeda"). For most periods, the Company has incurred recurring losses, has experienced negative operating cash flows and has required significant cash resources to execute its business plans, which the Company expects will continue for the foreseeable future. The Company had an accumulated deficit of $331.4 million as of September 30, 2025, working capital of $22.0 million and net cash used in operating activities of $27.9 million for the nine months ended September 30, 2025.

The Company recorded net losses of $12.2 million and $27.1 million, respectively, during the three and nine month periods ended September 30, 2025, and expects to incur losses for at least the next several years. The Company is highly dependent on its ability to find additional sources of funding through either equity offerings, debt financings, collaborations, strategic alliances, licensing agreements or a combination of any such transactions.

On October 6, 2025, the Company completed a private placement (the "Private Placement") of convertible preferred stock and warrants to purchase shares of the Company's common stock, resulting in net proceeds of $75.7 million. For a description of the Private Placement, see Note 15 — Subsequent Events.

As of the report date, the Company believes its existing cash, cash equivalents and marketable securities, including the proceeds received from the Private Placement, are sufficient to fund its operating and capital expenditure requirements for a period greater than 12 months from the date these unaudited interim condensed consolidated financial statements were issued. If the Company's available cash, cash equivalents and marketable securities are insufficient to satisfy its liquidity requirements, the Company may need to raise additional capital to fund its operations. No assurance can be given as to whether additional needed financing will be available on terms acceptable to the Company, if at all. If sufficient funds on acceptable terms are not available when needed, the Company may be required to suspend or forego certain planned activities. Failure to manage discretionary spending or raise additional financing, as needed, would adversely impact the Company's ability to achieve its intended business objectives and have an adverse effect on its results of operations and future prospects.

The Company is subject to risks and uncertainties and other challenges specific to its business and its ability to execute on its strategy, as well as risks and uncertainties common to early-stage companies in the pharmaceutical industry with regulated product development and commercial operations, including, without limitation, risks and uncertainties associated with: the ability to secure additional capital to fund operations; delays or problems in the supply of the Company's product candidates; loss of single source suppliers or failure to comply with manufacturing regulations; technological competition; identifying, acquiring or in-licensing additional products or product candidates; pharmaceutical product development and the inherent uncertainty of clinical success; the challenges of protecting and enhancing intellectual property rights; dependence on key personnel; complying with applicable regulatory requirements; obtaining regulatory approval of any of the Company's potential product candidates, among others.

Nasdaq Compliance

On February 10, 2025, the Company received a notification letter from the Listing Qualifications Department of the Nasdaq Stock Market LLC notifying the Company that the average closing bid price of the Company's shares of common stock was below the closing bid price of $1.00 per share during the last 31 consecutive trading days. The Company had an initial period of 180 calendar days, or until August 11, 2025, to regain compliance with the minimum bid price requirement. On August 12, 2025, the Company received approval from the Listing Qualifications Department of Nasdaq to transfer the listing of the Company's common stock from the Nasdaq Global Select Market to the Nasdaq Capital Market (the "Approval"). In connection with the Approval, the Company was granted an additional 180-day grace

period, or until February 9, 2026, to regain compliance with the minimum bid price requirement. On September 11, 2025, the Company received formal notification from Nasdaq that it had regained compliance with the minimum bid price requirement.

NOTE 2 – SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

(A) Unaudited Interim Condensed Consolidated Financial Statements

The interim condensed consolidated balance sheet at September 30, 2025 and the condensed consolidated statements of operations, comprehensive loss, cash flows, and stockholders' equity for the three and nine months ended September 30, 2025 and 2024 are unaudited. The accompanying unaudited condensed consolidated financial statements have been prepared in accordance with GAAP and follow the requirements of the SEC for interim reporting. As permitted under those rules, certain notes or other financial information that are normally required by GAAP are condensed or omitted. These condensed consolidated financial statements have been prepared on the same basis as the Company's annual financial statements and, in the opinion of management, reflect all adjustments, consisting only of normal recurring adjustments that are necessary for a fair statement of its financial information. The results of operations for the three and nine months ended September 30, 2025 and 2024 are not necessarily indicative of the results to be expected for the year ending December 31, 2025 or for any other future annual or interim period. The balance sheet as of December 31, 2024 included herein was derived from the audited financial statements as of that date. These interim condensed consolidated financial statements should be read in conjunction with the Company's audited financial statements as of and for the year ended December 31, 2024 included in the Company's Annual Report on Form 10-K.

(B) Basis of Presentation and Consolidation

The accompanying interim condensed consolidated financial statements have been prepared in conformity with GAAP and include the accounts of Ovid Therapeutics Inc. and its wholly owned subsidiaries, Ovid Therapeutics Australia Pty Ltd and Ovid Therapeutics Hong Kong Ltd. All intercompany transactions and balances have been eliminated in consolidation.

(C) Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of income and expenses during the reporting period. Actual results could differ materially from those estimates.

(D) Marketable Securities

Marketable securities consist of investments in U.S. treasury instruments which are considered available-for-sale securities. The Company classifies its marketable securities with maturities of less than one year from the balance sheet date as current assets on its condensed consolidated balance sheets. The Company classifies its marketable securities with original maturities of less than three months as cash equivalents on its condensed consolidated balance sheets. Unrealized gains and losses on these securities that are determined to be temporary are reported as a separate component of accumulated other comprehensive (loss) income in stockholders' equity.

(E) Restricted Cash

The Company classifies as restricted cash all cash pledged as collateral to secure long-term obligations and all cash whose use is otherwise limited by contractual provisions. Amounts are reported as noncurrent unless restrictions are expected to be released in the next 12 months.

(F) Long-Term Equity Investments

Long-term equity investments consist of equity investments in the preferred shares of Gensaic, Inc., formerly M13 Therapeutics, Inc. ("Gensaic"), and Graviton Bioscience Corporation ("Graviton"), both privately held corporations. The preferred shares are not considered in-substance common stock, and the investments are accounted for at cost, with adjustments for observable changes in prices or impairments, and are classified within long-term equity investments on the condensed consolidated balance sheets with adjustments recognized in other income (expense), net on the condensed consolidated statements of operations. The Company has determined that these equity investments do not have a readily determinable fair value and elected the measurement alternative. Therefore, the carrying amount of the equity investments will be adjusted to fair value at the time of the next observable price change for the identical or similar investment of the same issuer or when an impairment is recognized. Each reporting period, the Company performs a qualitative assessment to evaluate whether the investments are impaired. The assessment includes a review of recent operating results and trends, recent sales/acquisitions of the investees' securities, and other publicly available data. If an investment is determined to be

impaired, the Company will then write it down to its estimated fair value. As of September 30, 2025 and December 31, 2024, the equity investment in Gensaic had a carrying value of $5.1 million. As of September 30, 2025 and December 31, 2024, the equity investment in Graviton had a carrying value of $15.8 million. The initial investment in Graviton was $10.0 million, and cumulative measurement adjustments based on observable price increases totaling $5.8 million have been recognized, recorded in other income (expense), net within the statement of operations.

Long-term equity investments also consist of an equity investment in the common shares of Marinus Pharmaceuticals, Inc. ("Marinus") that were received as noncash consideration via the terms of a licensing agreement executed between the two companies effective March 2022. The equity shares are marked-to-market at each reporting date with changes in the fair value being reflected in the carrying value of the investment on the Company's condensed consolidated balance sheets and other income (expense), net on the Company's condensed consolidated statements of operations. As of December 31, 2024, the equity investment in Marinus had a carrying value of $0.1 million. In February 2025, Immedica, S.A. ("Immedica") closed a cash purchase of Marinus, resulting in the sale of the Company's equity position in Marinus to Immedica for $70,000.

(G) Fair Value of Financial Instruments

Financial Accounting Standards Board ("FASB") guidance specifies a hierarchy of valuation techniques based on whether the inputs to those valuation techniques are observable or unobservable. Observable inputs reflect market data obtained from independent sources, while unobservable inputs reflect market assumptions. The hierarchy gives the highest priority to unadjusted quoted prices in active markets for identical assets or liabilities (Level 1 measurement) and the lowest priority to unobservable inputs (Level 3 measurement).

The three levels of the fair value hierarchy are as follows:

- Level 1—Unadjusted quoted prices in active markets for identical assets or liabilities that the reporting entity has the ability to access at the measurement date. Level 1 primarily consists of financial instruments whose value is based on quoted market prices such as exchange-traded instruments and listed equities. The Company's Level 1 assets consisted of investments in a U.S. treasury money market fund of $20.5 million as of September 30, 2025, and U.S. treasury money market fund and equity securities totaling $25.8 million as of December 31, 2024.
- Level 2—Inputs other than quoted prices included within Level 1 that are observable for the asset or liability, either directly or indirectly (e.g., quoted prices of similar assets or liabilities in active markets, or quoted prices for identical or similar assets or liabilities in markets that are not active). Level 2 includes financial instruments that are valued using models or other valuation methodologies. The Company's Level 2 assets consisted of U.S. treasury bills, totaling $5.0 million as of September 30, 2025 and $26.8 million as of December 31, 2024.
- Level 3—Unobservable inputs for the asset or liability. Financial instruments are considered Level 3 when their fair values are determined using pricing models, discounted cash flows or similar techniques and at least one significant model assumption or input is unobservable. There were no Level 3 assets or liabilities as of September 30, 2025 or December 31, 2024.

The carrying amounts reported in the balance sheets for cash and cash equivalents, other current assets, accounts payable and accrued expenses approximate their fair values based on the short-term maturity of these instruments.

(H) Leases

The Company determines if an arrangement is a lease at inception and recognizes the lease in accordance with FASB Accounting Standards Codification ("ASC") 842. Operating leases are included in right-of-use ("ROU") assets, current liabilities, and long-term lease liability in the Company's condensed consolidated balance sheets. ROU assets represent the right to use an underlying asset for the lease term and lease liabilities represent the Company's obligation to make lease payments arising from the lease. Operating lease ROU assets and liabilities are recognized at the lease commencement date based on the present value of the lease payments over the lease term. The Company determines the portion of the lease liability that is current as the difference between the calculated lease liability at the end of the current period and the lease liability that is projected 12 months from the current period.

(I) Property and Equipment

Property and equipment are stated at cost and depreciated over their estimated useful lives of three years using the straight-line method. Repair and maintenance costs are expensed. The Company reviews the recoverability of all long-lived assets, including the related useful life, whenever events or changes in circumstances indicate that the carrying amount of a long-lived asset might not be recoverable.

(J) Research and Development Expenses

The Company expenses the cost of research and development as incurred. Research and development expenses are comprised of costs incurred in performing research and development activities, including clinical trial costs, manufacturing costs for both clinical and preclinical materials as well as contracted services, license fees, and other external costs. Research and development expenses also include the cost of licensing agreements acquired from third parties. Nonrefundable advance payments for goods and services that will be used in future research and development activities are expensed when the activity is performed or when the goods have been received in accordance with ASC 730, Research and Development.

(K) Royalty Monetization Liability

The Company accounted for its sale to Ligand Pharmaceuticals Incorporated ("Ligand") of a 13% share of royalties and milestones owed to the Company related to the potential approval and commercialization of soticlestat ("Ligand Agreement") in accordance with ASC 470, Debt, classifying the proceeds received from the sale to Ligand as debt as the Company determined that it had significant continuing involvement in the generation of the cash flows to Ligand. The Company further elected to account for the debt at fair value with changes in the fair value of the debt classified as other income (expense) in the consolidated statements of operations. In June 2024, Takeda issued a press release indicating the soticlestat trials missed their primary endpoints and noted that while Takeda would discuss the program with the FDA, Takeda fully impaired the asset representing soticlestat. In 2024, the Company recorded a gain of $30.0 million, reducing the fair value of the Ligand Agreement debt to zero as a result of the improbability that the program would be further developed into a commercial product by Takeda or another party. In January 2025, Takeda announced the discontinuation of the program.

(L) Stock-Based Compensation

The Company accounts for its stock-based compensation in accordance with ASC 718, Compensation—Stock Compensation, which establishes accounting for stock-based awards granted for services and requires companies to expense the estimated fair value of these awards over the requisite service period. The Company estimates the fair value of all awards granted using the Black-Scholes valuation model. Key inputs and assumptions include the expected term of the option, stock price volatility, risk-free interest rate, dividend yield, stock price and exercise price. Many of the assumptions require judgment and any changes could have an impact in the determination of stock-based compensation expense. The Company elected an accounting policy to record forfeitures as they occur. The Company recognizes stock-based compensation expense based on the fair value of the award on the date of the grant. The compensation expense is recognized over the vesting period under the straight-line method. The Company aggregates employee and nonemployee awards for certain disclosures since nonemployee awards are not material.

(M) Income Taxes

The Company accounts for income taxes under the asset and liability method, which requires deferred tax assets and liabilities to be recognized for the estimated future tax consequences attributable to differences between financial statement carrying amounts and respective tax bases of existing assets and liabilities, as well as for net operating loss carryforwards and research and development credits. Valuation allowances are provided if it is more likely than not that some portion or all of the deferred tax assets will not be realized. The impact of a change in the tax laws is recorded in the period in which the law is enacted.

(N) Net (Loss) Income per Share

The rights and preferences of the non-voting Series A convertible preferred stock ("Series A Preferred Stock") are negligible relative to common stock, therefore the Series A Preferred Stock is treated as in-substance common stock on an as-converted basis when allocating net (loss) income to actual and in-substance shares of common stock. The Company applies the two-class method to allocate earnings between common stock, Series A Preferred Stock as well as other securities deemed in-substance common stock and participating securities, if any.

Net (loss) income per share of Series A Preferred Stock is determined by dividing net (loss) income attributable to Series A preferred stockholders on an as-converted basis by the basic and diluted weighted-average shares of Series A Preferred Stock outstanding during the period. Net (loss) income per share of common stock is determined by dividing net income attributable to common stockholders by the basic and diluted weighted-average shares of common stock outstanding during the period.

Net (loss) income per diluted share attributable to common stockholders adjusts the basic earnings per share attributable to common stockholders and the weighted-average number of shares of common stock outstanding for the potential dilutive impact of stock options and restricted stock units using the treasury-stock method and the potential impact of any preferred stock using the if-converted method. Net (loss) income per diluted share attributable to common

stockholders omits the inclusion of stock options, common stock issuable upon conversion of Series A Preferred Stock, and unvested restricted stock units as these securities would be anti-dilutive.

(O) Retirement Plan

The Company maintains a 401(k) retirement plan for its employees that is intended to qualify under Sections 401(a) and 501(a) of the U.S. Internal Revenue Code of 1986, as amended (the "Code"). The Company provides all active employees with a 100% matching contribution equal to 3% of an employee's eligible deferred compensation and a 50% matching contribution on employee contributions that are between 3% and 5% of an employee's eligible deferred compensation. These safe harbor contributions vest immediately. For the three months ended September 30, 2025 and 2024, the Company contributed $41,000 and $54,000, respectively. For the nine months ended September 30, 2025 and 2024, the Company contributed $162,000 and $254,000, respectively.

(P) Revenue Recognition

Under ASC 606, Revenue from Contracts with Customers, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration that the entity expects to receive in exchange for those goods or services. In applying ASC 606, the Company performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the promises and performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) it satisfies the performance obligations. The Company only applies the five-step model to contracts when it is probable that it will collect the consideration to which it is entitled in exchange for the goods or services the Company transfers to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, the Company assesses the goods or services promised within each contract, determines those that are performance obligations and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

Prior to recognizing revenue, the Company makes estimates of the transaction price, including variable consideration that is subject to a constraint. Amounts of variable consideration are included in the transaction price to the extent that it is probable that a significant reversal in the amount of cumulative revenue recognized will not occur and when the uncertainty associated with the variable consideration is subsequently resolved.

If there are multiple distinct performance obligations, the Company allocates the transaction price to each distinct performance obligation based on its relative standalone selling price. The standalone selling price is generally determined using expected cost and comparable transactions. Revenue for performance obligations recognized over time is recognized by measuring the progress toward complete satisfaction of the performance obligations using an input measure.

Non-refundable upfront fees allocated to licenses that are not contingent on any future performance and require no consequential continuing involvement by the Company, are recognized as revenue when the license term commences and the licensed data, technology or product is delivered. The Company defers recognition of upfront license fees until the performance obligations are satisfied.

(Q) Segment Reporting

Under Accounting Standards Update ("ASU") 2023-07, Segment Reporting (Topic 280): "Improvements to Reportable Segment Disclosures" ("ASU 2023-07"), the Company discloses significant segment expenses regularly provided to the chief operating decision maker ("CODM") and discloses the title and position of the CODM.

(R) Recent Accounting Pronouncements

The Company has reviewed recently issued accounting standards and plans to adopt those that are applicable. The Company does not expect the adoption of those standards to have a material impact on its financial position, results of operations or cash flows.

In December 2023, the FASB issued ASU 2023-09, Improvements to Income Tax Disclosures, ("ASU 2023-09"), which is effective for annual periods beginning after December 15, 2024. ASU 2023-09 intends to enhance the transparency as well as usefulness of income tax disclosures, primarily related to the rate reconciliation and income taxes paid. The Company is currently evaluating the impact that the adoption of ASU 2023-09 will have on its related disclosures.

In November 2024, the FASB issued ASU 2024-03, Income Statement – Reporting Comprehensive Income – Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses ("ASU 2024-03"), which requires the disaggregation of certain expense captions into specified categories in disclosures within the notes to the

consolidated financial statements to provide enhanced transparency into the expense captions presented on the face of the statements of income and comprehensive income. ASU 2024-03 is effective for annual reporting periods beginning after December 15, 2026, with early adoption permitted, and may be applied either prospectively or retrospectively to financial statements issued for reporting periods after the effective date of ASU 2024-03 or retrospectively to any or all prior periods presented in the financial statements. The Company is currently evaluating the impact that the adoption of ASU 2024-03 will have on its related disclosures.

The Company adopts new pronouncements relating to GAAP applicable to the Company as they are issued, and based upon the effective dates included in the pronouncements. Management does not believe that any recently issued, but not yet effective accounting standards, if currently adopted, would have a material effect on the accompanying consolidated financial statements.

NOTE 3 – CASH, CASH EQUIVALENTS AND MARKETABLE SECURITIES

The following tables summarize the fair value of cash, cash equivalents and marketable securities as well as gross unrealized holding gains and losses as of September 30, 2025 and December 31, 2024:

	September 30, 2025			
(in thousands)	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Fair Value
Cash	$ 127	$ —	$ —	$ 127
Cash equivalents	20,484	—	—	20,484
Marketable securities	4,991	1	—	4,992
Total cash, cash equivalents and marketable securities	$ 25,602	$ 1	$ —	$ 25,603

	December 31, 2024			
(in thousands)	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Fair Value
Cash	$ 522	$ —	$ —	$ 522
Cash equivalents	25,779	—	—	25,779
Marketable securities	26,767	7	—	26,774
Total cash, cash equivalents and marketable securities	$ 53,068	$ 7	$ —	$ 53,075

The Company did not hold any securities that were in an unrealized loss position for more than 12 months as of September 30, 2025 and December 31, 2024.

There were no material realized gains or losses on available-for-sale securities during the three and nine months ended September 30, 2025 and 2024.

NOTE 4 – PROPERTY AND EQUIPMENT AND INTANGIBLE ASSETS

Property and equipment is summarized as follows:

(in thousands)	September 30, 2025	December 31, 2024
Furniture and equipment	$ 1,534	$ 1,534
Leasehold improvements	306	306
Less accumulated depreciation	(1,571)	(1,407)
Total property and equipment, net	$ 269	$ 433

Depreciation expense was $17,000 and $105,000 for the three months ended September 30, 2025 and 2024, respectively. Depreciation expense was $165,000 and $309,000 for the nine months ended September 30, 2025 and 2024, respectively.

Intangible assets, net of accumulated amortization, were $0 and $219,000 as of September 30, 2025 and December 31, 2024, respectively, and are included in other noncurrent assets on the condensed consolidated balance sheets. Amortization expense was $0 and $64,000 for the three months ended September 30, 2025 and 2024, respectively. Amortization expense was $91,000 and $144,000 for the nine months ended September 30, 2025 and 2024, respectively.

NOTE 5 – LEASES

In September 2021, the Company entered into a 10-year lease agreement for its corporate headquarters with a term commencing March 10, 2022, for approximately 19,000 square feet of office space at Hudson Commons in New York, New York. The lease provides for monthly rental payments over the lease term. The base rent under the lease is currently $2.3 million per year. Rent payments commenced in January 2023, and continue for 10 years following the rent commencement date. The Company issued an irrevocable letter of credit in the amount of $1.9 million in association with the execution of the lease agreement; the letter of credit is characterized as restricted cash on the Company's condensed consolidated balance sheets.

The Hudson Commons lease has a remaining lease term of approximately seven years and includes a single renewal option for an additional five years. The Company did not include the renewal option in the lease term when calculating the lease liability as the Company is not reasonably certain that it will exercise the renewal option. The present value of the lease payments was calculated using an incremental borrowing rate of 7.02%. Lease expense is included in general and administrative and research and development expenses in the condensed consolidated statements of operations.

ROU asset and lease liabilities related to the Company's operating lease are as follows:

(in thousands)	September 30, 2025	December 31, 2024
ROU asset, net	$ 11,916	$ 12,797
Current lease liability	1,409	1,336
Long-term lease liability	12,354	13,419

The components of operating lease cost for the three and nine months ended September 30, 2025 were as follows:

(in thousands)	Three Months Ended September 30, 2025	Nine Months Ended September 30, 2025
Operating lease cost	$ 542	$ 1,625
Variable lease cost	—	—
Short-term lease cost	—	—

Future minimum commitments under the non-cancelable operating lease are as follows:

(in thousands)	
2025	$ 579
2026	2,316
2027	2,316
2028	2,469
2029	2,469
Thereafter	7,408
	$ 17,557

NOTE 6 – ACCRUED EXPENSES

Accrued expenses consist of the following:

(in thousands)	September 30, 2025	December 31, 2024
Payroll and bonus accrual	$ 1,849	$ 2,959
Research and development accrual	346	2,779
Professional fees accrual	535	168
Other	229	88
Total	$ 2,959	$ 5,994

NOTE 7 – STOCKHOLDERS' EQUITY

The Company's capital structure consists of common stock and preferred stock. Pursuant to the Company's amended and restated certificate of incorporation, as amended, the Company is authorized to issue up to 125,000,000 shares of common stock and 10,000,000 shares of preferred stock. The Company has designated 10,000 shares of preferred stock as Series A Preferred Stock, of which 1,250 are issued and outstanding as of September 30, 2025.

The holders of common stock are entitled to one vote for each share held. The holders of common stock have no preemptive or other subscription rights, and there are no redemption or sinking fund provisions with respect to such shares. Subject to preferences that may apply to any outstanding series of preferred stock, holders of the common stock are entitled to receive ratably any dividends declared on a non-cumulative basis. The common stock is subordinate to all series of preferred stock with respect to rights upon liquidation, winding up and dissolution of the Company. The holders of common stock are entitled to liquidation proceeds after all liquidation preferences for the preferred stock are satisfied.

There were 1,250 shares of Series A Preferred Stock outstanding as of September 30, 2025 and December 31, 2024. Each share of Series A Preferred Stock is convertible into 1,000 shares of common stock at any time at the holder's option. However, the holder will be prohibited, subject to certain exceptions, from converting shares of Series A Preferred Stock into shares of common stock if, as a result of such conversion, the holder, together with its affiliates, would own more than, at the written election of the holder, either 9.99% or 14.99% of the total number of shares of common stock then issued and outstanding, which percentage may be changed at the holder's election to any other number less than or equal to 19.99% upon 61 days' notice to the Company; provided, however, that effective 61 days after delivery of such notice, such beneficial ownership limitations shall not be applicable to any holder that beneficially owns either 10.0% or 15.0%, as applicable based on the holder's initial written election noted above, of the total number of shares of common stock issued and outstanding immediately prior to delivery of such notice. In the event of a liquidation, dissolution, or winding up of the Company, holders of Series A Preferred Stock will receive a payment equal to $0.001 per share of Series A Preferred Stock before any proceeds are distributed to the holders of common stock. Holders of Series A Preferred Stock are entitled to receive dividends paid to holders of common stock at an equal rate, in the same form, and in the same manner on an as-if-converted basis.

Dividends

Through September 30, 2025, the Company has not declared any dividends. No dividends on the common stock shall be declared and paid unless dividends on the preferred stock have been declared and paid.

NOTE 8 – STOCK-BASED COMPENSATION

The Company's Board of Directors adopted, and the Company's stockholders approved, the 2017 Equity Incentive Plan ("2017 Plan"), which became effective on May 4, 2017. The initial reserve of shares of common stock issuable under the 2017 Plan was 3,052,059 shares. The 2017 Plan provides for the grant of incentive stock options, non-statutory stock options, restricted stock awards, restricted stock unit awards, stock appreciation rights, performance-based stock awards, and other forms of stock-based awards. Additionally, the 2017 Plan provides for the grant of performance cash awards. The Company's employees, officers, directors, consultants and advisors are eligible to receive awards under the 2017 Plan. Following the adoption of the 2017 Plan, no further awards will be granted under the Company's prior plan. Pursuant to the terms of the 2017 Plan, on each January 1st, the plan limit shall be increased by the lesser of (x) 5% of the number of shares of common stock outstanding as of the immediately preceding December 31 and (y) such lesser number as the Board of Directors may determine at its discretion. On January 1, 2025, no additional shares were reserved for

issuance under the 2017 Plan. As of September 30, 2025, there were 4,002,969 shares of the Company's common stock reserved and available for issuance under the 2017 Plan.

The Company's Board of Directors adopted, and the Company's stockholders approved, the 2017 Employee Stock Purchase Plan ("2017 ESPP"), which became effective on May 4, 2017. The initial reserve of shares of common stock issuable under the 2017 ESPP was 279,069 shares. The 2017 ESPP allows employees to purchase common stock of the Company at a 15% discount to the market price on designated semi-annual purchase dates. During the three months ended September 30, 2025 and 2024, 42,467 and 38,289 shares were purchased under the 2017 ESPP, respectively, and the Company recorded expense of $3,000 and $6,000, respectively. During the nine months ended September 30, 2025 and 2024, employees purchased 76,976 and 69,850 shares under the 2017 ESPP, respectively, and the Company recorded expense of $29,000 and $40,000, respectively. The number of shares of common stock reserved for issuance under the 2017 ESPP automatically increases on January 1 of each year, beginning on January 1, 2018 and continuing through and including January 1, 2027, by the lesser of (i) 1% of the total number of shares of the Company's common stock outstanding on December 31 of the preceding calendar year, (ii) 550,000 shares or (iii) such lesser number of shares determined by the Board of Directors. The Board of Directors acted prior to each of January 1, 2025 and January 1, 2024 to provide that there be no increase in the number of shares reserved for issuance under the 2017 ESPP on either such date. As of September 30, 2025, there were 206,020 shares of the Company's common stock reserved and available for issuance under the 2017 ESPP.

The Company's Board of Directors adopted, and the Company's stockholders approved, the 2014 Equity Incentive Plan ("2014 Plan"), which authorized the Company to grant shares of common stock in the form of incentive stock options, non-statutory stock options, stock appreciation rights, restricted stock and restricted stock units. The 2014 Plan was terminated as to future awards in May 2017, although it continues to govern the terms of options that remain outstanding under the 2014 Plan. No additional stock awards will be granted under the 2014 Plan, and all outstanding stock awards granted under the 2014 Plan that are repurchased, forfeited, expire or are cancelled will become available for grant under the 2017 Plan in accordance with its terms. As of September 30, 2025, options to purchase 898,483 shares of common stock were outstanding under the 2014 Plan.

Unless specified otherwise in an individual option agreement, stock options granted under the 2014 Plan and the 2017 Plan generally have a ten-year term and a four-year graded vesting period. The vesting requirement is generally conditioned upon the grantee's continued service with the Company during the vesting period. Once vested, all options granted are exercisable from the date of grant until they expire. The option grants are non-transferable. Vested options generally remain exercisable for 90 days under the 2017 Plan and 30 days under the 2014 Plan subsequent to the termination of the option holder's service with the Company. In the event of the option holder's death or disability while employed by or providing service to the Company, the exercisable period extends to 18 months or 12 months, respectively, under the 2017 Plan and six months under the 2014 Plan.

The fair value of options granted during the three and nine months ended September 30, 2025 and 2024 was estimated using the Black-Scholes option valuation model. The inputs for the Black-Scholes option valuation model require assumptions that are detailed in the table below. The risk-free interest rates are based on the rate for U.S. treasury securities at the date of grant with maturity dates approximately equal to the expected life at the grant date. The expected life is based on the simplified method in accordance with the SEC Staff Accounting Bulletin No. Topic 14D.

The Company granted no stock options and 1,747,660 stock options during the three months ended September 30, 2025 and 2024, respectively, and 3,675,900 and 4,499,810 during the nine months ended September 30, 2025 and 2024, respectively. There were 6,746,927 and 6,166,643 unvested options outstanding as of September 30, 2025 and 2024, respectively. Total expense recognized related to the stock options for the three months ended September 30, 2025 and 2024 was $1.1 million and $1.3 million, respectively. Total expense recognized related to the stock options for the nine months ended September 30, 2025 and 2024 was $3.6 million and $5.0 million, respectively. During the three and nine months ended September 30, 2025 and 2024, the Company had no outstanding performance-based option awards and did not record any related expense in those periods.

The Company did not grant any restricted stock units during the three months ended September 30, 2025 and 2024. The Company granted 249,201 and 348,575 restricted stock units during the nine months ended September 30, 2025 and 2024, respectively. At September 30, 2025, there were 341,955 restricted stock units outstanding.

The Company's stock-based compensation expense was recognized in operating expenses as follows:

	Three Months Ended		Nine Months Ended	
(in thousands)	September 30, 2025	September 30, 2024	September 30, 2025	September 30, 2024
Research and development	$ 331	$ 241	$ 1,108	$ 1,307
General and administrative	777	1,067	2,504	3,709
Total	$ 1,108	$ 1,308	$ 3,612	$ 5,016

	Three Months Ended		Nine Months Ended	
(in thousands)	September 30, 2025	September 30, 2024	September 30, 2025	September 30, 2024
Stock options and restricted stock units	$ 1,105	$ 1,301	$ 3,583	$ 4,976
Employee Stock Purchase Plan	3	7	29	40
Total	$ 1,108	$ 1,308	$ 3,612	$ 5,016

The fair value of options granted during the three and nine months ended September 30, 2025 and 2024 was estimated utilizing the following assumptions:

	Three Months Ended		Nine Months Ended	
	September 30, 2025	September 30, 2024	September 30, 2025	September 30, 2024
	Weighted Average	Weighted Average	Weighted Average	Weighted Average
Volatility	N/A	98.60 %	96.76 %	87.13 %
Expected term in years	N/A	5.72	6.01	5.92
Dividend rate	N/A	— %	— %	— %
Risk-free interest rate	N/A	4.00 %	4.35 %	4.20 %
Fair value of option on grant date	N/A	$ 0.82	$ 0.47	$ 2.11

The following table summarizes the number of options outstanding and the weighted average exercise price:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life in Years	Aggregate Intrinsic Value
Options outstanding December 31, 2024	15,341,356	$ 3.49	5.87	$ —
Vested and exercisable at December 31, 2024	9,652,613	4.07	5.87	$ —
Granted	3,675,900	0.55		
Exercised	—	—		
Forfeited or expired	(918,313)	5.32		
Options outstanding September 30, 2025	18,401,818	$ 2.77	6.27	$ 3,280,083
Vested and exercisable at September 30, 2025	11,654,891	$ 3.56	4.85	$ 258

At September 30, 2025, there was $9.5 million of unrecognized stock–based compensation expense related to stock option grants, which is expected to be recognized over a remaining average vesting period of 2.41 years.

NOTE 9 – INCOME TAXES

The Company's interim income tax provision consists of U.S. federal and state income taxes based on the estimated annual effective tax rate that the Company expects for the full year together with the tax effect of discrete items. Each quarter the Company updates its estimate of the annual effective tax rate and records cumulative adjustments as necessary. As of September 30, 2025, the Company was in a pre-tax loss position and is anticipated to remain so throughout the year. For the three and nine months ended September 30, 2025 and 2024, the Company did not record any tax benefit or expense.

In assessing the realizability of deferred tax assets, management evaluates whether it is more likely than not that some portion or all of the deferred tax assets will be realized. The ultimate realization of deferred tax assets is dependent upon the generation of future taxable income in those periods in which temporary differences become deductible and/or net operating losses can be utilized. Management assesses all positive and negative evidence when determining the amount of the net deferred tax assets that are more likely than not to be realized. This evidence includes, but is not limited to, prior earnings history, scheduled reversal of taxable temporary differences, tax planning strategies and projected future taxable income. Significant weight is given to positive and negative evidence that is objectively verifiable. Based on these factors, including cumulative losses in recent years, the Company continues to maintain a full valuation allowance against its net deferred tax assets as of September 30, 2025.

On July 4, 2025, the One Big Beautiful Bill Act ("OBBBA") was enacted in the United States. The OBBBA includes significant changes, such as the permanent extension of certain expiring provisions of the Tax Cuts and Jobs Act, modifications to certain aspects of the international tax framework and the restoration of favorable tax treatment for certain business expense provisions. The legislation has multiple effective dates, with certain provisions effective in 2025 and others implemented through 2027. The OBBBA did not have a material impact on the Company's interim condensed consolidated financial statements for the three-month period ended September 30, 2025, and the Company does not expect the OBBBA to have a material impact on the Company's provision for income taxes or net income (loss) in future periods.

NOTE 10 – COMMITMENTS AND CONTINGENCIES

License Agreements

Northwestern University License Agreement

In December 2016, the Company entered into a license agreement ("Northwestern Agreement") with Northwestern University ("Northwestern"), pursuant to which Northwestern granted the Company an exclusive, worldwide license to patent rights of certain inventions ("Northwestern Patent Rights") which relate to a specific compound and related methods of use for such compound, along with certain know-how related to the practice of the inventions claimed in the Northwestern Patent Rights. The Company is developing OV329 under this agreement.

Under the Northwestern Agreement, the Company was granted exclusive rights to research, develop, manufacture and commercialize products utilizing the Northwestern Patent Rights for all uses. The Company has agreed that it will not use the Northwestern Patent Rights to develop any products for the treatment of cancer, but Northwestern may not grant rights in the technology to others for use in cancer. The Company also has an option, exercisable during the term of the agreement to an exclusive license under certain intellectual property rights covering novel compounds with the same or similar mechanism of action as the primary compound that is the subject of the license agreement. Northwestern has retained the right, on behalf of itself and other non-profit institutions, to use the Northwestern Patent Rights and practice the inventions claimed therein for educational and research purposes and to publish information about the inventions covered by the Northwestern Patent Rights.

Upon entry into the Northwestern Agreement, the Company paid an upfront non-creditable one-time license issuance fee of $75,000 and is required to pay an annual license maintenance fee of $20,000, which will be creditable against any royalties payable to Northwestern following first commercial sale of licensed products under the agreement. The Company is responsible for all ongoing costs of filing, prosecuting and maintaining the Northwestern Patent Rights, but also has the right to control such activities using its own patent counsel. In consideration for the rights granted to the Company under the Northwestern Agreement, the Company is required to pay to Northwestern up to an aggregate of $5.3 million upon the achievement of certain development and regulatory milestones for the first product covered by the Northwestern Patent Rights, and upon commercialization of any such products, will be required to pay to Northwestern a tiered royalty on net sales of such products by the Company, its affiliates or sublicensees, at percentages in the low to mid-single-digits, subject to standard reductions and offsets. The Company's royalty obligations continue on a product-by-product and country-by-country basis until the later of the expiration of the last-to-expire valid claim in a licensed patent covering the applicable product in such country and 10 years following the first commercial sale of such product in such

country. If the Company sublicenses a Northwestern Patent Right, it will be obligated to pay to Northwestern a specified percentage of sublicense revenue received by the Company, ranging from the high single-digits to the low-teens.

The Northwestern Agreement requires that the Company use commercially reasonable efforts to develop and commercialize at least one product that is covered by the Northwestern Patent Rights.

Unless earlier terminated, the Northwestern Agreement will remain in force until the expiration of the Company's payment obligations thereunder. The Company has the right to terminate the agreement for any reason upon prior written notice or for an uncured material breach by Northwestern. Northwestern may terminate the agreement for the Company's uncured material breach or insolvency.

AstraZeneca AB License Agreement

In December 2021, the Company entered into an exclusive license agreement with AstraZeneca AB ("AstraZeneca"), for a library of early-stage small molecules targeting the KCC2 transporter, including lead candidate OV350. Upon execution of the agreement, the Company was obligated to pay an upfront cash payment of $5.0 million and issued shares of the Company's common stock in an amount that equaled $7.3 million based on the volume-weighted average price of shares of the Company's common stock for the 30 business days immediately preceding the execution date of the transaction.

Pursuant to the AstraZeneca license agreement, the Company agreed to potential milestone payments of up to $203.0 million upon the achievement of certain developmental, regulatory and sales milestones. The first payment of $3.0 million is due upon the successful completion of the first Phase 2 clinical study of a licensed product following a positive biomarker readout in a Phase 1 clinical study.

Gensaic Collaboration and Option Agreement

In August 2022, the Company entered into an equity agreement and a collaboration and option agreement with Gensaic ("Gensaic Collaboration Agreement"). Under the terms of the equity agreement, the Company invested a total of $5.1 million in exchange for convertible preferred stock in Gensaic. The Company also retained rights to invest in future equity financing rounds. Dr. Jeremy Levin, the Company's Chairman and Chief Executive Officer ("CEO"), is currently the Chairman of Gensaic's board of directors. The Gensaic Collaboration Agreement involves the research and development of Gensaic's proprietary platform for certain rare central nervous system ("CNS") disorder targets. Under the Gensaic Collaboration Agreement, Gensaic granted the Company an option to obtain an exclusive license with respect to certain identified lead phage-derived particle ("PDP") products, which is exercisable at any time prior to the expiration of the option period. Once a product is identified by the Company that demonstrates sufficient efficacy, the Company may exercise its option with respect to the specific research program for that PDP product.

The Company shall reimburse Gensaic for its research costs related to the specific research plan for PDP products identified. The research plan and budget shall be mutually agreed upon by the parties and shall not exceed $3.0 million in any research year. The Company will record these reimbursement payments as research and development costs in the period the research costs are incurred. In May 2023, the Company identified a lead PDP candidate for further research and provided $3.5 million to Gensaic to support the approved research plan and budget. The amount is expensed as the research and development occurs with the remaining amount included in prepaid expenses and other current assets in the condensed consolidated balance sheets. As of September 30, 2025 and December 31, 2024, $1.0 million remained in prepaid expenses and other current assets. No expense was recognized in the three months ended September 30, 2025 and $0.6 million was recognized for the same period in 2024.

If a product is ultimately commercialized under this agreement, the Company shall make tiered royalty payments to Gensaic in the mid-single to low double-digit range based on the net sales of all licensed PDP products during the royalty term. The Company is also responsible for potential tiered milestone payments of up to $452.0 million based upon the achievement of certain sales milestone events and developmental milestone approvals for three or more products. Gensaic also has the option to become a collaborative partner in the development and commercialization of PDP products in exchange for a fee based on a percentage of the costs incurred by the Company through the date Gensaic exercises its option. The Company would no longer be required to pay Gensaic royalty or milestone payments if Gensaic elects to exercise its option. The Company may terminate the Gensaic Collaboration Agreement by providing written notice to Gensaic 90 days in advance of the termination date.

As of September 30, 2025, none of these contingent payments were considered probable.

Non-Operating Loss

During the quarter ended September 30, 2024, the Company was the victim of a criminal scheme involving a business email compromise at one of its development collaborators, which led to a fraudulent transfer totaling $1.8 million

to a third-party impersonating one of the Company's development collaborators. The matter was reported to the U.S. Secret Service and Federal Bureau of Investigation and a loss was recorded in other income (expense) in the condensed consolidated statement of operations. In January 2025, the Company fully recovered the $1.8 million and recorded a gain in other income (expense) in the condensed consolidated statement of operations.

Contingencies

Liabilities for loss contingencies arising from claims, assessments, litigation, fines, and penalties and other sources are recorded when it is probable that a liability has been incurred and the amount can be reasonably estimated. Legal costs incurred in connection with loss contingencies are expensed as incurred. The Company is not currently involved in any legal matters arising in the normal course of business that are material to the Company.

Under the terms of their respective employment agreements, certain of our executive officers are eligible to receive severance payments and benefits upon a termination without "cause" or due to "permanent disability," or upon "resignation for good reason," contingent upon the executive officer's delivery to the Company of a satisfactory release of claims, and subject to the executive officer's compliance with non-competition and non-solicitation restrictive covenants.

NOTE 11 – COLLABORATION AND LICENSE AGREEMENTS

Takeda Collaboration

In January 2017, the Company entered into a license and collaboration agreement with Takeda under which the Company licensed from Takeda certain exclusive rights to develop and commercialize soticlestat in certain territories.

In March 2021, the Company entered into the RLT Agreement, pursuant to which Takeda secured rights to the Company's 50% global share in soticlestat, and the Company granted to Takeda an exclusive worldwide license under the Company's relevant intellectual property rights to develop and commercialize the investigational medicine soticlestat for the treatment of developmental and epileptic encephalopathies, including Dravet syndrome and Lennox-Gastaut syndrome.

Under the RLT Agreement, all rights in soticlestat are owned by Takeda or exclusively licensed to Takeda by the Company. Takeda assumed all responsibility for, and costs of, both development and commercialization of soticlestat, and the Company no longer has any financial obligation to Takeda under the original collaboration agreement, including milestone payments or any future development and commercialization costs. In March 2021, upon the closing of the RLT Agreement, the Company received an upfront payment of $196.0 million and, if soticlestat was successfully developed, the Company would be eligible to receive up to $660.0 million in milestone payments and royalties on product sales.

In October 2023, the Company sold a 13% stake in the royalty, regulatory and commercial milestone payments that the Company is eligible to receive under the RLT Agreement to Ligand for $30.0 million. The Company retained 87% of its interest in soticlestat's potential royalties and milestones.

During the three and nine months ended September 30, 2025 and 2024, no revenue or expense was recognized pursuant to the RLT Agreement. In June 2024, Takeda issued a press release indicating the soticlestat trials missed their primary endpoints and noted that while Takeda would discuss the program with FDA, Takeda had fully impaired the asset representing soticlestat. In January 2025, Takeda announced the discontinuation of the program. As of September 30, 2025, the Company has no debt or other obligations to Ligand.

Healx License and Option Agreement

In February 2022, the Company entered an exclusive license option agreement ("Healx License and Option Agreement") with Healx, Ltd. ("Healx"). Under the terms of the Healx License and Option Agreement, Healx secured a one-year option to investigate gaboxadol ("OV101") as part of a potential combination therapy for Fragile X syndrome in a Phase 1B/2A clinical trial, as well as a treatment for other indications, for an upfront payment of $0.5 million, and fees to support prosecution and maintenance of the Company's relevant intellectual property rights. At the end of the one-year option period, Healx had the option to secure rights to an exclusive license under the Company's relevant intellectual property rights, in exchange for an additional payment of $2.0 million, development and commercial milestone payments, and low to mid-tier double-digit royalties. In February 2023, the Company granted an extension of the option period for up to four months for Healx to continue to investigate gaboxadol. Royalties on net sales, if any, are payable on a country-by-country and product-by-product basis during the period beginning on the date of the first commercial sale of such product in such country and ending on the later to occur of the expiration of patent rights covering the product in such country and a specified anniversary of such first commercial sale.

Healx will assume all responsibility for, and costs of, both development and commercialization of gaboxadol following the exercise of the option. The Company will retain the option to co-develop and co-commercialize the program with Healx ("Ovid Opt-In Right"), at the end of a positive readout of clinical Phase 2B and would share net profits and

losses in lieu of the milestones and royalty payments. If the Ovid-Opt-In Right were exercised, the Company would be required to pay Healx 50% of development costs. The Company does not plan to conduct further trials of gaboxadol. The term of the Healx License and Option Agreement will continue until the later of (a) the expiration of all relevant royalty terms, or in the event that Healx does not exercise its option during the option period defined in the Healx License and Option Agreement ("Option Period"), the expiration of such period, or (b) in the event that Healx does exercise its option during the Option Period, and the Company does not exercise the Ovid Opt-In Right during the period of time it has to opt-in ("Opt-In Period") or the opt-in terms are otherwise terminated, upon the expiration of all payment obligations, or (c) in the event that Healx does exercise the Option during the Option Period, and the Company does exercise the Ovid Opt-In Right during the Opt-In Period, such time as neither Healx nor the Company is continuing to exploit gaboxadol. Further, if the Company exercises the Ovid Opt-In Right to co-develop and co-commercialize the program, it will owe an equal share of any net profits to a third party with which it previously established a licensing agreement. If the Company does not exercise the Ovid Opt-In Right, it will owe the third party an equal share of all milestone and royalty payments received.

In June 2023, the Company entered into an amendment to the Healx License and Option Agreement whereby revisions were made to terms regarding the timing of the option exercise fee payable by Healx to the Company, the clinical and regulatory milestone payment structure, and the royalty payment structure. Additionally, the parties agreed that following the exercise of the option, Healx would assume direct responsibility for patent maintenance and prosecution and that the Company would transfer to Healx all supply obligations with respect to the active pharmaceutical ingredient and finished gaboxadol products and any related licensed technology and know-how in the Company's possession that is relevant to the manufacture of such licensed products.

No revenue was recognized relating to the Healx License and Option Agreement during the three and nine month periods ended September 30, 2025 and 2024.

Marinus Pharmaceuticals Out-License Agreement

In March 2022, the Company entered into an exclusive patent license agreement with Marinus ("Marinus License Agreement"). Under the Marinus License Agreement, the Company granted Marinus an exclusive, non-transferable (except as expressly provided therein), royalty-bearing right and license under certain Ovid patents relating to ganaxolone to develop, make, have made, commercialize, promote, distribute, sell, offer for sale and import licensed products in the territory (which consists of the United States, the European Economic Area, United Kingdom and Switzerland) for the treatment of CDKL5 deficiency disorders. Following the date of regulatory approval by the FDA of the first licensed product in the territory, which was received in March 2022, Marinus issued, at the Company's option, 123,255 shares of Marinus common stock, par value $0.001 per share, as payment. The Marinus License Agreement also provides for payment of royalties from Marinus to the Company in single-digits on net sales of each such licensed product sold.

The Company had unrealized losses on the Marinus common stock of $1.1 million for the nine months ended September 30, 2024, which were recorded as unrealized gains (losses) on equity securities and were reflected in other income (expense), net in the condensed consolidated statements of operations. In February 2025, Immedica closed a cash purchase of Marinus, resulting in the sale of the Company's equity position in Marinus for $70,000.

In June 2025, the Company entered into an amendment to the Marinus License Agreement with Immedica wherein the parties agreed to replace ongoing royalty payment obligations and add additional licensing for a one-time payment of $7.0 million, which was remitted to the Company pursuant to the agreement within 10 days of execution. $6.3 million of the $7.0 million is related to the royalties and existing licenses which the Company recognized as revenue in the three-month period ended June 30, 2025. The remaining $0.7 million is related to additional licensure to be granted within six months of the effective date of the amendment which the Company recorded as deferred revenue until the additional licenses are transferred or granted.

Graviton License Agreement and Equity Purchase

In April 2023, the Company entered into a collaboration and license agreement with Graviton ("Graviton Agreement"), whereby it secured from Graviton an exclusive license to develop and commercialize Graviton's library of ROCK2 inhibitors including their lead program GV101 (OV888) in rare CNS disorders (excluding amyotrophic lateral sclerosis) worldwide (excluding China, Hong Kong, Macau and Taiwan). Under the Graviton Agreement, the Company and Graviton plan to investigate GV101 (OV888) in cerebral cavernous malformations as well as Graviton's library of ROCK2 inhibitors in other rare CNS disorders. The Company will be responsible for all development and commercialization costs of the products. Should the Company receive regulatory approval and commercialize any of Graviton's ROCK2 inhibitors, it will pay Graviton tiered royalties on net sales ranging from the mid- to high-teens. As part of the Graviton Agreement, the Company also purchased shares of Graviton's preferred stock for $10.0 million. The Company recorded the purchase of the preferred stock as a long-term equity investment on its condensed consolidated balance sheets. In December 2023 and March 2024, the Company recognized unrealized gains on the investment due to an

observable change in price, and recorded the gain in other income (expense), net in the condensed consolidated statements of operations. The program related to the Graviton Agreement is currently paused.

In May 2025, the Company and Graviton reviewed disputed amounts invoiced pursuant to the Graviton Agreement pertaining to activity in the second half of 2024. A settlement was agreed upon and payment of approximately $0.36 million less than the originally invoiced amount was made by the Company. As of September 30, 2025, all amounts due to Graviton have been paid in full and prior Graviton-related accruals reduced to zero.

NOTE 12 – RELATED PARTY TRANSACTIONS

In March 2021, the Company entered into the RLT Agreement with Takeda. For a description of the RLT Agreement, see Note 11 – Collaboration and License Agreements.

NOTE 13 – NET LOSS PER SHARE

The basic and diluted net loss per common share is presented in conformity with the two-class method required for participating securities and multiple classes of shares. The Company considers its preferred stock to be in-substance common stock (see Note 2). The Series A Preferred Stock was excluded from the calculation of net loss per share and presented as anti-dilutive in prior reporting periods, but is deemed in-substance common stock and is now reflected as a class of common stock for purposes of calculating net loss per share for the three and nine month periods ended September 30, 2025 and 2024. The impact of the change to previously reported net loss per share is not material.

Basic net loss per common share is calculated based upon the allocation of net loss to the weighted-average number of common shares outstanding during the period. For any period in which the Company records net income, diluted net income per share is calculated in the same manner as basic net loss per share, except that diluted net income per common share includes outstanding common stock, common shares underlying outstanding options, and unvested restricted stock units in the number of shares used to allocate net income to share classes and in the denominator in calculating diluted net income per common share. Diluted net income per share also considers the potential impact of preferred stock using the if-converted method.

Diluted net loss per common share is equivalent to the basic net loss per common share due to the exclusion of outstanding stock options and unvested restricted stock units because the inclusion of these securities would result in an anti-dilutive effect on per common share amounts.

The following table summarizes the calculation of basic and diluted net loss per share:

	For the Three Months Ended September 30, 2025		For the Three Months Ended September 30, 2024	
(in thousands, except share and per share data)	Series A Preferred Stock	Common Stock	Series A Preferred Stock	Common Stock
Net loss per share, basic and diluted:				
Allocation of net loss	$ (210)	$ (11,948)	$ (242)	$ (13,764)
Weighted average shares outstanding, basic and diluted	1,250	71,114,181	1,250	70,975,785
Net loss per share, basic and diluted	$ (168.01)	$ (0.17)	$ (193.92)	$ (0.20)

	For the Nine Months Ended September 30, 2025		For the Nine Months Ended September 30, 2024	
(in thousands, except share and per share data)	Series A Preferred Stock	Common Stock	Series A Preferred Stock	Common Stock
Net loss per share, basic and diluted:				
Allocation of net loss	$ (468)	$ (26,609)	$ (298)	$ (16,882)
Weighted average shares outstanding, basic and diluted	1,250	71,090,052	1,250	70,870,224
Net loss per share, basic and diluted	$ (374.30)	$ (0.37)	$ (238.21)	$ (0.24)

The following potentially dilutive securities have been excluded from the computations of diluted weighted-average shares outstanding as they would be anti-dilutive:

	September 30, 2025	September 30, 2024
Stock options to purchase common stock	18,401,818	17,036,145
Common stock issuable upon conversion of Series A Preferred Stock	1,250,000	1,250,000
Unvested restricted stock units	341,105	209,700
	19,992,923	18,495,845

NOTE 14 – SEGMENT REPORTING

The Company has determined that it operates as one segment focused on developing small molecule medicines for brain conditions with significant unmet need. The Company's precommercial drug development candidates have similar economic and other characteristics, including all being in the small molecule therapeutic class which shares target markets, development pathways, and regulatory environments. The CODM is the Chairman and CEO, who reviews profit and loss information on a consolidated basis to assess performance and make operating and planning decisions, including resource allocations among programs. The determination of the single segment is consistent with the information provided to the CODM. As the Company's operations are comprised of a single reporting segment, the segment assets are reflected on the accompanying condensed consolidated balance sheet as "total assets." Segment asset information is not used by the CODM to make operating and planning decisions or allocate resources.

The following tables summarize the Company's segment information as presented to the CODM and as required in ASU 2023-07 for the periods indicated:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
(in thousands)	2025	2024	2025	2024
Revenue	$ 132	$ 173	$ 6,534	$ 490
Payroll and payroll-related expenses	1,778	1,631	5,417	8,466
Direct program expenses				
OV350/KCC2 library	1,573	2,795	6,980	7,166
OV329	1,607	1,011	4,063	3,938
OV888 (GV101)	—	1,051	(224)	6,851
Gensaic projects	—	500	—	1,493
Other programs	304	170	998	737
Total direct program expenses	3,485	5,526	11,818	20,186
Other research and development expenses	607	698	1,758	2,192
Total research and development expenses	5,870	7,855	18,993	30,844
Payroll and payroll-related expenses	2,045	2,707	7,424	11,435
Legal and professional fees	3,593	1,549	6,322	5,362
General office expenses	1,147	1,288	3,941	4,012
Total general and administrative expenses	6,785	5,544	17,687	20,809
Total operating expenses	12,655	13,399	36,680	51,653
Operating loss	(12,523)	(13,226)	(30,146)	(51,163)
Other income (expense), net	365	(780)	3,069	33,983
Net loss	$ (12,158)	$ (14,006)	$ (27,077)	$ (17,180)

The program expense for OV888 (GV101) is negative for the nine month period ended September 30, 2025 because the Company recognized a contra-expense upon settlement of the amounts due to its collaboration partner and on reversal of an immaterial accrual estimate on final determination of amounts owed.

Other research and development expenses include general office expenses allocated to research and development, including costs related to rent, depreciation of leasehold improvements and nonclinical contract labor. Other income/

expense includes the impact of a fraudulent funds transfer, change in valuation of royalty monetization liability, unrealized net gain on equity investments and interest/accretion income on securities.

Other significant segment information includes:

(in thousands)	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2025	2024	2025	2024
Stock-based compensation expense	1,108	1,308	3,612	5,016
Interest/accretion income on securities	303	947	1,179	3,265
Severance expense	47	137	482	3,550
Gain on recovery of fraudulent funds transfer	—	—	1,800	—
Loss on fraudulent funds transfer	—	1,800	—	1,800
Change in valuation of royalty monetization liability	—	—	—	29,028
Unrealized net gain on equity investments	—	73	—	3,500
Depreciation and amortization	17	168	256	453

NOTE 15 – SUBSEQUENT EVENTS

October 2025 Private Placement

On October 2, 2025, Company entered into a Securities Purchase Agreement (the "Purchase Agreement") with the purchasers named therein (the "Investors"), pursuant to which the Company agreed to issue and sell an aggregate of (i) 57,722 shares of its Series B convertible preferred stock, par value $0.001 per share (the "Series B Preferred Stock"), (ii) Series A warrants (the "Series A Warrants") to purchase 38,481,325 shares of the Company's common stock and/or pre-funded warrants to purchase common stock (the "Pre-Funded Warrants") and (iii) Series B warrants to purchase 28,861,000 shares of common stock and/or Pre-Funded Warrants (the "Series B Warrants" and, together with the Series A Warrants, the "Warrants") to the Investors in the Private Placement. Each share of Series B Preferred Stock was sold together with a Series A Warrant to purchase 666.66 shares of common stock and/or Pre-Funded Warrants (rounded down to next whole share based on each investor's aggregate purchase) and a Series B Warrant to purchase 500 shares of common stock and/or Pre-Funded Warrants (together, a "Security"). The Securities were sold at a purchase price of $1,400.00 per Security to the Investors, which included the purchase of 71 Series B Preferred Shares, 47,333 Series A Warrants, and 35,500 Series B Warrants by the Company's Chief Executive Officer, pending shareholder approval of the Chief Executive Officer's participation in the Private Placement. The Warrants each have an exercise price of $1.40 per share.

Subject to the terms and limitations contained in the Certificate of Designation of Preferences, Rights and Limitations of Series B Convertible Preferred Stock (the "Certificate of Designation"), the Series B Preferred Stock issued in the Private Placement will not become convertible until the Company's stockholders have approved each of (i) an increase in the number of authorized shares of common stock to enable the Company to issue all of the shares of common stock that are issuable upon the conversion of the Series B Preferred Stock (the "Conversion Shares") and the shares of common stock that are issuable upon the exercise of the Warrants (the "Warrant Shares"), and (ii) the issuance of the Conversion Shares and the Warrant Shares in accordance with the listing rules of the Nasdaq Stock Market (collectively, the "Stockholder Approval"). Effective as of 5:00 p.m. Eastern Time on the second business day after the date of the Stockholder Approval and subject to the Company filing an amendment to the Certificate of Incorporation with the Secretary of State of the State of Delaware evidencing such Stockholder Approval, each share of Series B Preferred Stock shall automatically convert into common stock at the Conversion Ratio (as defined in the Certificate of Designation), subject to the terms and limitations contained in the Certificate of Designation.

The Private Placement may result in gross proceeds of up to $175.1 million to the Company, which includes the initial gross proceeds of $80.8 million before placement agent fees and offering expenses, or approximately $75.7 million in net proceeds.

Special Meeting of Stockholders

On October 27, 2025, the Company filed a Proxy Statement for a special meeting of stockholders (the "Special Meeting") to be held December 11, 2025. The Special Meeting will be held virtually and the record date for the special meeting is October 31, 2025. The Company's stockholders are scheduled for a vote on three matters at the Special Meeting: (i) to approve an authorized share increase from 125,000,000 shares to 315,000,000 shares, (ii) to approve Dr. Levin's participation in the Private Placement in accordance with Nasdaq Listing Rule 5635(c), and (iii) to approve the

issuance of common shares on the conversion of the Series B convertible preferred shares and on exercise of the Class A and Class B common warrants in accordance with Nasdaq Listing Rule 5635(d).

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following information should be read in conjunction with our unaudited condensed consolidated financial statements and notes thereto included in this Quarterly Report on Form 10-Q and the audited financial information and the notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2024, which was filed with the Securities and Exchange Commission ("SEC") on March 11, 2025. In addition to historical financial information, the following discussion contains forward-looking statements based upon our current plans, expectations and beliefs that involve risks, uncertainties and assumptions. Our actual results and the timing of selected events may differ materially from those described in or implied by these forward-looking statements as a result of many factors, including those set forth under the section titled "Risk Factors" in Part II, Item 1A. You should carefully read the "Risk Factors" section of this Quarterly Report on Form 10-Q to gain an understanding of the important factors that could cause actual results to differ materially from our forward-looking statements. Please also see the section entitled "Special Note Regarding Forward-Looking Statements."

Overview

We are a biopharmaceutical company dedicated to developing small molecule medicines for brain conditions with significant unmet need. Our approach to achieve this goal is scientifically driven, patient focused, and coupled with an integrated and disciplined approach to research, clinical development and business development. Our team has significant experience with and understanding of epilepsies and other neurological conditions, and we continue to gain insight into the ways the different molecular mechanisms and pathways underlying these disorders impact the symptoms patients experience. We have developed a differentiated pipeline of drug candidates containing three novel mechanisms of action ("MoAs") to target seizures and believe we are the only company that holds a portfolio of direct activators of potassium-chloride cotransporter 2 ("KCC2"). Two of our programs are in clinical trials, and a third is expected to begin a clinical trial in the second quarter of 2026. We are initially pursuing therapeutic drug candidates for epilepsy and psychoses associated with Parkinson's disease and Lewy body dementia. If successfully developed and marketed to treat these conditions, we intend to explore these drug candidates for broader neurologic indications. Our cohesive focus in brain conditions with significant unmet need reinforces our belief that we can develop and produce multiple novel medicines, scale our infrastructure, positively impact patients' lives and create long-term stockholder value.

Since our inception in April 2014, we have devoted substantially all of our efforts to organizing and planning our business, building our management and technical team, acquiring and developing assets and raising capital. During the three and nine month periods ended September 30, 2025, we recorded $0.1 million and $6.5 million of royalty and licensing revenue, respectively. We recorded net losses of $12.2 million and $27.1 million, respectively, during the three and nine month periods ended September 30, 2025. We are highly dependent on our ability to find additional sources of funding through either equity offerings, debt financings, collaborations, strategic alliances, licensing agreements or a combination of any such transactions. Through September 30, 2025, we had raised net proceeds of $275.4 million from the sale of our preferred and common stock. As of September 30, 2025, we had $25.6 million in cash, cash equivalents and marketable securities and an accumulated deficit of $331.4 million. In October 2025, we closed a Private Placement (as defined below), which provided initial net proceeds of $75.7 million, after placement agent fees and offering expenses. Management believes the cash, cash equivalents and marketable securities as of September 30, 2025, plus the initial proceeds from the Private Placement, will be sufficient to fund our current operating plans for at least the next 12 months from the issuance of the financial statements contained in this Quarterly Report on Form 10-Q.

We expect to continue to incur significant expenses and operating losses for at least the next several years. Our net losses may fluctuate significantly from period to period, depending on the timing of our planned clinical trials and expenditures on our other research and development and commercial development activities. We expect our expenses will increase substantially over time as we:

- continue the ongoing and planned preclinical and clinical development of our drug candidates;
- build a portfolio of drug candidates through the development, acquisition or in-license of drugs, drug candidates or technologies;
- initiate preclinical studies and clinical trials for any additional drug candidates that we may pursue in the future;
- seek marketing approvals for our current and future drug candidates that successfully complete clinical trials;
- establish a sales, marketing and distribution infrastructure to commercialize any drug candidate for which we may obtain marketing approval;

- develop, maintain, expand and protect our intellectual property portfolio;
- implement operational, financial and management systems; and
- attract, hire and retain additional administrative, clinical, regulatory, manufacturing, commercial and scientific personnel.

The following chart sets forth the status and mechanism of action of our drug candidates:

Programs	Indication opportunities	Preclinical	Phase 1	Phase 2	Anticipated milestones
GABA-aminotransferase (GABA-AT) inhibitor					
OV329	Drug-resistant adult focal onset seizures (FOS)				Findings for 7 mg dose safety, tolerability, PK & exposure in healthy volunteers (Q1 2026) Phase 2a initiation (Q2 2026); Phase 2a topline results (mid-2027)
Potassium-chloride cotransporter 2 (KCC2) direct activator portfolio					
OV350 IV	First-in-human direct activation of KCC2				Phase 1 topline findings (Q4 2025) to include safety, tolerability and PK results
OV4071 oral	Psychosis assoc. with Parkinson's disease and Lewy body dementia and schizophrenia				Regulatory submission & clearance (Q1 2026) Phase 1 initiation (Q2 2026) Proof-of-mechanism ketamine study (mid-2026) Patient Phase 1b initiations (Q3 2026) Topline PoC results in PDP/LBD and SCZ (Q1 2027)
OV4041 oral	Generalized anxiety disorder and Rett syndrome				Initiating IND-enabling late 2025 IND submission late H2 2026

Recent Developments

Private Placement

On October 2, 2025, we entered into a Securities Purchase Agreement with the purchasers named therein (the "Investors"), pursuant to which we agreed to issue and sell an aggregate of (i) 57,722 shares of our Series B convertible preferred stock, par value $0.001 per share (the "Series B Preferred Stock"), (ii) Series A warrants (the "Series A Warrants") to purchase 38,481,325 shares of our common stock, par value $0.001 per share and/or pre-funded warrants to purchase common stock (the "Pre-Funded Warrants") and (iii) Series B warrants to purchase 28,861,000 shares of common stock and/or Pre-Funded Warrants (the "Series B Warrants" and, together with the Series A Warrants, the "Warrants") to the Investors in a private placement (the "Private Placement"). Each share of Series B Preferred Stock was sold together with a Series A Warrant to purchase 666.66 shares of common stock and/or Pre-Funded Warrants (rounded down to next whole share based on each investor's aggregate purchase) and a Series B Warrant to purchase 500 shares of common stock and/or Pre-Funded Warrants (together, a "Security"). The Securities were sold at a purchase price of $1,400.00 per Security to the Investors, which includes our Chief Executive Officer. The Warrants each have an exercise price of $1.40 per share.

The Private Placement may result in gross proceeds of up to $175.1 million to us, which includes the initial gross proceeds of $80.8 million, before estimated placement agent fees and offering expenses, or $75.7 million in estimated net proceeds received at closing.

Data Release

On October 3, 2025, we announced positive topline Phase 1 trial data for our next-generation GABA-aminotransferase inhibitor, OV329. The data demonstrate strong inhibitory activity and a potential best-in-category safety profile for GABA-aminotransferase inhibitors.

Significant Risks and Uncertainties

The global economic slowdown, the overall disruption of global healthcare systems and other risks and uncertainties associated with public health crises and global geopolitical tensions, like tensions between China and Taiwan and the ongoing wars involving Ukraine and Israel, may have a material adverse effect on our business, financial condition, results of operations and growth prospects. The resulting fluctuations in inflation rates may materially affect our business and corresponding financial position and cash flows. Inflationary factors, such as increases in the cost of our clinical trial

materials and supplies, interest rates and overhead costs may adversely affect our operating results. Relatively high interest rates also present a recent challenge impacting the U.S. economy and could make it more difficult for us to obtain traditional financing on acceptable terms, if at all, in the future. Furthermore, economic conditions have produced downward pressure on share prices. Although we do not believe that inflation has had a material impact on our financial position or results of operations to date, we may experience increases in the near future (especially if inflation rates remain relatively high or begin to rise again) on our operating costs, including our labor costs and research and development costs, due to supply chain constraints, global geopolitical tensions as a result of tensions between China and Taiwan and the ongoing wars involving Ukraine and Israel, worsening global macroeconomic conditions and employee availability and wage increases, which may result in additional stress on our working capital resources. Moreover, there is great uncertainty with respect to potential changes in trade regulations, ongoing changes to U.S. and international tariffs and other trade restrictions and trade barriers, renegotiation of international trade agreements or further escalation of trade tensions, sanctions and export controls which also increase volatility in the global economy.

In addition, we are subject to other challenges and risks specific to our business and our ability to execute on our strategy, as well as risks and uncertainties common to companies in the pharmaceutical industry with development and commercial operations, including, without limitation, risks and uncertainties associated with: identifying, acquiring or in-licensing products or product candidates; obtaining regulatory approval of product candidates; pharmaceutical product development and the inherent uncertainty of clinical success; the challenges of protecting and enhancing our intellectual property rights; and complying with applicable regulatory requirements.

We recorded net losses of $12.2 million and $27.1 million, respectively, during the three and nine month periods ended September 30, 2025, had an accumulated deficit of $331.4 million and working capital of $22.0 million as of September 30, 2025, and expect to incur losses for at least the next several years. We are highly dependent on our ability to find additional sources of funding through either equity offerings, debt financings, collaborations, strategic alliances, licensing agreements or a combination of any such transactions.

Financial Operations Overview

Revenue

We have generated revenue under various licensing and collaboration agreements. We have not generated any revenue from commercial drug sales, and we do not expect to generate any further revenue unless or until we obtain regulatory approval and commercialize one or more of our current or future drug candidates. In the future, we may also seek to generate revenue from a combination of research and development payments, license fees and other upfront or milestone payments.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our product discovery efforts and the development of our product candidates, which include, among other things:

- employee-related expenses, including salaries, benefits and stock-based compensation expense;
- fees paid to consultants for services directly related to our drug development and regulatory efforts;
- expenses incurred under agreements with contract research organizations, as well as contract manufacturing organizations and consultants that conduct preclinical studies and clinical trials;
- costs associated with preclinical activities and development activities;
- costs associated with technology and intellectual property licenses;
- milestone payments and other costs and payments under licensing agreements, research agreements and collaboration agreements; and
- depreciation expense for assets used in research and development activities.

Costs incurred in connection with research and development activities are expensed as incurred. Costs for certain development activities, such as clinical trials, are recognized based on an evaluation of the progress to completion of specific tasks using data such as patient enrollment, clinical site activations or other information provided to us by our vendors.

Research and development activities are and will continue to be central to our business model. We expect our research and development expenses to increase for the foreseeable future as we advance our current and future drug candidates through preclinical studies and clinical trials. The process of conducting preclinical studies and clinical trials necessary to obtain regulatory approval is costly and time-consuming. It is difficult to determine with certainty the duration

and costs of any preclinical study or clinical trial that we may conduct. The duration, costs and timing of clinical trial programs and development of our current and future drug candidates will depend on a variety of factors that include, but are not limited to, the following:

- number of clinical trials required for approval and any requirement for extension trials;
- per patient trial costs;
- number of patients who participate in the clinical trials;
- number of sites included in the clinical trials;
- countries in which the clinical trial is conducted;
- length of time required to enroll eligible patients;
- number of doses that patients receive;
- drop-out or discontinuation rates of patients;
- potential additional safety monitoring or other studies requested by regulatory agencies;
- duration of patient follow-up; and
- efficacy and safety profile of the drug candidates.

In addition, the probability of success for any of our current or future drug candidates will depend on numerous factors, including competition, manufacturing capability and commercial viability. We will determine which programs to pursue and how much to fund each program in response to the scientific and clinical success of each drug candidate, as well as an assessment of each drug candidate's commercial potential.

General and Administrative Expenses

General and administrative expenses consist primarily of employee-related expenses, including salaries, benefits and stock-based compensation expense, related to our executive, finance, legal, business development and support functions. Other general and administrative expenses include costs associated with operating as a public company, travel expenses, conferences, and professional fees for auditing, tax and legal services.

Other Income (Expense), net

Other income (expense), net primarily consists of interest income and accretion of discount on investments in marketable securities, changes in valuation of royalty monetization liability, unrealized gains (losses) on long-term equity investments and the impact of a fraudulent funds transfer.

Results of Operations

Comparison of the Three Months Ended September 30, 2025 and 2024

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

(in thousands)	Three Months Ended September 30, 2025	Three Months Ended September 30, 2024	Change $
Revenue:			
License and other revenue	$ 132	$ 173	$ (41)
Total revenue	132	173	(41)
Operating expenses:			
Research and development	5,870	7,855	(1,985)
General and administrative	6,785	5,544	1,241
Total operating expenses	12,655	13,399	(744)
Loss from operations	(12,523)	(13,226)	703
Other income (expense), net	365	(780)	1,145
Loss before provision for income taxes	(12,158)	(14,006)	1,848
Provision for income taxes	—	—	—
Net loss	$ (12,158)	$ (14,006)	$ 1,848

Revenue

Revenue of $0.1 million and $0.2 million was recorded in the three months ended September 30, 2025 and 2024, respectively, which related to quarterly royalties on net sales pursuant to the Marinus License Agreement.

Research and Development Expenses

(in thousands)	Three Months Ended September 30, 2025	Three Months Ended September 30, 2024	Change $
Preclinical and clinical development expenses	$ 3,485	$ 5,526	$ (2,041)
Payroll and payroll-related expenses	1,778	1,631	147
Other expenses	607	698	(91)
Total research and development	$ 5,870	$ 7,855	$ (1,985)

During the three months ended September 30, 2025, research and development expenses were $5.9 million, compared to $7.9 million for the same period in 2024, representing a decrease of $2.0 million related primarily due to the pause of the OV888 (GV101) program.

General and Administrative Expenses

(in thousands)	Three Months Ended September 30, 2025	Three Months Ended September 30, 2024	Change $
Payroll and payroll-related expenses	$ 2,045	$ 2,707	$ (662)
Legal and professional fees	3,593	1,549	2,044
General office expenses	1,147	1,288	(141)
Total general and administrative	$ 6,785	$ 5,544	$ 1,241

General and administrative expenses were $6.8 million and $5.5 million for the three months ended September 30, 2025 and 2024, respectively. The increase in general and administrative expenses is primarily due to business development professional fees.

Other Income (Expense), net

Other income (expense), net, for the three months ended September 30, 2025 and 2024 were a gain of $0.4 million and a loss of $0.8 million, respectively. Items reflected in other income (expense), net, in both periods were primarily interest income and accretion on U.S. treasury investments, as well as a loss recognized on a long-term equity investment in the three months ended September 30, 2024.

Comparison of the Nine Months Ended September 30, 2025 and 2024

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

	Nine Months Ended September 30, 2025	Nine Months Ended September 30, 2024	Change $
		(in thousands)	
Revenue:			
License and other revenue	$ 6,534	$ 490	$ 6,044
Total revenue	6,534	490	6,044
Operating expenses:			
Research and development	18,993	30,844	(11,851)
General and administrative	17,687	20,809	(3,122)
Total operating expenses	36,680	51,653	(14,973)
Loss from operations	(30,146)	(51,163)	21,017
Other income (expense), net	3,069	33,983	(30,914)
Loss before provision for income taxes	(27,077)	(17,180)	(9,897)
Provision for income taxes	—	—	—
Net loss	$ (27,077)	$ (17,180)	$ (9,897)

Revenue

Revenue of $6.5 million was generated in the nine months ended September 30, 2025, compared to revenue of $0.5 million recognized for the same period in 2024. Revenue originated from quarterly royalties on net sales pursuant to the Marinus License Agreement for both periods and from the amended Marinus License Agreement in the nine months ended September 30, 2025.

Research and Development Expenses

	Nine Months Ended September 30, 2025	Nine Months Ended September 30, 2024	Change $
		(in thousands)	
Preclinical and clinical development expenses	$ 11,818	$ 20,186	$ (8,368)
Payroll and payroll-related expenses	5,417	8,466	(3,049)
Other expenses	1,758	2,192	(434)
Total research and development	$ 18,993	$ 30,844	$ (11,851)

During the nine months ended September 30, 2025, research and development expenses were $19.0 million compared to $30.8 million for the same period in 2024. The decrease of $11.9 million is primarily due to the pause of the OV888 (GV101) program, cost decreases associated with the organizational restructuring announced in June 2024, and a reduction in overall average headcount between the nine month periods ended September 30, 2024 and 2025. Payroll and payroll-related costs of the organizational restructuring of $1.7 million were recognized during the nine months ended September 30, 2024 and paid over an extended period.

General and Administrative Expenses

	Nine Months Ended September 30, 2025	Nine Months Ended September 30, 2024	Change $
	(in thousands)		
Payroll and payroll-related expenses	$ 7,424	$ 11,434	$ (4,010)
Legal and professional fees	6,322	5,362	960
General office expenses	3,941	4,013	(72)
Total general and administrative	$ 17,687	$ 20,809	$ (3,122)

General and administrative expenses were $17.7 million for the nine months ended September 30, 2025 compared to $20.8 million for the same period in 2024. The decrease of $3.1 million was primarily due to cost decreases associated with the organizational restructuring announced in June 2024, reduced average headcount in 2025 and the realization of cost reduction strategies initiated since June 30, 2024. Costs of the organizational restructuring of $2.0 million were recognized during the nine months ended June 30, 2024 and paid over an extended period.

Other Income (Expense), net

Other income (expense), net for the nine months ended September 30, 2025 was $3.1 million compared to $34.0 million for the same period in 2024. The decrease of $30.9 million was primarily due to other income of $29.0 million that was recognized in June 2024 relating to an adjustment to the royalty monetization liability under the Ligand Agreement. The remainder of the decrease resulted from the net of unrealized gain (loss) on long-term equity investments, interest earned and accretion of discount on marketable securities, and an unrealized gain due to the recovery of a fraudulent funds transfer.

Liquidity and Capital Resources

Overview

We believe that our available cash, cash equivalents and marketable securities, plus the initial proceeds from the Private Placement discussed above, will be sufficient to fund our current operating plans for at least the next 12 months from the issuance of the financial statements contained in this Quarterly Report on Form 10-Q.

Similar to other development-stage biotechnology companies, we have generated limited revenue, which has been through various license and collaboration agreements, and have financed our operations primarily through the sale of equity securities. We have historically incurred losses and experienced negative operating cash flows for most periods since our inception and anticipate that we will incur losses and experience negative operating cash flows in the future. We recorded net losses of $12.2 million and $27.1 million, respectively, during the three and nine month periods ended September 30, 2025, had an accumulated deficit of $331.4 million and working capital of $22.0 million as of September 30, 2025, and expect to incur losses for at least the next several years.

Future Funding Requirements

Our primary uses of capital are, and we expect will continue to be, compensation and related expenses, third-party clinical research and development services, clinical costs, legal and other regulatory expenses and general overhead costs. We have based our estimates on assumptions that may prove to be incorrect, and we could use our capital resources sooner than we currently expect. Additionally, the process of testing drug candidates in clinical trials is costly, and the timing of progress in these trials is uncertain. We cannot estimate the actual amounts necessary to successfully complete the development and commercialization of our product candidates or whether, or when, we may achieve profitability.

As of September 30, 2025, we had no long-term debt and no material non-cancelable purchase commitments with service providers, as we have generally contracted on a cancellable, purchase order basis. We cannot estimate whether we will receive or the timing of any potential contingent payments upon the achievement by us of clinical, regulatory and commercial events, as applicable. In addition, we cannot estimate the timing of any potential royalty payments that we may be required to make under license agreements we have entered into with various entities pursuant to which we have in-licensed certain intellectual property as contractual obligations or commitments, including agreements with AstraZeneca and Northwestern. Pursuant to these license agreements, we have agreed to make milestone payments up to an aggregate of $279.3 million upon the achievement of certain development, regulatory and sales milestones. We excluded these contingent payments from the condensed consolidated financial statements, given that the timing, probability, and amount, if any, of such payments cannot be reasonably estimated at this time.

In September 2021, we entered into a 10-year lease agreement for our corporate headquarters in New York, New York. The lease provides for monthly rental payments over the lease term. The base rent under the lease is currently $2.3 million per year. Payment obligations under the lease agreement include $2.3 million in the 12 months subsequent to September 30, 2025 and $17.6 million over the remaining term of the agreement. For additional information see Note 5 to our condensed consolidated financial statements under the heading "Leases."

We have no products approved for commercial sale and have not generated any revenue from product sales to date. Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings and additional funding from license and collaboration arrangements. Except for any obligations of our collaborators to reimburse us for research and development expenses or to make milestone or royalty payments under our agreements with them, we will not have any committed external source of liquidity. To the extent that we raise additional capital through future equity offerings or debt financings, ownership interests may be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt and equity financings, 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. There can be no assurance that such financings will be obtained on terms acceptable to us, if at all.

Additionally, while the long-term economic impact of geopolitical tensions, including tensions between China and Taiwan and the ongoing wars involving Ukraine and Israel, is difficult to assess or predict, each of these events has caused significant disruptions to the global financial markets and contributed to a general global economic slowdown. Furthermore, inflation rates have increased recently to levels not seen in decades, which may also be impacted by the implementation of tariffs by the United States and other countries. Moreover, there is great uncertainty with respect to potential changes in trade regulations, tariffs, sanctions and export controls which also increase volatility in the global economy. In addition, the U.S. Federal Reserve has raised interest rates in the past in response to concerns about inflation. Relatively high interest rates and fluctuations in inflation, especially if coupled with a significant change in government spending and volatility in financial markets, may further increase economic uncertainty and heighten these risks. If the disruptions and slowdown deepen or persist, we may not be able to access additional capital on favorable terms, or at all, which could in the future negatively affect our ability to pursue our business strategy.

If we raise additional funds through collaborations, strategic alliances or licensing agreements with third parties for one or more of our current or future drug candidates, we may be required to relinquish valuable rights to our technologies, future revenue streams, research programs or drug candidates or to grant licenses on terms that may not be favorable to us. Our failure to raise capital as and when needed would have a material adverse effect on our financial condition and our ability to pursue our business strategy. We may be required to take additional actions beyond the cost preservation measures initiated to address our liquidity needs, including exploring other strategic options, continuing to further reduce operating expense or delaying, reducing the scope of, discontinuing or altering our research and development activities. See "Risk Factors" for additional risks associated with our capital requirements.

At-the-Market Offering Program

In November 2023, we filed a shelf registration statement on Form S-3 (Registration No. 333-275307) (the "S-3 Registration Statement") that allows us to sell up to an aggregate of $250.0 million of our common stock, preferred stock, debt securities and/or warrants, which includes a prospectus covering the issuance and sale of up to $75.0 million of common stock pursuant to an at-the-market ("ATM") offering program. During the three months ended September 30, 2025, we did not have any sales pursuant to our ATM program.

Cash Flows

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

(in thousands)	Nine Months Ended September 30, 2025	Nine Months Ended September 30, 2024
Net cash (used in) provided by:		
Operating activities	$ (27,926)	$ (45,948)
Investing activities	22,208	33,302
Financing activities	28	622
Net increase in cash, cash equivalents and restricted cash	$ (5,690)	$ (12,024)

Net Cash Used In Operating Activities

Net cash used in operating activities was $27.9 million for the nine months ended September 30, 2025, which consisted primarily of net loss of $27.1 million with a reduction of $4.6 million of accounts payable and accrued expenses offset by $3.6 million of noncash stock-based compensation expense. Net cash used in operating activities was $45.9 million for the nine months ended September 30, 2024, which primarily consisted of net loss of $17.2 million, $29.0 million fair value adjustment of the royalty monetization liability, $3.5 million of unrealized gain on long term equity investments, and $5.0 million of noncash stock-based compensation expense.

Net Cash Provided By Investing Activities

Net cash provided by investing activities was $22.2 million and $33.3 million for the nine months ended September 30, 2025 and 2024, respectively, which was primarily due to the maturity of marketable securities during the periods.

Net Cash Provided By Financing Activities

Net cash provided by financing activities during the nine months ended September 30, 2025 and 2024 resulted solely from proceeds from the exercise of stock options and purchases under the employee stock purchase plan.

Smaller Reporting Company Status and a Non-Accelerated Filer

We are a smaller reporting company as defined in the Exchange Act. We may take advantage of certain of the scaled disclosures available to smaller reporting companies and will be able to take advantage of these scaled disclosures for so long as (i) our voting and non-voting common stock held by non-affiliates is less than $250.0 million measured on the last business day of our second fiscal quarter or (ii) our annual revenue is less than $100.0 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is less than $700.0 million measured on the last business day of our second fiscal quarter.

As a smaller reporting company, we are permitted to comply with scaled-back disclosure obligations in our SEC filings compared to other issuers, including with respect to disclosure obligations regarding executive compensation in our periodic reports and proxy statements. We have elected to adopt the accommodations available to smaller reporting companies, including but not limited to:

- reduced disclosure obligations regarding our executive compensation arrangements; and
- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced “Management’s Discussion and Analysis of Financial Condition and Results of Operations” disclosure.

Additionally, as a non-accelerated filer, we may continue to take advantage of the exception from compliance with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended.

Critical Accounting Policies and Estimates

Our management’s discussion and analysis of financial condition and results of operations is based on our financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States. 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 revenue and expenses incurred during the reported periods. On an ongoing basis, we evaluate our estimates and judgments, including those related to accrued expenses and stock-based compensation. We base our estimates on 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 apparent from other sources. Changes in estimates are reflected in reported results for the period in which they become known. Actual results may differ from these estimates under different assumptions or conditions.

During the three and nine month periods ended September 30, 2025, there were no material changes to our critical accounting policies as reported for the year ended December 31, 2024 as part of our Annual Report on Form 10-K, which was filed with the SEC on March 11, 2025. In addition, see Note 2 of our condensed consolidated financial statements under the heading “Recent Accounting Pronouncements” for new accounting pronouncements or changes to the accounting pronouncements during the three and nine months ended September 30, 2025.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

The primary objectives of our investment activities are to ensure liquidity and to preserve capital. As of September 30, 2025, we had cash, cash equivalents and marketable securities totaling $25.6 million. Our primary exposure

to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the short-term maturities of our cash equivalents and marketable securities and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our cash equivalents and marketable securities. To minimize the risk in the future, we intend to maintain our portfolio of cash equivalents and marketable securities in institutional market funds that are comprised of U.S. treasury and U.S. treasury-backed repurchase agreements as well as treasury notes and high quality short-term corporate bonds. We maintain our cash, cash equivalents and marketable securities with domestic financial institutions of high credit quality.

Item 4. Controls and Procedures

Management's Evaluation of our Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Securities Exchange Act of 1934, as amended (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 officer and principal financial officer, to allow timely decisions regarding required disclosure.

As of September 30, 2025, our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act). 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 management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our principal executive officer and principal financial officer have concluded based upon the evaluation described above that, as of September 30, 2025, our disclosure controls and procedures were effective.

Management believes that the consolidated financial statements included in this Quarterly Report on Form 10-Q fairly present, in all material respects, our financial position, results of operations, and cash flows as of and for the periods presented, in accordance with GAAP.

Changes in Internal Control over Financial Reporting

There have been no changes in our internal control over financial reporting during our most recent quarter ended September 30, 2025 that materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

We are not currently subject to any material legal proceedings.

Item 1A. Risk Factors

An investment in our securities involves a high degree of risk. You should carefully consider the following information about these risks, together with the other information appearing elsewhere in this Quarterly Report on Form 10-Q, including our unaudited condensed consolidated financial statements and related notes hereto, before deciding to invest in our common stock. The occurrence of any of the following risks could have a material adverse effect on our business, financial condition, results of operations and future growth prospects or cause our actual results to differ materially from those contained in forward-looking statements we have made in this report and those we may make from time to time. In these circumstances, the market price of our common stock could decline and stockholders may lose all or part of their investment. We cannot assure you that any of the events discussed below will not occur.

Summary of Select Risks Associated with Our Business

Our business faces significant risks and uncertainties. If any of the following risks are realized, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected. Some of the more significant risks we face include the following:

- We will require additional capital to finance our operations, which may not be available on acceptable terms, if at all. Failure to obtain this necessary capital when needed will force us to delay, limit or terminate certain of our drug development efforts or other operations.
- We expect to continue to incur substantial operating losses for the foreseeable future and may never achieve or maintain profitability.
- Our operating history may make it difficult to evaluate the success of our business to date and to assess our future viability.
- Our business could be adversely affected by economic downturns, changes in inflation and interest rates, changes in trade policy, natural disasters, political crises, geopolitical events, or other macroeconomic conditions, which may in the future negatively impact our business and financial performance.
- Our ability to raise capital may be limited by applicable laws and regulations.
- We may sell additional equity or debt securities or enter into other arrangements to fund our operations, which may result in dilution to our stockholders and impose restrictions or limitations on our business.
- Sale of a substantial number of shares of our common stock in the public market could cause the market price of our common stock to drop significantly.
- We are early in our drug development efforts of our current drug candidates and all of our drug candidates are in clinical trials or preclinical development. If we are unable to successfully develop, receive regulatory approval for and commercialize our drug candidates, or successfully develop any other drug candidates, or experience significant delays in doing so, our business will be harmed.
- Our future success is dependent on the successful clinical development, regulatory approval and commercialization of our current and future drug candidates. If we, or our licensees or collaborators, are not able to obtain the required regulatory approvals, we, or our licensees or collaborators, will not be able to commercialize our drug candidates, and our ability to generate revenue will be adversely affected.
- Because the results of preclinical studies or earlier clinical trials are not necessarily predictive of future results, our drug candidates may not have favorable results in planned or future preclinical studies or clinical trials, or may not receive regulatory approval.
- Interim topline and preliminary results 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, which could result in material changes in the final data.
- Preclinical studies and clinical trials are very expensive, time-consuming and difficult to design and implement and involve uncertain outcomes. Further, we may encounter substantial delays in our clinical

trials or we may fail to demonstrate safety and efficacy in our preclinical studies and clinical trials to the satisfaction of applicable regulatory authorities.

- If we are not successful in discovering, developing and commercializing additional drug candidates, our ability to expand our business and achieve our strategic objectives would be impaired.
- Our drug candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial potential or result in significant negative consequences following any potential marketing approval.
- Even if our current or future drug candidates receive marketing approval, they may fail to achieve market acceptance by physicians, patients, third-party payors or others in the medical community necessary for commercial success.
- Our relationships with customers, physicians, and third-party payors may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws, and other healthcare laws and regulations. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.
- Coverage and adequate reimbursement may not be available for our current or any future drug candidates, which could make it difficult for us to sell profitably, if approved.
- If we are unable to obtain and maintain patent protection for our current or any future drug candidates, or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.
- We may be involved in lawsuits to protect or enforce our patents, the patents of our licensors or our other intellectual property rights, which could be expensive, time consuming and unsuccessful.
- We do not have our own manufacturing capabilities and will rely on third parties to produce clinical and commercial supplies of our current and any future drug candidates.
- We intend to rely on third parties to conduct, supervise and monitor our preclinical studies and clinical trials, and if those third parties perform in an unsatisfactory manner, it may harm our business.
- We may need to expand our organization, and we may experience difficulties in managing this growth, which could disrupt our operations.
- We may be subject to numerous and varying privacy and security laws, and our failure to comply could result in penalties and reputational damage.
- We have previously identified material weaknesses in our internal control over financial reporting. In the future, if we are unable to identify or remediate material weaknesses, or if we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial condition, results of operations or cash flows, which may adversely affect investor confidence in us and, as a result, the value of our common stock.
- If we fail to satisfy all applicable requirements of Nasdaq and it determines to delist our common stock, the delisting could adversely affect the market liquidity of our common stock and the market price of our common stock could decrease.

Risks Related to Our Financial Position and Need for Additional Capital

We will require additional capital to finance our operations, which may not be available on acceptable terms, if at all. Failure to obtain this necessary capital when needed will force us to delay, limit or terminate certain of our drug development efforts or other operations.

Our operations have consumed substantial amounts of cash since our inception. As of September 30, 2025, our cash, cash equivalents and marketable securities were $25.6 million and we had an accumulated deficit of $331.4 million. We believe that our cash, cash equivalents and marketable securities as of September 30, 2025, plus the initial proceeds from the Private Placement, will be sufficient to fund our operating and capital expenditure requirements for a period greater than 12 months from the date our unaudited interim condensed consolidated financial statements were available to be issued with our Quarterly Report on Form 10-Q. However, our operating plans may change because of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, third-party funding, marketing and distribution arrangements, as well as other collaborations, strategic alliances and licensing arrangements, or any combination of these approaches.

We expect our expenses to continue to increase in connection with our ongoing activities, particularly as we conduct clinical trials of, and seek marketing approval for, our product candidates and advance our other programs. Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is a very time-consuming, expensive and uncertain process that takes years to complete. Other unanticipated costs may also arise. Because the design and outcome of our ongoing and anticipated clinical trials are highly uncertain, we cannot reasonably estimate the actual amount of resources and funding that will be necessary to successfully complete the development and commercialization of any product candidate we develop.

We may never generate the necessary data or results required to obtain regulatory approval in order to generate revenue from product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we do not expect to be commercially available for several years, if at all. Adequate additional financing may not be available to us on acceptable terms, or at all. Our ability to raise additional capital may be adversely impacted by potential worsening global economic conditions, inflation expectations, and the recent disruptions to and volatility in the credit and financial markets in the United States and worldwide resulting from public health crises and geopolitical tensions.

There have been and may continue to be significant disruptions to the global financial markets and general global economic slowdown due to macroeconomic factors. If disruptions and slowdown deepen or persist, we may not be able to access additional capital on favorable terms, or at all, which could in the future negatively affect our financial condition and our ability to pursue our business strategy. If we are unable to raise additional capital when needed, we will be required to delay, limit, reduce or terminate our drug development or future commercialization efforts, or grant rights to develop and market drug candidates that we would otherwise develop and market ourselves. If we are unable to obtain adequate financing when needed, we will be required to implement additional cost reduction measures, such as further reducing operating expenses, or otherwise be required to delay, reduce the scope of or suspend one or more of our preclinical studies, clinical trials, research and development programs or commercialization efforts, or terminate our operations.

For additional details, see elsewhere in these Risk Factors, including *"Our business could be adversely affected by economic downturns, changes in inflation and interest rates, changes in trade policy (including tariffs), natural disasters, political crises, geopolitical events, or other macroeconomic conditions, which may in the future negatively impact our business and financial performance."*

We expect to continue to incur substantial operating losses for the foreseeable future and may never achieve or maintain profitability.

We have historically incurred significant operating losses. Our net loss for the quarter ended September 30, 2025 was $12.2 million and we had an accumulated deficit of $331.4 million as of that date. We expect to incur operating losses in the future. Since inception, we have devoted substantially all of our efforts to research and preclinical and clinical development of our drug candidates, as well as hiring employees and building our infrastructure.

We have no drugs approved for commercialization and have never generated any revenue from drug sales. Most of our drug candidates are still in the preclinical testing stage. It could be several years, if ever, before we have a commercialized drug. We expect to incur significant expenses and operating losses in the future, and the net losses we incur may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially if, and as, we:

- continue the ongoing and planned preclinical and clinical development of our drug candidates;
- continue to build a portfolio of drug candidates through the acquisition or in-license of drugs, drug candidates or technologies;
- initiate preclinical studies and clinical trials for any additional drug candidates that we may pursue in the future;
- seek marketing approvals for our current and future drug candidates that successfully complete clinical trials;
- establish a sales, marketing and distribution infrastructure to commercialize any drug candidate for which we may obtain marketing approval;
- develop, maintain, expand and protect our intellectual property portfolio;
- implement operational, financial and management systems; and
- attract, hire and retain additional administrative, clinical, regulatory and scientific personnel.

Even if we complete the development and regulatory processes described above, we anticipate incurring significant costs associated with launching and commercializing our current and future drug candidates.

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 would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations.

Our operating history may make it difficult to evaluate the success of our business to date and to assess our future viability.

Our operations have consumed substantial amounts of cash since our inception, primarily due to research and development of our drug candidates, organizing and staffing our company, business planning, raising capital, and acquiring assets. We have not yet demonstrated the ability to obtain marketing approvals, manufacture a commercial-scale drug or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we had more experience developing drug candidates.

We expect our financial condition and operating results to continue to fluctuate from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. We will need to eventually transition from a company with a research and development focus to a company capable of undertaking commercial activities. We may encounter unforeseen expenses, difficulties, complications and delays and may not be successful in such a transition.

Our business could be adversely affected by economic downturns, changes in inflation and interest rates, changes in trade policy (including tariffs), natural disasters, political crises, geopolitical events, or other macroeconomic conditions, which may in the future negatively impact our business and financial performance.

The global economy, including credit and financial markets, has experienced extreme volatility and disruptions, including, among other things, severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, supply chain shortages, fluctuations in inflation and interest rates, and uncertainty about economic stability. For example, during 2022 and 2023, the Federal Reserve raised interest rates multiple times in response to concerns about inflation. Higher interest rates, coupled with reduced government spending, tariffs, and volatility in financial markets may increase economic uncertainty and affect consumer spending. Similarly, geopolitical tensions including between China and Taiwan and the wars involving Ukraine and Israel have created volatility in the global capital markets and are expected to have further global economic consequences, including disruptions of the global supply chain and energy markets. Increased inflation may result in increased operating costs (including labor costs) and may affect our operating budgets. In addition, the U.S. Federal Reserve has raised interest rates in response to concerns about inflation.

Trade disputes, tariffs, restrictions and other political tensions between the U.S. and other countries may also exacerbate unfavorable macroeconomic conditions, including inflationary pressures, foreign exchange volatility, financial market instability, and economic recessions or downturns, which may also limit our access to capital, or otherwise negatively impact our business and operations. The U.S. government has enacted, and continues to enact, a series of new tariffs, including a tariff on all imports and additional “reciprocal” tariffs targeting imports from specified countries. Additionally, current or future tariffs will result in increased research and development expenses, including with respect to increased costs associated with APIs, raw materials, laboratory equipment and research materials and components. In addition, such tariffs will increase our supply chain complexity and could also potentially disrupt our existing supply chain. Trade policies, geopolitical disputes and other trade restrictions affecting the import of materials necessary for clinical trials could result in delays to our development timelines. With the current administration in the U.S., additional and higher tariffs and sanctions may be imposed on goods imported from other countries which could increase the cost of goods needed to commercialize our products and continue development of our product candidates. Further, such actions by the U.S. could result in retaliatory action by those countries which could impact our ability to profitably commercialize our products in those jurisdictions. As a result, our business, operations and financial condition could be materially harmed.

Any such volatility and disruptions may adversely affect our business or the third parties on whom we rely. If the equity and credit markets deteriorate, including as a result of political unrest or war, it may make any necessary debt or equity financing more difficult to obtain in a timely manner or on favorable terms, more costly or more dilutive. Increased inflation rates can adversely affect us by increasing our costs, including labor and employee benefit costs.

The ultimate impact of current or future tariffs and trade restrictions remains uncertain and could materially and adversely affect our business, financial condition, and prospects. While we actively monitor these risks, any prolonged economic downturn, escalation in trade tensions, or deterioration in international perception of U.S.-based companies could materially and adversely affect our business, ability to access the capital markets or other financing sources, results of operations, financial condition and prospects. In addition, tariffs and other trade developments have and may continue to heighten the risks related to the other risk factors described elsewhere in this Quarterly Report on Form 10-Q.

Our ability to raise capital may be limited by applicable laws and regulations.

Using a shelf registration statement on Form S-3 to raise additional capital generally takes less time and is less expensive than other means, such as conducting an offering under a Form S-1 registration statement. However, our ability to raise capital using a shelf registration statement may be limited by, among other things, SEC rules and regulations. Under SEC rules and regulations, if our public float (the market value of our common stock held by non-affiliates) is less than $75.0 million, then the aggregate market value of securities sold by us or on our behalf under our Form S-3 in any 12-month period is limited to an aggregate of one-third of our public float. As our public float is currently less than $75.0 million, we are currently subject to this limitation. If our ability to utilize a Form S-3 registration statement for a primary offering of our securities continues to be limited to one-third of our public float, we may need to conduct such an offering pursuant to an exemption from registration under the Securities Act or under a Form S-1 registration statement, which would increase the cost of raising additional capital relative to utilizing a Form S-3 registration statement.

We may sell additional equity or debt securities or enter into other arrangements to fund our operations, which may result in dilution to our stockholders and impose restrictions or limitations on our business.

Until such time as we can generate substantial revenue from drug sales, if ever, we expect to finance our cash needs through a combination of equity and debt financings, strategic alliances, and license and development agreements in connection with any collaborations. We do not have any committed external source of funds. To the extent that we issue additional equity securities, our stockholders may experience substantial dilution, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our stockholders. In addition, we may issue equity or debt securities as consideration for obtaining rights to additional compounds.

Debt and equity financings, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as redeeming our shares, making investments, issuing additional equity, incurring additional debt, making capital expenditures, declaring dividends or placing limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could negatively impact our ability to conduct our business. If we raise additional capital through future collaborations, strategic alliances or third-party licensing arrangements, we may have to relinquish valuable rights to our intellectual property, future revenue streams, research programs or drug candidates, or grant licenses on terms that may not be favorable to us. Any of these events could significantly harm our business, financial condition and prospects.

Sales of a substantial number of shares of our common stock in the public market could cause the market price of our common stock to drop significantly.

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 intend to sell shares, could reduce the market price of our common stock. Some of the holders of our securities have rights, subject to certain conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. Registration of these shares would result in the shares becoming freely tradable without restriction under the Securities Act except for shares held by our affiliates. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

Risks Related to the Development and Commercialization of Our Drug Candidates

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

We are early in our drug development efforts. In order to commercialize any product that achieves regulatory approval, we will need to build a commercial organization or successfully outsource commercialization, all of which will require substantial investment and significant marketing efforts before we have the ability to generate any revenue from drug sales. We do not have any drugs that are approved for commercial sale, and we may never be able to develop or commercialize marketable drugs.

Our ability to generate revenue from drug sales and achieve profitability depends on our ability, alone or with any current or future collaborative partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, our current and future drug candidates. We do not anticipate generating revenue from drug sales for the next several years, if ever. Our ability to generate revenue from drug sales depends heavily on our, or any current or future collaborators', success in the following areas, including but not limited to:

- timely and successfully completing preclinical and clinical development of our current and future drug candidates;

- obtaining regulatory approvals for our current and future drug candidates for which we successfully complete clinical trials;
- launching and commercializing any drug candidates for which we obtain regulatory approval by establishing a sales force, marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- qualifying for coverage and adequate reimbursement by government and third-party payors for any drug candidates for which we obtain regulatory approval, both in the United States and internationally;
- developing, validating and maintaining a commercially viable, sustainable, scalable, reproducible and transferable manufacturing process for our current and future drug candidates that is compliant with cGMP;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide an adequate amount and quality of drugs and services to support clinical development, as well as the market demand for our current and future drug candidates, if approved;
- obtaining market acceptance, if and when approved, of our current or any future drug candidates as a viable treatment option by physicians, patients, third-party payors and others in the medical community;
- effectively addressing any competing technological and market developments;
- implementing additional internal systems and infrastructure, as needed;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter and performing our obligations pursuant to such arrangements;
- obtaining and maintaining orphan drug exclusivity for any of our current and future drug candidates for which we obtain regulatory approval;
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how;
- avoiding and defending against third-party interference or infringement claims; and
- securing appropriate pricing in the United States, the European Union and other regions.

If we are not successful with respect to one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize the drug candidates we develop, which would materially harm our business. If we do not receive marketing approvals for any drug candidate we develop, we may not be able to continue our operations.

Our future success is dependent on the successful clinical development, regulatory approval and commercialization of our current and future drug candidates. If we, or our licensees or collaborators, are not able to obtain the required regulatory approvals, we, or our licensees or collaborators, will not be able to commercialize our drug candidates, and our ability to generate revenue will be adversely affected.

We do not have any drugs that have received regulatory approval. Our business is dependent on our ability to successfully complete preclinical and clinical development of, obtain regulatory approval for, and, if approved, successfully commercialize our current and future drug candidates in a timely manner. Activities associated with the development and commercialization of our current and future drug candidates are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and similar regulatory authorities outside the United States. Failure to obtain regulatory approval in the United States or other jurisdictions would prevent us from commercializing and marketing our current and future drug candidates. An inability to effectively develop and commercialize our current and future drug candidates could have an adverse effect on our business, financial condition, results of operations and growth prospects.

Further, activities associated with the development and commercialization of our current and future drug candidates are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and similar regulatory authorities outside the United States. Failure to obtain regulatory approval in the United States or other jurisdictions would prevent us from commercializing and marketing our current and future drug candidates.

Even if we obtain approval from the FDA and comparable foreign regulatory authorities for our current and future drug candidates, any approval might contain significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, or may be subject to burdensome post-approval study or risk management requirements. If we are unable to obtain regulatory approval, or any approval contains significant limitations, we may not

be able to obtain sufficient funding or generate sufficient revenue to continue the development of that drug candidate or any other drug candidate that we may in-license, develop or acquire in the future. In certain circumstances, our third-party licensees or collaborators are responsible for obtaining regulatory approvals in the countries covered by the license, and we are dependent on their efforts in order to achieve the necessary approvals in order to commercialize our products. If any future licensees or collaborators fail to perform their obligations to develop and obtain regulatory approvals for the licensed products, we may not be able to commercialize our products in the affected countries, or our ability to do so may be substantially delayed.

Furthermore, even if we obtain regulatory approval for our current and future drug candidates, we will still need to develop a commercial organization, establish a commercially viable pricing structure and obtain approval for adequate reimbursement from third-party and government payors. If we are unable to successfully commercialize our current and future drug candidates, we may not be able to generate sufficient revenue to continue our business.

Because the results of preclinical studies or earlier clinical trials are not necessarily predictive of future results, our drug candidates may not have favorable results in planned or future preclinical studies or clinical trials, or may not receive regulatory approval.

Success in preclinical testing and early clinical trials does not ensure that subsequent clinical trials will generate similar results or otherwise provide adequate data to demonstrate the efficacy and safety of a drug candidate. Frequently, drug candidates that have shown promising results in early clinical trials have subsequently suffered significant setbacks in later clinical trials. The results from preclinical studies of our current and future drug candidates may not be predictive of the effects of these compounds in later stage clinical trials. For example, we co-developed soticlestat with Takeda Company Limited ("Takeda") through completion of Phase 2 clinical trials and subsequently sold our rights back to Takeda under a royalty, license and termination agreement ("RLT Agreement"). Although we successfully completed Phase 1b/2a and Phase 2 clinical trials of soticlestat, in June 2024, Takeda reported that soticlestat failed to meet its primary endpoints in two Phase 3 trials evaluating soticlestat for the treatment of Dravet and Lennox-Gastaut syndromes and, in January 2025, Takeda announced discontinuation of the program. If we do not observe favorable results in clinical trials of one of our drug candidates, we may decide to delay or abandon clinical development of that drug candidate. Any such delay or abandonment could harm our business, financial condition, results of operations and prospects.

Interim topline and preliminary results 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, which could result in material changes in the final data.

From time to time, we have and may in the future publish or report preliminary or interim data from our clinical trials. Preliminary or interim data from our clinical trials and those of our partners may not be indicative of the final results of the trial and are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and/or more patient data become available. Preliminary or topline results also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published or reported. As a result, preliminary or interim data should be considered carefully and with caution until final data are available. Differences between preliminary or interim data and final data could significantly harm our business prospects and may cause the trading price of our common stock to fluctuate significantly.

Preclinical studies and clinical trials are very expensive, time-consuming and difficult to design and implement and involve uncertain outcomes. Further, we may encounter substantial delays in our clinical trials or we may fail to demonstrate safety and efficacy in our preclinical studies and clinical trials to the satisfaction of applicable regulatory authorities.

All of our current drug candidates are in early clinical or preclinical development and their risk of failure is high. We must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that each of our drug candidates are safe and effective for its intended indications before we are prepared to submit an NDA or BLA for regulatory approval. We cannot predict with any certainty if or when we might submit an NDA or BLA for any of our product candidates or whether any such application will be approved by the FDA. Human clinical trials are very expensive and difficult to design and implement, in part because they are subject to rigorous review and regulatory requirements by numerous government authorities in the United States and in other countries where we intend to test and market our product candidates. For instance, the FDA or similar regulatory authorities in other countries may not agree with our proposed endpoints for any future clinical trial of our product candidates, which may delay the commencement of such clinical trial.

We estimate that the successful completion of clinical trials of our product candidates will take at least several years to complete, if not longer or at all. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. Furthermore, failure can occur at any stage and we could encounter problems that cause us

to abandon or repeat clinical trials. Events that may prevent successful or timely completion of clinical development include:

- our inability to generate sufficient preclinical, toxicology or other data to support the initiation of clinical trials;
- our inability to develop and validate disease-relevant clinical endpoints;
- delays in reaching a consensus with regulatory authorities on trial design;
- delays in reaching agreement on acceptable terms with prospective clinical research organizations ("CROs") and clinical trial sites;
- delays in opening investigational sites;
- delays or difficulty in recruiting and enrollment of suitable patients to participate in our clinical trials;
- imposition of a clinical hold by regulatory authorities because of a serious adverse event, concerns with a class of drug candidates or after an inspection of our clinical trial operations or trial sites;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- occurrence of serious adverse events associated with the drug candidate that are viewed to outweigh its potential benefits;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols; or
- business interruptions resulting from global geopolitical tensions, including tensions between China and Taiwan and the ongoing wars involving Ukraine and Israel and any other war or the perception that hostilities may be imminent, terrorism, natural disasters or public health crises.

Further, clinical endpoints for certain diseases we are targeting, including brain conditions with significant unmet need, have not been established, and accordingly, we may have to develop new modalities or modify existing endpoints to measure efficacy, which may increase the time it takes for us to commence or complete clinical trials. In addition, we believe investigators in this area may be inexperienced in conducting trials in this area due to the current lack of drugs to treat these disorders, which may result in increased time and expense to train investigators and open clinical sites.

Any inability to successfully complete preclinical and clinical development could result in additional costs to us or impair our ability to generate revenue from future drug sales and regulatory and commercialization milestones. In addition, if we make manufacturing or formulation changes to our drug candidates, we may need to conduct additional testing to bridge our modified drug candidate to earlier versions. Clinical trial delays could also shorten any periods during which we may have the exclusive right to commercialize our drug candidates, if approved, or allow our competitors to bring comparable drugs to market before we do, which could impair our ability to successfully commercialize our drug candidates and may harm our business, financial condition, results of operations and prospects.

Additionally, if the results of our clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with our drug candidates, we may:

- be delayed in obtaining marketing approval, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to additional post-marketing testing requirements;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the drug 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.

Our drug development costs will also increase if we experience delays in testing or obtaining marketing approvals. We do not know whether any of our preclinical studies or clinical trials will begin as planned, need to be restructured or be completed on schedule, if at all.

Further, we, the FDA or an IRB may suspend our clinical trials at any time if it appears that we or our collaborators are failing to conduct a trial in accordance with regulatory requirements, including the FDA's current Good Clinical Practice ("GCP") regulations, that we are exposing participants to unacceptable health risks, or if the FDA finds deficiencies in our IND applications or the conduct of these trials. Therefore, we cannot predict with any certainty the schedule for commencement and completion of future clinical trials. If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of our drug candidates could be negatively impacted, and our ability to generate revenues from our drug candidates may be delayed.

If we are not successful in discovering, developing and commercializing additional drug candidates, our ability to expand our business and achieve our strategic objectives would be impaired.

A key element of our current strategy is to discover, develop and potentially commercialize a portfolio of drug candidates to treat certain brain conditions with significant unmet need. However, our business development activities and research activities may present attractive opportunities outside of our current areas of focus and we may choose to pursue drug candidates in other areas of interest including other disorders that we believe would be in the best interest of the Company and our stockholders. We plan to continuously review our strategies and modify as necessary based on attractive areas of interest and assets that we choose to pursue. We intend to develop our portfolio of drug candidates by in-licensing and entering into collaborations with biopharmaceutical companies or academic institutions for new drug candidates. Identifying new drug candidates requires substantial technical, financial and human resources, whether or not any drug candidates are ultimately identified. Even if we identify drug candidates that initially show promise, we may fail to in-license or acquire these assets and may also fail to successfully develop and commercialize such drug candidates for many reasons, including the following:

- the research methodology used may not be successful in identifying potential drug candidates;
- competitors may develop alternatives that render any drug candidate we develop obsolete;
- any drug candidate we develop may nevertheless be covered by third parties' patents or other exclusive rights;
- a drug candidate may, on further study, be shown to have harmful side effects or other characteristics that indicate it is unlikely to be effective or otherwise does not meet applicable regulatory criteria;
- a drug candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a drug candidate may not be accepted as safe and effective by physicians, patients, the medical community or third-party payors, even if approved.

We have limited financial and management resources and, as a result, we may forego or delay the pursuit of opportunities with other drug candidates or for other indications that later prove to have greater market potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial drugs or profitable market opportunities. 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 circumstances under which it would have been more advantageous for us to retain sole development and commercialization rights to such drug candidate.

If we are unsuccessful in identifying and developing additional drug candidates or are unable to do so, our key growth strategy and business will be harmed.

Enrollment and retention of patients in clinical trials is an expensive and time-consuming process and could be made more difficult or rendered impossible by multiple factors outside our control.

We focus our research and drug development on treatments for brain conditions with significant unmet need. This specifically includes medicines to treat symptoms of excess neural excitation such as seizures and psychoses that manifest in specific epilepsies, psychiatric, neurodegenerative and neurodevelopmental conditions. We are developing medicines for rare and broader conditions. Identifying and qualifying patients to participate in our clinical trials is critical to our success. The number of patients suffering from the disorders that we are targeting is small and has not been established with precision. If the actual number of patients with these disorders is smaller than we anticipate, we may encounter difficulties in enrolling patients in our clinical trials, thereby delaying or preventing development and approval of our drug candidates. Even once enrolled we may be unable to retain a sufficient number of patients to complete any of our trials. Patient

enrollment and retention in clinical trials depends on many factors, including the size of the patient population, the nature of the trial protocol, the existing body of safety and efficacy data, the number and nature of competing treatments and ongoing clinical trials of competing therapies for the same indication, the proximity of patients to clinical sites and the eligibility criteria for the trial, any such enrollment issues could cause delays or prevent development and approval of our drug candidates. Because we are focused on addressing rare neurological disorders, there are limited patient pools from which to draw in order to complete our clinical trials in a timely and cost-effective manner. Furthermore, our efforts to build relationships with patient communities may not succeed, which could result in delays in patient enrollment in our clinical trials. In addition, any negative results we may report in clinical trials of our drug candidate may make it difficult or impossible to recruit and retain patients in other clinical trials of that same drug candidate. Delays or failures in planned patient enrollment or retention may result in increased costs, program delays or both, which could have a harmful effect on our ability to develop our drug candidates, or could render further development impossible.

Our drug candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial potential or result in significant negative consequences following any potential marketing approval.

During the conduct of clinical trials, patients report changes in their health, including illnesses, injuries and discomforts, to their doctor. Often, it is not possible to determine whether or not the drug candidate being studied caused these conditions. Regulatory authorities may draw different conclusions or require additional testing to confirm these determinations, if they occur. In addition, it is possible that illnesses, injuries, discomforts or other adverse events that were observed in earlier trials, as well as conditions that did not occur or went undetected in previous trials, will be reported by subjects as we test our drug candidates in larger, longer and more extensive clinical programs, or, as use of these drug candidates becomes more widespread if they receive regulatory approval. Many times, side effects are only detectable after investigational drugs are tested in large-scale, Phase 3 trials or, in some cases, after they are made available to patients on a commercial scale after approval. If clinical experience indicates that any of our drug candidates causes adverse events or serious or life-threatening adverse events, the development of that drug candidate may fail or be delayed, or, if the drug candidate has received regulatory approval, such approval may be revoked, which would harm our business, prospects, operating results and financial condition.

Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of our drug candidates, the commercial prospects of our drug candidates may be harmed and our ability to generate revenue through their sale may be delayed or eliminated. Any of these occurrences may harm our business, financial condition and prospects significantly.

Additionally, if any of our drug candidates receive marketing approval, the FDA could require us to include a black box warning in our label or adopt REMS to ensure that the benefits outweigh its risks, which may include, among other things, a medication guide outlining the risks of the drug for distribution to patients and a communication plan to health care practitioners. Furthermore, if we or others later identify undesirable side effects caused by our drug candidates, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such drug candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a drug candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients;
- we may need to conduct a recall; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of our drug candidates and could significantly harm our business, prospects, financial condition and results of operations.

If the market opportunities for our drug candidates are smaller than we believe they are, even assuming approval of a drug candidate, our business may suffer. Because the patient populations in the market for our drug candidates may be small and difficult to assess, we must be able to successfully identify patients and acquire a significant market share to achieve profitability and growth.

We focus our research and drug development on treatments for brain conditions with significant unmet need. This specifically includes medicines to treat symptoms of excess neural excitation such as seizures and psychoses that manifest in specific epilepsies, psychiatric, neurodegenerative and neurodevelopmental conditions. We are developing medicines for both rare and broader conditions. As a result of the disorders that we are targeting for drug development, our eligible patient population and pricing estimates may differ significantly from the actual market addressable by our drug

candidates. Our projections of both the number of people who have these disorders, as well as the subset of people with these disorders who have the potential to benefit from treatment with our drug candidates, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including scientific literature, patient foundations, or market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these disorders. The number of patients may turn out to be lower than expected. Likewise, the potentially addressable patient population for each of our drug candidates may be limited or may not be amenable to treatment with our drug candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business.

We face substantial competition, which may result in others developing or commercializing drugs before or more successfully than us.

The development and commercialization of new drugs is highly competitive. We face competition with respect to our current drug candidates and will face competition with respect to any other drug candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell drugs or are pursuing the development of drug candidates for the treatment of the indications that we are pursuing. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

More established companies may have a competitive advantage over us due to their greater size, resources and institutional experience. In particular, these companies have greater experience and expertise in securing collaboration or partnering relationships, reimbursement, government contracts, relationships with key opinion leaders, conducting testing and clinical trials, obtaining and maintaining regulatory approvals and distribution relationships to market products, and marketing approved drugs. These companies also have significantly greater research and marketing capabilities than we do. If we are not able to compete effectively against existing and potential competitors, our business and financial condition may be harmed.

As a result of these factors, our competitors may obtain regulatory approval of their drugs before we are able to, which may limit our ability to develop or commercialize our drug candidates. Our competitors may also develop therapies that are safer, more effective, more widely accepted and cheaper than ours, and may also be more successful than us in manufacturing and marketing their drugs. These appreciable advantages could render our drug candidates obsolete or non-competitive before we can recover the expenses of such drug candidates' development and commercialization.

Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific, management and commercial personnel, establishing clinical trial sites and subject registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Even if our current or future drug candidates receive marketing approval, they may fail to achieve market acceptance by physicians, patients, third-party payors or others in the medical community necessary for commercial success.

Even if our current or future drug candidates receive marketing approval, they may fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If they do not achieve an adequate level of acceptance, we may not generate significant drug revenue and may not become profitable. The degree of market acceptance of our current or future drug candidates, if approved for commercial sale, will depend on a number of factors, including but not limited to:

- the efficacy and potential advantages compared to alternative treatments and therapies;
- the safety profile of our drug candidate compared to alternative treatments and therapies;
- effectiveness of sales and marketing efforts;
- the strength of our relationships with patient communities;
- the cost of treatment in relation to alternative treatments and therapies, including any similar generic treatments;
- our ability to offer such drug for sale at competitive prices;
- the convenience and ease of administration compared to alternative treatments and therapies;

- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- the availability of third-party coverage and adequate reimbursement;
- the prevalence and severity of any side effects; and
- any restrictions on the use of the drug together with other medications.

Our efforts to educate physicians, patients, third-party payors and others in the medical community on the benefits of our drug candidates may require significant resources and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of our drug candidates. Because we expect sales of our drug candidates, if approved, to generate substantially all of our drug revenues for the foreseeable future, the failure of our drugs to find market acceptance would harm our business and could require us to seek additional financing.

Even if we obtain and maintain approval for our current or future drug candidates from the FDA, we may never obtain approval for our current or future drug candidates outside of the United States, which would limit our market opportunities and could harm our business.

Approval of a drug candidate in the United States by the FDA does not ensure approval of such drug candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Sales of our current and future drug candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a drug candidate, comparable regulatory authorities of foreign countries also must approve the manufacturing and marketing of the drug candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, which may require additional preclinical studies or clinical trials. In many countries outside the United States, a drug candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for any drug candidates, if approved, is also subject to approval. Obtaining approval for our current and future drug candidates in the European Union from the European Commission following the opinion of the European Medicines Agency, if we choose to submit a marketing authorization application there, would be a lengthy and expensive process. The FDA and comparable foreign regulatory authorities have the ability to limit the indications for which the drug may be marketed, require extensive warnings on the drug labeling or require expensive and time-consuming additional clinical trials or reporting as conditions of approval. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our current and future drug candidates in certain countries. In certain cases, we are dependent on third parties to obtain such foreign regulatory approvals, and any delay or failure of performance of such third parties could delay or prevent our ability to commercialize our products in the affected countries.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Also, regulatory approval for our drug candidates may be withdrawn. If we fail to comply with the regulatory requirements, our target market will be reduced and our ability to realize the full market potential of our current and future drug candidates will be harmed and our business, financial condition, results of operations and prospects could be harmed.

If we seek approval to commercialize our current or future drug candidates outside of the United States, a variety of risks associated with international operations could harm our business.

If we seek approval of our current or future drug candidates outside of the United States, we expect that we will be subject to additional risks in commercialization including:

- different regulatory requirements for approval of therapies in foreign countries;
- reduced protection for intellectual property rights;
- the potential requirement of additional clinical studies in international jurisdictions;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;

- foreign reimbursement, pricing and insurance regimes;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical tensions, including tensions between China and Taiwan and the ongoing wars involving Ukraine and Israel and any other war or the perception that hostilities may be imminent, terrorism, natural disasters or public health crises.

We have no prior experience in these areas. In addition, there are complex regulatory, tax, labor and other legal requirements imposed by many of the individual countries in and outside of Europe with which we will need to comply. Many biopharmaceutical companies have found the process of marketing their own products in foreign countries to be very challenging.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit commercialization of any drug candidate that we may develop.

We face an inherent risk of product liability exposure related to the testing of our current and any future drug candidates in clinical trials and may face an even greater risk if we commercialize any drug candidate that we may develop. If we cannot successfully defend ourselves against claims that any such drug candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any drug candidate that we may develop;
- loss of revenue;
- substantial monetary awards to trial participants or patients;
- significant time and costs to defend the related litigation;
- withdrawal of clinical trial participants;
- the inability to commercialize any drug candidate that we may develop; and
- injury to our reputation and significant negative media attention.

Although we maintain product liability insurance coverage, such insurance may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage each time we commence a clinical trial and if we successfully commercialize any drug candidate. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

Risks Related to Licensing and Collaboration Arrangements

We may be required to relinquish important rights to and control over the development and commercialization of our drug candidates to any future collaborators.

Our current and future collaborations could subject us to a number of risks, including:

- we may be required to undertake the expenditure of substantial operational, financial and management resources;
- we may be required to issue equity securities that would dilute our stockholders' percentage of ownership;
- we may be required to assume substantial actual or contingent liabilities;
- we may not be able to control the amount and timing of resources that our strategic collaborators devote to the development or commercialization of our drug candidates;
- strategic collaborators may delay clinical trials, provide insufficient funding, terminate a clinical trial or abandon a drug candidate, repeat or conduct new clinical trials or require a new version of a drug candidate for clinical testing;
- strategic collaborators may not pursue further development and commercialization of products resulting from the strategic collaboration arrangement or may elect to discontinue research and development programs;

- strategic collaborators may not commit adequate resources to the marketing and distribution of our drug candidates, limiting our potential revenues from these products;
- we rely on our current collaborators to manufacture drug substance and drug product and may do so with respect to future collaborators, which could result in disputes or delays;
- disputes may arise between us and our strategic collaborators that result in 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's attention and consumes resources;
- disputes may arise between us and our current or future collaborators regarding any termination of any collaboration, license, or other business development arrangement in which we may enter;
- strategic collaborators may experience financial difficulties;
- strategic collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in a manner that could jeopardize or invalidate our proprietary information or expose us to potential litigation;
- business combinations or significant changes in a strategic collaborator's business strategy may also adversely affect a strategic collaborator's willingness or ability to complete its obligations under any arrangement;
- strategic collaborators could decide to move forward with a competing drug candidate developed either independently or in collaboration with others, including our competitors; and
- strategic collaborators could terminate the arrangement or allow it to expire, which would delay the development and may increase the cost of developing our drug candidates.

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.

Our business plan is to continue to evaluate various acquisitions and strategic partnerships, including licensing or acquiring complementary drugs, intellectual property rights, technologies, or businesses. Any potential acquisition or strategic partnership may entail numerous risks, including:

- increased operating expenses and cash requirements;
- the assumption of additional indebtedness or contingent liabilities;
- assimilation of operations, intellectual property and drugs of an acquired company, including difficulties associated with integrating new personnel;
- the diversion of our management's attention from our existing drug programs and initiatives in pursuing such a strategic partnership, 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 drugs or drug candidates and regulatory approvals;
- our inability to generate revenue from acquired technology and/or drugs sufficient to meet our objectives in undertaking the acquisition or even to offset the associated acquisition and maintenance costs;
- challenges related to integrating acquired businesses or entering into or realizing the benefits of strategic transactions generally; and
- risks associated with potential international acquisition transactions, including in countries where we do not currently have a material presence.

In addition, if we engage in future acquisitions or strategic partnerships, 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. Moreover, we may not be able to locate suitable acquisition opportunities and this inability could impair our ability to grow or obtain access to technology or drugs that may be important to the development of our business.

We may explore additional strategic collaborations that may never materialize or may fail.

Our business strategy is based on acquiring or in-licensing compounds directed at certain epilepsies, seizure-related disorders, and rare neurological disorders. As a result, we intend to periodically explore a variety of possible additional strategic collaborations in an effort to gain access to additional drug candidates or resources. At the current time, we cannot predict what form such a strategic collaboration might take. We are likely to face significant competition in seeking appropriate strategic collaborators, and strategic collaborations can be complicated and time consuming to negotiate and document. We may not be able to negotiate strategic collaborations on acceptable terms, or at all. We are unable to predict when, if ever, we will enter into any additional strategic collaborations because of the numerous risks and uncertainties associated with establishing them, and we cannot predict the success of any collaborations that we enter into. We may enter into strategic collaborations that we subsequently no longer wish to pursue.

If our strategic collaborations do not result in the successful development and commercialization of products, or if one of our collaborators or licensees terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration or license, as appropriate. If we do not receive the potential funding under these agreements, our development of product candidates could be delayed, and we may need additional resources to develop product candidates. For example, in March 2021, we entered into the RLT Agreement, pursuant to which Takeda secured rights to our global share in soticlestat and had sole discretion over the conduct of the development and commercialization of soticlestat. In June 2024, Takeda reported that soticlestat failed to meet its primary endpoints in two Phase 3 trials evaluating soticlestat for the treatment of Dravet and Lennox-Gastaut syndromes and, in January 2025, Takeda announced discontinuation of the program. In addition, if one of our collaborators or licensees terminates its agreement with us, we may find it more difficult to find a suitable replacement collaborator or licensee or attract new collaborators or licensees, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected.

Risks Related to Regulatory Compliance

Our relationships with customers, physicians, and third-party payors may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws, and other healthcare laws and regulations. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

Healthcare providers and third-party payors in the United States and elsewhere will 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, principal investigators, consultants, customers and third-party payors may subject us to various federal and state fraud and abuse laws and other healthcare laws, including, without limitation, the federal Anti-Kickback Statute, the federal civil and criminal false claims laws and the law commonly referred to as the Physician Payments Sunshine Act and regulations. These laws will impact, among other things, our clinical research, proposed sales, marketing and educational programs. In addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct or may conduct our business. The laws that will affect our operations include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons or entities 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, in return for the purchase, recommendation, leasing or furnishing of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers and formulary managers on the other. The Patient Protection and Affordable Care Act, as amended ("PPACA"), amended the intent requirement of the federal Anti-Kickback Statute. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation;
- federal civil and criminal false claims laws, including, without limitation, the False Claims Act, and civil monetary penalty laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid or other government payors that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. The PPACA provides, and recent government cases against pharmaceutical and medical device manufacturers support, the view that federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, may implicate the False Claims Act;

- HIPAA, which created additional federal criminal statutes that prohibit a person from knowingly and willfully executing a scheme or making false or fraudulent statements to defraud any healthcare benefit program, regardless of the payor (e.g., public or private);
- HIPAA, as amended by HITECH, and their implementing regulations, and as amended again by the final HIPAA omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach Notification Rules Under HITECH and the Genetic Information Nondiscrimination Act, published in January 2013, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, healthcare clearinghouses and certain healthcare providers, known as covered entities, and their respective business associates, individuals or entities that perform certain services on behalf of a covered entity that involves the use or disclosure of individually identifiable health information and their subcontractors that use, disclose or otherwise process individually identifiable health information;
- Physician Payments Sunshine Act, which is part of the PPACA, requires that certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, information related to: (i) payments or other "transfers of value" made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other healthcare professionals (such as physician assistants and nurse practitioners), and teaching hospitals; and (ii) ownership and investment interests held by physicians and their immediate family members;
- state and foreign law equivalents of each of the above federal laws, state laws that require manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures and/or information regarding drug pricing, state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or to adopt compliance programs as prescribed by state laws and regulations, or that otherwise restrict payments that may be made to healthcare providers, state laws and regulations that require drug manufacturers to file reports relating to drug pricing and marketing information, and state and local laws that require the registration of pharmaceutical sales representatives; and
- state and foreign laws that govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws.

It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from participation in government funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws and the curtailment or restructuring of our operations.

The risk of our being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. The shifting compliance environment and the need to build and maintain robust and expandable systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the possibility that a healthcare company may run afoul of one or more of the requirements.

Coverage and adequate reimbursement may not be available for our current or any future drug candidates, which could make it difficult for us to sell profitably, if approved.

Market acceptance and sales of any drug candidates that we commercialize, if approved, will depend in part on the extent to which coverage and adequate reimbursement for these drugs and related treatments will be available from third-

party payors, including government health administration authorities, managed care organizations and other private health insurers. Third-party payors decide which therapies they will pay for and establish reimbursement levels. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own coverage and reimbursement policies. However, decisions regarding the extent of coverage and amount of reimbursement to be provided for any drug candidates that we develop will be made on a payor-by-payor basis. One third-party payor's determination to provide coverage for a drug does not assure that other payors will also provide coverage, and adequate reimbursement, for the drug. Additionally, a third-party payor's decision to provide coverage for a therapy does not imply that an adequate reimbursement rate will be approved. Each third-party payor determines whether or not it will provide coverage for a therapy, what amount it will pay the manufacturer for the therapy, and on what tier of its formulary it will be placed. The position on a third-party payor's list of covered drugs, or formulary, generally determines the co-payment that a patient will need to make to obtain the therapy and can strongly influence the adoption of such therapy by patients and physicians. Patients who are prescribed treatments for their conditions and providers prescribing such services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Patients are unlikely to use our drugs unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our drugs.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. We cannot be sure that coverage and reimbursement will be available for any drug that we commercialize and, if reimbursement is available, what the level of reimbursement will be. Inadequate coverage and reimbursement may impact the demand for, or the price of, any drug for which we obtain marketing approval. If coverage and adequate reimbursement are not available, or are available only to limited levels, we may not be able to successfully commercialize our current and any future drug candidates that we develop. Further, coverage policies and third-party payor reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained, less favorable coverage policies and reimbursement rates may be implemented in the future.

Healthcare legislative reform measures may have a negative impact on our business and results of operations.

In the United States and some foreign jurisdictions, there have been, and continue to be, several legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of drug candidates, restrict or regulate post-approval activities, and affect our ability to profitably sell any drug candidates for which we obtain marketing approval.

Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and/or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. In March 2010, the PPACA was passed, which substantially changed the way healthcare is financed by both the government and private insurers, and significantly impacts the U.S. pharmaceutical industry.

There have been amendments to and executive, judicial, congressional and executive branch challenges to certain aspects of the PPACA. For example, on August 16, 2022, the Inflation Reduction Act of 2022 ("the IRA") was signed into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in PPACA 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 and by creating a new manufacturer discount program. It is possible that the PPACA will be subject to judicial or congressional challenges in the future. It is unclear how any such challenges and the healthcare reform measures of the current administration will impact the PPACA and our business.

Other legislative changes have been proposed and adopted since the PPACA was enacted. For example, on July 4, 2025, the annual reconciliation bill, the "One Big Beautiful Bill Act" ("OBBBA"), was signed into law which is expected to reduce Medicaid spending and enrollment by implementing work requirements for some beneficiaries, capping state-directed payments, reducing federal funding, and limiting provider taxes used to fund the program. OBBBA also narrows access to PPACA marketplace exchange enrollment and declines to extend the PPACA enhanced advanced premium tax credits, set to expire at the end of 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. Further 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 due to subsequent legislative amendments to the statute will remain in effect until 2032 unless additional congressional action is taken.

Additional changes that may affect our business include the expansion of new programs such as Medicare payment for performance initiatives for physicians under the Medicare Access and CHIP Reauthorization Act of 2015 ("MACRA"), which ended the use of the statutory formula and established a quality payment program, also referred to as the Quality Payment Program. This program provides clinicians with two ways to participate, including through the Advanced Alternative Payment Models ("APMs") and the Merit-based Incentive Payment System ("MIPS"). Under both

APMs and MIPS, performance data collected each performance year will affect Medicare payments in later years, including potentially reducing payments.

Also, there has been heightened governmental scrutiny recently over the manner in which drug manufacturers set prices for their marketed products, which have resulted in several presidential executive orders, congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. At the federal level, the IRA, among other things, (i) directs the U.S. Department of Health and Human Services ("HHS") to negotiate the price of certain high-expenditure, single-source drugs that have been on the market for at least seven years and biologics for at least 11 years covered under Medicare (the "Medicare Drug Price Negotiation Program"), and (ii) imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation. These provisions began to take effect progressively starting in fiscal year 2023. On August 15, 2024, HHS announced the agreed-upon price of the first ten drugs that were subject to price negotiations, although the Medicare Drug Price Negotiation Program is currently subject to legal challenges. On January 17, 2025, HHS selected fifteen additional products covered under Part D for price negotiation in 2025. Each year thereafter more Part B and Part D products will become subject to the Medicare Drug Price Negotiation Program. Further, on December 7, 2023, an initiative to control the price of prescription drugs through the use of march-in rights under the Bayh-Dole Act was announced. On December 8, 2023, the National Institute of Standards and Technology published for comment a Draft Interagency Guidance Framework for Considering the Exercise of March-In Rights which for the first time includes the price of a product as one factor an agency can use when deciding to exercise march-in rights. While march-in rights have not previously been exercised, it is uncertain if that will continue under the new framework.

At the state level, legislatures have increasingly passed and implemented 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.

These and other healthcare reform measures may be adopted in the future, particularly in light of the recent U.S. presidential and congressional elections, and may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved drug. The current presidential administration is pursuing policies to reduce regulations and expenditures across government including at HHS, the FDA, CMS and related agencies. These actions, presently directed by executive orders or memoranda from the Office of Management and Budget, may propose policy changes that create additional uncertainty for our business. For example, on September 30, 2025, the current administration announced the first agreement with a major pharmaceutical company that requires the drug manufacturer to offer, through a direct to consumer platform, U.S. patients and Medicaid programs prescription drug Most-Favored Nation pricing equal to or lower than those paid in other developed nations, with additional mandates for direct-to-patient discounts and repatriation of foreign revenues. Other recent actions and proposals include, for example, (i) reducing agency workforce and cutting programs; (ii) rescinding a previous executive order tasking the Center for Medicare and Medicaid Innovation to consider new payment and healthcare models to limit drug spending; (iii) eliminating the previous executive order that directed HHS to establish an AI task force and develop a strategic plan; (iv) directing HHS and other agencies to lower prescription drug costs for Medicare through a variety of initiatives, including by improving upon the Medicare Drug Price Negotiation Program and establishing Most-Favored-Nation pricing for pharmaceutical products; (v) imposing tariffs on imported pharmaceutical products; (vi) directing certain federal agencies to enforce existing law regarding hospital and plan price transparency and by standardizing prices across hospitals and health plans and (vii) as part of the Make America Healthy Again (MAHA) Commission's recent Strategy Report, working across government agencies to increase enforcement on direct-to-consumer pharmaceutical advertising. These actions and policies may significantly reduce U.S. drug prices, potentially impacting manufacturers' global pricing strategies and profitability, while increasing their operational costs and compliance risks. Additionally, in its June 2024 decision in Loper Bright Enterprises v. Raimondo, the U.S. Supreme Court overturned the longstanding Chevron doctrine, under which courts were required to give deference to regulatory agencies' reasonable interpretations of ambiguous federal statutes. The Loper Bright decision could result in additional legal challenges to current regulations and guidance issued by federal agencies applicable to our operations, including those issued by the FDA. Congress may introduce and ultimately pass health care related legislation that could impact the drug approval process and make changes to the Medicare Drug Price Negotiation Program created under the IRA. 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 drugs.

We may not be able to obtain or maintain orphan drug designations or exclusivity for our drug candidates, which could limit the potential profitability of our drug candidates.

Regulatory authorities in some jurisdictions, including the United States, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a drug as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States. Generally, if a drug with an orphan drug designation subsequently receives the

first marketing approval for an indication for which it receives the designation, then the drug is entitled to a period of marketing exclusivity that precludes the applicable regulatory authority from approving another marketing application for the same drug for the same indication for the exclusivity period except in limited situations. For purposes of small molecule drugs, the FDA defines "same drug" as a drug that contains the same active moiety and is intended for the same use as the drug in question. A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation.

Obtaining orphan drug designations is important to our business strategy; however, obtaining an orphan drug designation can be difficult and we may not be successful in doing so. Even if we were to obtain orphan drug designation for a drug candidate, we may not obtain orphan exclusivity and that exclusivity may not effectively protect the drug from the competition of different drugs for the same condition, which could be approved during the exclusivity period. Additionally, after an orphan drug is approved, the FDA could subsequently approve another application for the same drug for the same indication if the FDA concludes that the later drug is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug exclusive marketing rights in the United States also may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. The failure to obtain an orphan drug designation for any drug candidates we may develop, the inability to maintain that designation for the duration of the applicable period, or the inability to obtain or maintain orphan drug exclusivity could reduce our ability to make sufficient sales of the applicable drug candidate to balance our expenses incurred to develop it, which would have a negative impact on our operational results and financial condition.

Even if we obtain regulatory approval for our current or future drug candidates, they will remain subject to ongoing regulatory oversight.

Even if we obtain any regulatory approval for our current or future drug candidates, such approvals will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping and submission of safety and other post-market information. Any regulatory approvals that we receive for our current or future drug candidates may also be subject to a REMS, limitations on the approved indicated uses for which the drug may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 trials, and surveillance to monitor the quality, safety and efficacy of the drug.

In addition, drug manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP requirements and adherence to commitments made in the NDA, BLA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with a drug, such as adverse events of unanticipated severity or frequency, or problems with the facility where the drug is manufactured or if a regulatory authority disagrees with the promotion, marketing or labeling of that drug, a regulatory authority may impose restrictions relative to that drug, the manufacturing facility or us, including requesting a recall or requiring withdrawal of the drug from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements following approval of our current or future drug candidates, a regulatory authority may:

- issue an untitled letter or warning letter asserting that we are in violation of the law;
- seek an injunction or impose administrative, civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending NDA or comparable foreign marketing application (or any supplements thereto) submitted by us or our strategic partners;
- restrict the marketing or manufacturing of the drug;
- seize or detain the drug or otherwise require the withdrawal of the drug from the market;
- refuse to permit the import or export of drug candidates; or
- refuse to allow us to enter into supply contracts, including government contracts.

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.

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

In addition, the FDA's policies, and those of equivalent foreign regulatory agencies, may change and additional government regulations may be enacted that could cause changes to or delays in the drug review process, or suspend or restrict regulatory approval of our drug candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. 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, which would harm our business, financial condition, results of operations and prospects.

Disruptions at the FDA and other government agencies caused by layoffs, funding shortages or global health concerns could negatively impact our business.

The ability of the FDA to review proposed clinical trials or approve new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory, and policy changes, the FDA's ability to hire and retain key personnel and accept payment of user fees, 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 other government agencies that fund research and development activities is subject to the political process, including executive and congressional priorities, the impacts of which are inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may slow the time necessary for new product candidates to be reviewed and/or approved, which would adversely affect our business. For example, over the last several years, including in October 2025, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities. If the current government shutdown continues, or a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. In addition, the current administration has implemented substantial reductions in force at various government agencies including the FDA, and has implemented layoffs at the FDA, which could significantly reduce the FDA's capacity to perform its functions in a manner consistent with its past practices and could delay reviews and negatively impact our business.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain patent protection for our current or any future drug candidates, or if the scope of the patent protection obtained is not sufficiently broad, we may not be able to compete effectively in our markets.

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our development programs and drug candidates. Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our current and any future drug candidates. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our current and future development programs and drug candidates. The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our current or any future drug candidates in the United States or in other foreign countries. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found, which can invalidate a patent or prevent a patent from issuing from a pending patent application. Even if patents do successfully issue and even if such patents cover our current or any future drug candidates, third parties may challenge their validity, enforceability or scope, which may result in such patents being narrowed, invalidated, or held unenforceable. Any successful opposition to these patents or any other patents owned by or licensed to us could deprive us of rights necessary for the successful commercialization of any drug candidates or companion diagnostic that we may develop. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a drug candidate and companion diagnostic under patent protection could be reduced.

If the patent applications we hold or have in-licensed with respect to our development programs and drug candidates fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our current or any future drug candidates, it could dissuade companies from collaborating with us to develop

drug candidates, and threaten our ability to commercialize future drugs. Any such outcome could have a negative effect on our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does. Publications of discoveries in scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or drugs, in whole or in part, or which effectively prevent others from commercializing competitive technologies and drugs. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. In December 2011, the Leahy-Smith America Invents Act ("Leahy-Smith Act") was signed into law. The Leahy-Smith Act includes a number of significant changes to United States patent law. These include provisions that affect the way patent applications are prosecuted and may also affect patent litigation. The United States Patent Office recently developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, only became effective in March 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could harm our business and financial condition.

Moreover, we may be subject to a third-party pre-issuance submission of prior art to the USPTO or become involved in opposition, derivation, reexamination, inter partes review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or drugs and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize drugs without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future drug candidates.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. An adverse determination in any such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and drugs, or limit the duration of the patent protection of our technology and drugs. Moreover, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years from the earliest filing date of a non-provisional patent application. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. Without patent protection for our current or future drug candidates, we may be open to competition from generic versions of such drugs. 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 owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing drugs similar or identical to ours.

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

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the USPTO and various government patent agencies outside of the United States over the lifetime of our owned and licensed patents and/or applications and any patent rights we may own or license in the future. We rely on our outside counsel or our licensing partners to pay these fees due to non-U.S. patent agencies. The USPTO and various non-U.S. government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply and we are also dependent on our licensors to take the necessary action to comply with

these requirements with respect to our licensed intellectual property. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market and this circumstance could harm our business.

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

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. We expect to seek extensions of patent terms in the United States and, if available, in other countries where we are prosecuting patents. In the United States, the Drug Price Competition and Patent Term Restoration Act of 1984 permits a patent term extension of up to five years beyond the normal expiration of the patent, which is limited to the approved indication (or any additional indications approved during the period of extension). However, the applicable authorities, including the FDA and the USPTO in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. If this occurs, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data and launch their drug earlier than might otherwise be the case.

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

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. The following examples are illustrative:

- others may be able to make compounds or formulations that are similar to our drug candidates but that are not covered by the claims of any patents, should they issue, that we own or control;
- we or any strategic partners might not have been the first to make the inventions covered by the issued patents or pending patent applications that we own or control;
- we might not have been the first to file patent applications covering certain of our 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 our pending patent applications will not lead to issued patents;
- issued patents that we own or control may not provide us with any competitive advantages, or may be held invalid or unenforceable because of legal challenges;
- our competitors might conduct research and development activities in the United States and other countries that provide a safe harbor from patent infringement claims for certain research and development activities, as well as in countries where we do not have patent rights and then use the information learned from such activities to develop competitive drugs for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable; and
- the patents of others may have an adverse effect on our business.

The proprietary map of disease-relevant biological pathways underlying orphan disorders of the brain that we developed would not be appropriate for patent protection and, as a result, we rely on trade secrets to protect this aspect of our business.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a negative impact on the success of our business.

Our commercial success depends, in part, upon our ability and the ability of our current or future collaborators to develop, manufacture, market and sell our current and any future drug candidates and use our proprietary technologies without infringing the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may in the future become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our current and any future drug candidates and technology, including interference proceedings, post grant review and inter partes review before the USPTO. Third parties may assert infringement claims

against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could have a negative impact on our ability to commercialize our current and any future drug candidates. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. If we are found to infringe a third party's valid and enforceable intellectual property rights, we could be required to obtain a license from such third party to continue developing, manufacturing and marketing our drug candidate(s) and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing technology or drug candidate. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from manufacturing and commercializing our current or any future drug candidates or force us to cease some or all of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business, financial condition, results of operations and prospects. See the section herein titled "Legal Proceedings" for additional information.

We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Certain of our employees, consultants or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. 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. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property.

We may be involved in lawsuits to protect or enforce our patents, the patents of our licensors or our other intellectual property rights, which could be expensive, time consuming and unsuccessful.

Competitors may infringe or otherwise violate our patents, the patents of our licensors or our other intellectual property rights. To counter infringement or unauthorized use, we may be required to file legal claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing. The initiation of a claim against a third party may also cause the third party to bring counter claims against us such as claims asserting that our patents are invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, non-enablement or lack of statutory subject matter. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant material information from the USPTO, or made a materially misleading statement, during prosecution. Third parties may also raise similar validity claims before the USPTO in post-grant proceedings such as ex parte reexaminations, inter partes review, or post-grant review, or oppositions or similar proceedings outside the United States, in parallel with litigation or even outside the context of litigation. The outcome following legal assertions of invalidity and unenforceability is unpredictable. We cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. For the patents and patent

applications that we have licensed, we may have limited or no right to participate in the defense of any licensed patents against challenge by a third party. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of any future patent protection on our current or future drug candidates. Such a loss of patent protection could harm our business.

We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States. Our business could be harmed if in litigation the prevailing party does not offer us a license on commercially reasonable terms. Any litigation or other proceedings to enforce our intellectual property rights may fail, and even if successful, may result in substantial costs and distract our management and other employees.

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

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

The United States has recently enacted and implemented wide-ranging patent reform legislation. 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 actions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce patents that we have licensed or that we might obtain in the future. Similarly, changes in patent law and regulations in other countries or jurisdictions or changes in the governmental bodies that enforce them or changes in how the relevant governmental authority enforces patent laws or regulations may weaken our ability to obtain new patents or to enforce patents that we have licensed or that we may obtain in the future.

We may not be able to protect our intellectual property rights throughout the world, which could negatively impact our business.

Filing, prosecuting and defending patents covering our current and any future drug candidates throughout the world would be prohibitively expensive. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own drugs and, further, may export otherwise infringing drugs to territories where we may obtain patent protection, but where patent enforcement is not as strong as that in the United States. These drugs may compete with our drugs in jurisdictions where we do not have any issued or licensed patents and any future patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so competing.

Reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

If we rely on third parties to manufacture or commercialize our current or any future drug candidates, or if we collaborate with additional third parties for the development of our current or any future drug candidates, we must, at times, share trade secrets with them. We may also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure could have an adverse effect on our business and results of operations.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third parties, independent development or publication of information by any third-party collaborators. A competitor's discovery of our trade secrets would harm our business.

Risks Related to Our Dependence on Third Parties

We do not have our own manufacturing capabilities and will rely on third parties to produce clinical and commercial supplies of our current and any future drug candidates.

We do not own or operate, and we do not expect to own or operate, facilities for drug manufacturing, drug formulation, storage and distribution or testing. We have been in the past, and will continue to be, dependent on third parties to manufacture the clinical supplies of our drug candidates.

Further, we also will rely on third-party manufacturers to supply us with sufficient quantities of our drug candidates to be used, if approved, for commercialization. Any significant delay in the supply of a drug candidate, or the raw material components thereof, for an ongoing clinical trial due to the need to replace a third-party manufacturer could considerably delay completion of our clinical trials, product testing and potential regulatory approval of our drug candidates.

Further, our reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured drug candidates ourselves including:

- inability to meet our drug specifications and quality requirements consistently;
- delay or inability to procure or expand sufficient manufacturing capacity;
- issues related to scale-up of manufacturing;
- costs and validation of new equipment and facilities required for scale-up;
- failure to comply with cGMP and similar foreign standards;
- inability to negotiate manufacturing agreements with third parties under commercially reasonable terms, if at all;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us;
- reliance on single sources for drug components;
- lack of qualified backup suppliers for those components that are currently purchased from a sole or single source supplier;
- operations of our third-party manufacturers or suppliers could be disrupted by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier; and
- carrier disruptions or increased costs that are beyond our control.

Any of these events could lead to clinical trial delays, failure to obtain regulatory approval or impact our ability to successfully commercialize our current or any future drug candidates once approved. Some of these events could be the basis for FDA action, including injunction, request for recall, seizure, or total or partial suspension of production.

We rely on third parties to conduct, supervise and monitor our preclinical studies and clinical trials, and if those third parties perform in an unsatisfactory manner, it may harm our business.

We do not currently have the ability to independently conduct any preclinical studies or clinical trials. We rely on CROs and clinical trial sites to ensure the proper and timely conduct of our preclinical studies and clinical trials, and we expect to have limited influence over their actual performance. We rely upon CROs to monitor and manage data for our clinical programs, as well as the execution of future preclinical studies. We are able to control only certain aspects of our CROs' activities. Nevertheless, we will be responsible for ensuring that each of our preclinical studies or clinical trials are conducted in accordance with the applicable protocol, legal, regulatory and scientific standards and our reliance on the CROs does not relieve us of our regulatory responsibilities.

We and our CROs will be required to comply with GLPs and GCPs, which are regulations and guidelines enforced by the FDA and are also required by the Competent Authorities of the Member States of the European Economic Area and comparable foreign regulatory authorities in the form of International Council for Harmonization guidelines for any of our drug candidates that are in preclinical and clinical development. The regulatory authorities enforce GCPs through periodic inspections of trial sponsors, principal investigators and clinical trial sites. Although we will rely on CROs to conduct GCP-compliant clinical trials, we remain responsible for ensuring that each of our GLP preclinical studies and clinical trials is conducted in accordance with its investigational plan and protocol and applicable laws and regulations, and our reliance on the CROs does not relieve us of our regulatory responsibilities. If we or our CROs fail to comply with GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign

regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. Accordingly, if our CROs fail to comply with these regulations or fail to recruit a sufficient number of subjects, we may be required to repeat clinical trials, which would delay the regulatory approval process.

While we have agreements governing their activities, our CROs are not our employees, and we do not control whether or not they devote sufficient time and resources to our future clinical and preclinical programs. These CROs 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 harm our business. We face the risk of potential unauthorized disclosure or misappropriation of our intellectual property by CROs, which may reduce our trade secret protection and allow our potential competitors to access and exploit our proprietary technology. If our CROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, 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 any other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize any drug candidate that we develop. As a result, our financial results and the commercial prospects for any drug candidate that we develop would be harmed, our costs could increase, and our ability to generate revenue could be delayed.

In addition, 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. The FDA may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the trial. The FDA 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 and may ultimately lead to the denial of marketing approval of our current and future drug candidates.

If our relationship with these CROs terminates, we may not be able to enter into arrangements with alternative CROs or do so on commercially reasonable terms. Switching or adding additional CROs involves substantial cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can negatively impact our ability to meet our desired clinical development timelines. Though we intend to carefully manage our relationships with our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a negative impact on our business, financial condition and prospects.

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

We are highly dependent on the services of our senior management team, including our Chairman and Chief Executive Officer, Dr. Jeremy Levin, and if we are not able to retain these members of our management team or recruit and retain additional management, clinical and scientific personnel, our business will be harmed.

We are highly dependent on our senior management team, including our Chairman and Chief Executive Officer, Dr. Levin. The employment agreements we have with these officers do not prevent such persons from terminating their employment with us at any time. The loss of the services of any of these persons could impede the achievement of our research, development, operational, financial and commercialization objectives.

In addition, we are dependent on our continued ability to attract, retain and motivate highly qualified additional management, clinical and scientific personnel. If we are not able to retain our management and to attract, on acceptable terms, additional qualified personnel necessary for the continued development of our business, we may not be able to sustain our operations or grow. This risk may be further amplified given the particularly competitive hiring market in New York City, the location of our corporate headquarters.

We may not be able to attract or retain qualified personnel in the future due to the intense competition for qualified personnel among biotechnology, pharmaceutical and other businesses. Many of the other pharmaceutical companies that we compete against for qualified personnel and consultants have greater financial and other resources, different risk profiles and a longer history in the industry than we do. They also may provide more diverse opportunities and better chances for career advancement. Some of these characteristics may be more appealing to high-quality candidates and consultants than what we have to offer. If we are unable to continue to attract, retain and motivate high-quality personnel and consultants to accomplish our business objectives, the rate and success at which we can discover and develop drug candidates and our business will be limited and we may experience constraints on our development objectives.

Our future performance will also depend, in part, on our ability to successfully integrate newly hired executive officers into our management team and our ability to develop an effective working relationship among senior management. Our failure to integrate these individuals and create effective working relationships among them and other members of

management could result in inefficiencies in the development and commercialization of our drug candidates, harming future regulatory approvals, sales of our drug candidates and our results of operations. Additionally, we do not currently maintain "key person" life insurance on the lives of our executives or any of our employees.

We may need to expand our organization, and we may experience difficulties in managing this growth, which could disrupt our operations.

In connection with our future growth plans, we may need to hire additional employees. We may have difficulties in connection with identifying, hiring, integrating and retaining new personnel. Future growth would impose significant additional responsibilities on management, including the need to identify, recruit, maintain, motivate and integrate additional employees, consultants and contractors. Also, our management may need to divert a disproportionate amount of its attention away from our day-to-day operations and devote a substantial amount of time to managing these growth activities, which could be a particular challenge as a result of the workforce reduction completed in June 2024. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational inefficiencies, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of our current and potential future drug candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and grow revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance, our ability to commercialize drug candidates, develop a scalable infrastructure and compete effectively will depend, in part, on our ability to effectively manage any future growth.

Many of the other pharmaceutical and healthcare companies that we compete against for qualified personnel and consultants have greater financial and other resources, different risk profiles and a longer operating history in the industry than us. They also may provide more diverse opportunities and better chances for career advancement. Some of these opportunities may be more appealing to high-quality candidates and consultants than what we have to offer. If we are unable to continue to attract and retain high-quality personnel and consultants, the rate and success at which we can discover and develop our products and product candidates will be harmed, which could negatively impact our financial condition, results of operations and cash flows.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk that our employees, consultants, distributors, principal investigators, collaborators and commercial partners may engage in fraudulent or illegal activity. Misconduct by these parties could include intentional, reckless or negligent conduct or disclosure of unauthorized activities to us that violates the regulations of the FDA and non-U.S. regulators, including those laws requiring the reporting of true, complete and accurate information to such regulators, manufacturing standards, healthcare fraud and abuse laws and regulations in the United States and abroad or laws that require the true, complete and accurate reporting of financial information or data. In particular, sales, marketing and business arrangements in the healthcare industry, including the sale of pharmaceuticals, 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. It is not always possible to identify and deter misconduct by our employees and other third 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. Further, because of our hybrid-work policies, information that is normally protected, including confidential company information, may be less secure. If actions are instituted against us and we are not successful in defending ourselves or asserting our rights, those actions could result in the imposition of significant fines or other sanctions, including the imposition of civil, criminal and administrative penalties, damages, monetary fines, imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal 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, contractual damages, reputational harm, diminished profits and future earnings and curtailment of operations, any of which could adversely affect our ability to operate our business and our results of operations. Whether or not we are successful in defending against such actions or investigations, we could incur substantial costs, including legal fees, and divert the attention of management in defending ourselves against any of these claims or investigations.

If our information technology systems or data, or those of the third parties upon with whom we work, are or were compromised, we could experience adverse impacts resulting from such compromise, including, but not limited to, regulatory investigations or actions, litigation, fines and penalties, interruptions to our operations such as our clinical

trials, claims that we breached our data protection obligations, harm to our reputation, and a loss of future customers or sales and other adverse consequences.

We are increasingly dependent on information technology systems and infrastructure, including mobile technologies, to operate our business. In the ordinary course of our business, we collect, store, process and transmit large amounts of sensitive data, and, as a result, we and the third parties with whom we work face a variety of continuously evolving threats that have caused and could cause future security incidents. In addition, many of those third parties in turn subcontract or outsource some of their responsibilities to other third parties. While all information technology operations are inherently vulnerable to inadvertent or intentional security breaches, incidents, attacks and exposures, the accessibility and distributed nature of our information technology systems, and the sensitive data stored on those systems, make such systems vulnerable to unintentional or malicious, internal and external attacks on our technology environment. We have also outsourced elements of our operations (including elements of our information technology infrastructure) to third parties, and as a result, certain third parties with whom we work have access to our computer networks and/or our sensitive data. In addition, many of those third parties in turn subcontract or outsource some of their responsibilities to other third parties. Our ability to monitor the third parties' information security practices is limited, and these third parties may not have adequate information security measures in place. When the third parties with whom we work experience a security incident or other interruption, which has occurred in the past, we could experience adverse consequences. For example, in the third quarter of 2024, we were notified of a business email compromise impacting a development collaborator, which resulted in our payment being misdirected to a fraudulent account. The funds were fully recovered in January 2025. In the absence of recovery of funds or other remuneration, 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. Increasing global tensions, including tensions between China and Taiwan and the ongoing wars involving Ukraine and Israel, among others, are likely to increase the frequency of cybersecurity attacks.

In addition, hybrid work has increased risks to our information technology systems and data as our employees utilize network connections, computer and devices outside our premises or network, including working at home, while in transit and in public locations. Additionally, 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. Furthermore, we may discover security issues that were not identified during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

We take steps designed to detect, mitigate, and remediate vulnerabilities in our information systems (such as our hardware and/or software, including that of third parties upon which we rely); however, we may not detect and remediate all such vulnerabilities on a timely basis. Further, we may and have experienced delays in deploying remedial measures and patches designed to address identified vulnerabilities. For example, we have had situations in which a vulnerability has been identified, but the remediating patch download and installation was delayed due to the user being offline or the computer not being rebooted in a timely manner to finalize the installation. Non-remediated vulnerabilities could be exploited and result in a security incident.

Cyberattacks, malicious internet-based activity, online and offline fraud, and other similar activities are increasing in their frequency, levels of persistence, sophistication and intensity, and are also being conducted by sophisticated and organized groups and individuals with a wide range of motives (including, but not limited to, industrial espionage) and expertise, including organized criminal groups, "hacktivists," nation states and others. Such attacks could include the deployment of harmful malware (including as a result of advanced persistent threat intrusions), ransomware attacks, denial-of-service attacks, credential stuffing and/or harvesting, social engineering (including through deep fakes, which may be increasingly more difficult to identify as fake, and phishing attacks), supply-chain attacks, software bugs, server malfunctions, software or hardware failures, loss of sensitive data or other information technology assets, adware, attacks enhanced or facilitated by artificial intelligence, telecommunications failures, earthquakes, fires, floods and other means to affect service reliability and threaten the confidentiality, integrity and availability of our information systems and sensitive data. In particular, severe ransomware attacks are becoming increasingly prevalent and can lead to significant interruptions in our operations, ability to provide our products or services, loss of sensitive data and income, reputational harm, and diversion of funds. Extortion payments may alleviate the negative impact of a ransomware attack, but we may be unwilling or unable to make such payments due to, for example, applicable laws or regulations prohibiting such payments.

Significant disruptions of our, our third-party vendors' and/or business partners' information technology systems or other similar data security incidents could adversely affect our business operations and/or result in the loss, misappropriation, and/or unauthorized access, use or disclosure of, or the prevention of access to, sensitive data, which could result in financial, legal, regulatory, business and reputational harm to us. In addition, information technology system disruptions, whether from attacks on our technology environment or from computer viruses, natural disasters, terrorism,

war and telecommunication and electrical failures, could result in a material disruption of our development programs and our business operations. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data.

It may be difficult and/or costly to detect, investigate, mitigate, contain and remediate a security incident. Our efforts to do so may not be successful. For example, we utilize a phishing reporting feature that allows our IT function to analyze and assess whether suspicious emails are phishing attempts. This system is reliant on artificial intelligence and employee awareness and diligence, both of which may be exploited or not always successful. Actions taken by us or the third parties with whom we work to detect, investigate, mitigate, contain and remediate a security incident could result in outages, data losses, and disruptions of our business. Threat actors may also gain access to other networks and systems after a compromise of our networks and systems. We have in the past and will in the future expend significant resources or modify our business activities to try to protect against current and constantly evolving security incidents. Additionally, certain data privacy and security obligations will require us to implement and maintain specific security measures or industry-standard or reasonable security measures to protect our information technology systems and sensitive data.

Applicable data privacy and security obligations may require us to notify relevant stakeholders, including affected individuals, customers, regulators, and investors, of security incidents. Such disclosures are costly, and the disclosure or the failure to comply with such requirements could lead to adverse consequences.

If we (or a third party with whom we work) experience a security incident or are perceived to have experienced a security incident, including but not limited to a security incident involving personal information regarding our patients or employees, we may experience adverse consequences, such as disruptions to our business, harm to our reputation, government enforcement actions (for example, investigations, fines, penalties, audits, and inspections), additional reporting requirements, and/or oversight, or we may otherwise be subject to liability under laws, regulations and contractual obligations, including those that protect the privacy and security of personal information. This could result in increased costs to us, and result in significant legal and financial exposure and/or reputational harm. In addition, any failure or perceived failure by us or our vendors or business partners to comply with our privacy, confidentiality or data security-related legal or other obligations to third parties, or any further security incidents or other inappropriate access events that result in the unauthorized access, release or transfer of sensitive data, may result in governmental investigations, enforcement actions, regulatory fines, litigation, or public statements against us by advocacy groups or others, and could cause third parties, including clinical sites, regulators or current and potential partners, to lose trust in us or we could be subject to claims by third parties that we have breached our privacy- or confidentiality-related obligations, which could materially and adversely affect our business and prospects. Moreover, data security incidents and other inappropriate access can be difficult to detect, and any delay in identifying them may lead to increased harm of the type described above.

While we have implemented security measures intended to protect our information technology systems and infrastructure, there can be no assurance that such measures will be effective. 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.

While we are seeking cybersecurity insurance coverage to cover certain aspects of the cyber risks described above, there is no guarantee that we will obtain cybersecurity insurance coverage sufficient to cover the risks described here. Furthermore, any losses suffered by the company may not be adequately covered by insurance or other contractual rights available to us. The successful assertion of one or more large claims against us that exceed or are not covered by our insurance coverage or changes in our insurance policies, including premium increases or the imposition of large deductible or co-insurance requirements, could make us unable to acquire such insurance and may have an adverse effect on our business, financial condition, and results of operations.

In addition to experiencing a security incident, third parties may gather, collect or infer sensitive information about us from public sources, data brokers or other means that reveal competitively sensitive details about our organization and could be used to undermine our competitive advantage or market position. Additionally, sensitive company information could be leaked, disclosed or revealed as a result of or in connection with our employees', personnel's, or vendors' use of generative artificial intelligence technologies.

We and the third parties with whom we work are subject to stringent and evolving privacy and security laws, regulations, contractual obligations, industry standards, policies, and other obligations, and our failure or perceived failure to comply with such obligations could result in regulatory investigations or actions, litigation (including class actions), fines and penalties, disruptions of our business operations, loss of revenue or profits, reputational damage and other adverse business consequences.

In the ordinary course of business, we and the third parties with whom we work collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, process)

personal data and other sensitive information, including proprietary and confidential business data, trade secrets, intellectual property, sensitive third-party data, business plans, transactions, clinical trial data and financial information (collectively, sensitive data).

Our data processing activities subject us to laws and regulations covering data privacy and the protection of personal information and other sensitive data. The legislative and regulatory landscape for privacy and data protection continues to evolve, and there has been an increasing focus on privacy and data protection issues which may affect our business. In the United States, there are state security breach notification laws, state health information privacy laws and federal and state consumer protections laws which impose requirements for the collection, use, disclosure and transmission of personal information. Each of these laws is subject to varying interpretations by courts and government agencies. If we fail to comply with applicable laws and regulations we could be subject to penalties or sanctions, including criminal penalties if we knowingly obtain individually identifiable health information from a covered entity in a manner that is not authorized or permitted by HIPAA or for aiding and abetting the violation of HIPAA.

Numerous U.S. states have enacted comprehensive privacy laws that impose certain obligations on covered businesses, including providing specific disclosures in privacy notices and affording residents with certain rights concerning their personal data. As applicable, such rights may include the right to access, correct, or delete certain personal data, and to opt-out of certain data processing activities, such as targeted advertising, profiling, and automated decision-making. The exercise of these rights may impact our business and ability to provide our products and services if we become subject to these laws. For example, California enacted the California Consumer Privacy Act ("CCPA") which applies to personal information of consumers, business representatives, and employees, and requires businesses to provide specific disclosures in privacy notices and honor requests of California residents to exercise certain privacy rights The CCPA provides for civil penalties for violations, as well as a private right of action for certain data breaches. Although there are limited exemptions for clinical trial data under the CCPA (and the other similar state privacy laws), the CCPA and other similar laws may impact (possibly significantly) our business activities depending on how it is interpreted, should we become subject to the CCPA and other state comprehensive privacy laws in the future.

Numerous other countries have, or are developing, laws governing the collection, use and transmission of personal information as well. For example, the European Union's General Data Protection Regulation ("EU GDPR"), the United Kingdom's GDPR ("UK GDPR"), impose strict requirements for processing personal data. The EU GDPR provides for fines of up to 20 million Euros or 4% of annual global revenue, whichever is greater.

In the ordinary course of business, we transfer personal data from Europe and other jurisdictions to the United States or other countries. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal data to other countries. In particular, the European Economic Area (EEA) and the United Kingdom (UK) have significantly restricted the transfer of personal data to the United States and other countries whose privacy laws it believes are inadequate. Other jurisdictions have and may continue to adopt similarly stringent interpretations of their data localization and cross-border data transfer laws. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and UK to the United States in compliance with law, such as the EEA and UK's standard contractual clauses, these mechanisms are subject to legal challenges, and there is no assurance that we can satisfy or rely on these measures to lawfully transfer personal data to the United States. If there is no lawful manner for us to transfer personal data from the EEA, the UK or other jurisdictions to the United States, or if the requirements for a legally-compliant transfer are too onerous, we could face significant adverse consequences, including the interruption or degradation of our operations, the need to relocate part of or all of our business or data processing activities to other jurisdictions at significant expense, increased exposure to regulatory actions, substantial fines and penalties, the inability to transfer data and work with partners, vendors and other third parties, and injunctions against our processing or transferring of personal data necessary to operate our business. Additionally, companies that transfer personal data out of the EEA and UK to other jurisdictions, particularly to the United States, are subject to increased scrutiny from regulators, individual litigants, and activist groups. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers out of Europe for allegedly violating the GDPR's cross-border data transfer limitations. Additionally, the U.S. Department of Justice issued a rule entitled the Preventing Access to U.S. Sensitive Personal Data and Government-Related Data by Countries of Concern or Covered Persons, which places additional restriction on certain data transactions involving countries of concern (e.g., China, Russia, Iran) and covered individuals (i.e., individuals and entities located in or controlled by individuals or entities located in those jurisdictions) that may impact certain business activities such as vendor engagements, employment of certain individuals, and investor agreements. Violations of the rule could lead to significant civil and criminal fines and penalties. The rule applies regardless of whether data is anonymized, key-coded, pseudonymized, de-identified or encrypted, which presents particular challenges for companies like ours and may impact our ability to transfer data in connection with certain transactions or agreements.

Our employees and personnel use generative artificial intelligence ("AI") technologies to perform their work, and the disclosure and use of personal data in generative AI technologies is subject to various privacy laws and other privacy

obligations. Governments have passed and are likely to pass additional laws regulating generative AI. Our use of this technology could result in additional compliance costs, regulatory investigations and actions, and lawsuits. If we are unable to use generative AI, it could make our business less efficient and result in competitive disadvantages.

In addition to data privacy and security laws, we are bound by certain contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. We also publish privacy policies, marketing materials, and other statements regarding data privacy and security. Regulators are increasingly scrutinizing these statements, and if these policies, materials, or statements are found to be deficient, lacking in transparency, deceptive, unfair, misleading or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators, or other adverse consequences.

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 with whom we work may fail to comply with such obligations, which could negatively impact our business operations. If we or the third parties with whom we work fail, or are perceived to have failed, to address or comply with applicable data privacy and security obligations, we could face significant consequences, including but not limited to: government enforcement actions (e.g., investigations, fines, penalties, audits, inspections and similar); litigation (including class-action claims) and mass arbitration demands; additional reporting requirements and/or oversight; bans on processing personal data (including clinical trial data); and orders to destroy or not use personal data.

Our ability to use our net operating loss ("NOL") carryforwards and certain other tax attributes to offset future taxable income may be subject to limitation.

Our NOL carryforwards could expire unused and be unavailable to offset future income tax liabilities because of their limited duration or because of restrictions under U.S. tax law. Our federal NOLs generated in tax years beginning on or before December 31, 2017, are permitted to be carried forward for only 20 years under applicable U.S. tax law. Under the Tax Cuts and Jobs Act, or the Tax Act, as modified by the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, federal NOLs incurred in taxable years beginning after December 31, 2017, may be carried forward indefinitely, but the utilization of such federal NOLs is limited.

In addition, under Section 382 and Section 383 of the Internal Revenue Code of 1986, as amended (the "Code"), and corresponding provisions of state law, if a corporation undergoes an "ownership change," its ability to use its pre-change NOL carryforwards and other pre-change tax attributes (such as research tax credits) to offset its post-change income may be limited. A Section 382 "ownership change" generally occurs if one or more stockholders or groups of stockholders who own at least 5% of our stock increase their ownership by more than 50 percentage points (by value) over their lowest ownership percentage over a rolling three-year period. We may have experienced ownership changes in the past and may experience ownership changes in the future as a result of shifts in our stock ownership (some of which are outside our control). As a result, if we earn net taxable income, our ability to use our pre-change NOLs and certain other tax attributes to offset such taxable income may be subject to limitations. Similar provisions of state tax law may also apply to limit our use of accumulated state tax attributes. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

For the three months ended September 30, 2025, we recorded no U.S. federal or state income tax provision, based on a pre-tax loss of $12.2 million. As of September 30, 2025, we had available $249.5 million of unused NOL carryforwards for U.S. federal income tax purposes. As of December 31, 2024, we had $14.9 million of unused NOL carryforwards for Massachusetts income tax purposes, $164.1 million of unused NOL carryforwards for New York income tax purposes, and $163.8 million of unused NOL carryforwards for New York City income tax purposes and $18.3 million of unused NOL carryforwards for Pennsylvania income tax purposes that may be applied against future taxable income. Our NOL carryforwards are significantly limited such that even if we achieve profitability in future periods, we may not be able to utilize most of the NOL carryforwards, which could have a material adverse effect on cash flow and results of operations.

Risks Related to Being a Public Company

We are a "smaller reporting company" and the reduced disclosure requirements applicable to such companies may make our common stock less attractive to investors.

We are currently a "smaller reporting company" as defined in the Securities Exchange Act of 1934, as amended. We will be a smaller reporting company and may take advantage of the scaled-back disclosures available to smaller reporting companies for so long as (i) the market value of our voting and non-voting ordinary shares held by non-affiliates is less than $250.0 million measured on the last business day of our most recently completed second fiscal quarter or (ii) (a) our annual revenue is less than $100.0 million during the most recently completed fiscal year and (b) the market value of

our voting and non-voting ordinary shares held by non-affiliates is less than $700.0 million measured on the last business day of our most recently completed second fiscal quarter.

As a smaller reporting company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation. If investors consider our common stock less attractive as a result of our election to use the scaled-back disclosure permitted for smaller reporting companies, there may be a less active trading market for our common shares and our stock price may be more volatile.

We have previously identified material weaknesses in our internal control over financial reporting. If we fail to maintain an effective system of internal control over financial reporting in the future, we may not be able to accurately report our financial condition, results of operations or cash flows, which may adversely affect investor confidence in us and, as a result, the value of our common stock.

During the three and nine month periods ended September 30, 2024, we identified material weaknesses in our internal control over financial reporting, which were subsequently remediated as of December 31, 2024. In the future, if we are unable to identify or remediate material weaknesses, or if we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial condition, results of operations or cash flows, which may adversely affect investor confidence in us and, as a result, the value of our common stock.

The Sarbanes-Oxley Act requires, among other things, that we maintain effective internal controls over financial reporting and disclosure controls and procedures. We are required, under Section 404, to furnish a report by management on, among other things, the effectiveness of our internal control over financial reporting. This assessment will need to include disclosure of any material weaknesses identified by our management in our internal control over financial reporting. Section 404 also generally requires an attestation from our independent registered public accounting firm on the effectiveness of our internal control over financial reporting. We will not be required to have our auditors formally attest to the effectiveness of our internal control over financial reporting unless we cease to be a smaller reporting company.

During the quarter ended September 30, 2024, we were the victim of a criminal scheme involving a business email compromise at one of our development collaborators, which led to a fraudulent transfer of $1.8 million to a third-party impersonating one of our development collaborators. The funds were subsequently fully recovered. As a result of the misdirection of funds, management re-evaluated the effectiveness of our disclosure controls and procedures and internal control over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act. Based on this assessment, management identified material weaknesses related to fund transfers and vendor-related information updates. A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that a reasonable possibility exists that a material misstatement of our annual or condensed interim financial statements would not be prevented or detected on a timely basis.

Effective internal controls are necessary for us to provide reliable financial reports and prevent fraud. Immediately following the incident, we initiated a reassessment of our processes and controls related to fund transfers and vendor-related information updates and developed an action plan that remediated this matter, as concluded in Part II, Item 9a of our Annual Report on Form 10-K for the year ended December 31, 2024.

Our compliance with Section 404 in future periods may require that we incur substantial expense and expend significant management efforts. We currently do not have an internal audit group and rely on experienced consultants to support this function. We may need to hire additional consultants or accounting and financial staff with appropriate public company experience and technical accounting knowledge in order to continually comply with Section 404. We may not be able to complete our evaluation, testing and any required remediation in a timely fashion. We cannot assure you that there will not be additional material weaknesses or significant deficiencies in our internal control over financial reporting in the future. Any failure to maintain internal control over financial reporting could severely inhibit our ability to accurately report our financial condition, results of operations or cash flows. If we are unable to conclude that our internal control over financial reporting is effective, or if our independent registered public accounting firm determines we have any additional material weakness in our internal control over financial reporting, we could lose investor confidence in the accuracy and completeness of our financial reports, the market price of our common stock could decline, and we could be subject to sanctions or investigations by The Nasdaq Stock Market LLC, the SEC or other regulatory authorities. Failure to remedy material weaknesses in our internal control over financial reporting, or to implement or maintain other effective control systems required of public companies, could also restrict our future access to the capital markets.

Risks Related to the Ownership of Our Common Stock and Other General Matters

If we fail to satisfy all applicable requirements of Nasdaq and it determines to delist our common stock, the delisting could adversely affect the market liquidity of our common stock and the market price of our common stock could decrease.

To maintain the listing of our common stock on Nasdaq, we are required to meet certain listing requirements, including, a minimum closing bid price of $1.00 per share. On February 10, 2025, we received notice from Nasdaq that we are no longer in compliance with Nasdaq's Listing Rule 5450(a)(1) because the closing bid price of our common stock had fallen below $1.00 per share for 31 consecutive days. In accordance with Nasdaq Listing Rule 5810(c)(3)(A), we had an initial period of 180 calendar days, or until August 11, 2025, to regain compliance with the minimum bid price requirement. We did not regain compliance with the minimum bid price requirement prior to August 11, 2025. However, on August 12, 2025, we received approval from the Listing Qualifications Department of Nasdaq to transfer the listing of our common stock from the Nasdaq Global Select Market to the Nasdaq Capital Market (the "Approval"). In connection with the Approval, we were granted an additional 180-day grace period, or until February 9, 2026 (the "Compliance Date"), to regain compliance with the minimum bid price requirement. On September 11, 2025, the Company received formal notification from Nasdaq that it had regained compliance with the minimum bid price requirement.

There can be no assurance that we will maintain compliance with the requirements for listing our common stock on Nasdaq. If we are unable to satisfy the Nasdaq criteria for continued listing, our common stock would be subject to delisting. A delisting of our common stock could negatively impact us by, among other things, (i) reducing the liquidity and market price of our common stock; (ii) reducing the number of investors willing to hold or acquire our common stock, which could negatively impact our ability to raise equity financing; (iii) decreasing the amount of news and analyst coverage of us; (iv) limiting our ability to issue additional securities or obtain additional financing in the future; (v) limiting our ability to use a registration statement to offer and sell freely tradable securities, thereby preventing us from accessing the public capital markets; and (vi) impairing our ability to provide equity incentives to our employees. In addition, delisting from Nasdaq may negatively impact our reputation and, consequently, our business.

The market price of our common stock has been and likely will remain volatile and fluctuate substantially, which could result in substantial losses for holders of our common stock.

The market price of our common stock has been and likely will remain volatile. For example, during the nine months ended September 30, 2025, the closing price of our common stock on The Nasdaq Global Select Market ranged from $0.26 per share to $1.73 per share. In June 2024, we experienced a stock price drop following our announcement of Takeda's release of topline Phase 3 study results for soticlestat, noting that soticlestat narrowly missed its primary endpoint and showed clinically meaningful and significant effects in multiple key secondary efficacy endpoints with respect to Dravet syndrome and missed its primary endpoint with respect to Lennox-Gastaut syndrome. In addition, the stock market in general and the market for biopharmaceutical or pharmaceutical companies in particular, has experienced extreme volatility that has often been unrelated to the operating performance of particular companies, which has resulted in decreased stock prices for many companies notwithstanding the lack of a fundamental change in their underlying business models or prospects. Broad market and industry factors, including potentially worsening economic conditions and other adverse effects or developments relating to new or ongoing public health crises or other inflationary factors, may negatively affect the market price of our common stock, regardless of our actual operating performance. As a result of this volatility, stockholders may lose all or part of their investment in our common stock since they may be unable to sell their shares at or above the price paid for the shares. The market price for our common stock may be influenced by many factors, including:

- results of clinical trials of our current and any future drug candidates, or those of our competitors;
- the success of competitive drugs or therapies;
- 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;
- the level of expenses related to our current and any future drug candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional drug candidates;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;

- our inability to obtain or delays in obtaining adequate drug supply for any approved drug or inability to do so at acceptable prices;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions, including supply chain disruptions and inflationary pressure; and
- the other factors described in this "Risk Factors" section.

In addition, in the past, stockholders have initiated class action lawsuits against companies following periods of volatility in the market prices of these companies' stock. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management's attention and resources.

There is no public market for our Series A or Series B Preferred Stock.

There is no established public trading market for our Series A or Series B Preferred Stock, and we do not expect a market to develop. In addition, we do not intend to apply for listing of the Series A or Series B Preferred Stock on any national securities exchange or other nationally recognized trading system. Without an active market, the liquidity of the Series A and Series B Preferred Stock will be limited.

Stockholders will be diluted by any conversions of outstanding Series A Preferred Stock and exercises of outstanding options.

As of September 30, 2025, we had outstanding options to purchase an aggregate of 18,401,818 shares of our common stock at a weighted average exercise price of $2.77 per share and 1,250,000 shares of common stock issuable upon conversion of outstanding Series A Preferred Stock for no additional consideration. Such Series A Preferred Stock is convertible any time at the option of the holder thereof subject to the beneficial ownership limitations described in Note 7 to the condensed consolidated financial statements contained in this Quarterly Report on Form 10-Q. The exercise of such options and conversion of the Series A Preferred Stock for shares of our common stock will result in further dilution of our stockholders' investment and could negatively affect the market price of our common stock. In addition, stockholders may experience further dilution if we issue common stock, or securities convertible into common stock, in the future. As a result of this dilution, stockholders may receive significantly less than the full purchase price paid for the shares in the event of liquidation.

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

Based upon shares of our common stock outstanding as of September 30, 2025, our executive officers, directors and stockholders who owned more than 5% of our outstanding common stock, in the aggregate, beneficially own shares representing approximately 41.8% of our outstanding common stock.

Takeda, a greater than 5% holder, has agreed to, among other things, (i) a standstill provision, (ii) restrictions on its ability to sell or otherwise transfer its shares of our stock, (iii) vote its shares on certain matters in accordance with the holders of a majority of shares of our common stock, and (iv) restrictions on the percentage of our outstanding common stock it may own, in accordance with the terms of the RLT Agreement.

If our executive officers, directors and stockholders who owned more than 5% of our outstanding common stock acted together, they may be able to significantly influence all matters requiring stockholder approval, including the election and removal of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. The concentration of voting power, Takeda standstill provisions, voting obligations and transfer restrictions could delay or prevent an acquisition of our company on terms that other stockholders may desire or result in the management of our company in ways with which other stockholders disagree.

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 relies, in part, on the research and reports that industry or financial analysts publish about us or our business. We currently have research coverage offered by several industry or financial analysts. We do not have any control over these analysts. If one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If additional analysts cease to cover our stock or fail to regularly publish reports, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be stockholders' sole source of gain.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be our stockholders' sole source of gain for the foreseeable future.

Provisions in our organizational documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our amended and restated certificate of incorporation ("Certificate of Incorporation") and our amended and restated bylaws ("Bylaws") may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which they might otherwise receive a premium for their shares. These provisions also could limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that not all members of the board of directors are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board of directors;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a stockholder rights plan, or so-called "poison pill," that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 66 2/3% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our Certificate of Incorporation or Bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law (the "DGCL"), which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

Additionally, the Takeda standstill provisions and transfer restrictions in the RLT Agreement may delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which stockholders might otherwise receive a premium for their shares.

We may be subject to securities litigation, which is expensive and could divert management attention.

The market price of our common stock may be volatile. In the past, companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. We may be the target of this type of litigation in the future. Securities litigation against us could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business.

Some provisions of our organizational documents and the DGCL may have anti-takeover effects that could discourage an acquisition of us by others, even if an acquisition would benefit our stockholders and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our Certificate of Incorporation and Bylaws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us or increase the cost of acquiring us, even if doing so would benefit our stockholders, or remove our current management. These provisions include:

- authorizing the issuance of "blank check" preferred stock, the terms of which we may establish and shares of which we may issue without stockholder approval;
- prohibiting cumulative voting in the election of directors, which would otherwise allow for less than a majority of stockholders to elect director candidates;
- prohibiting stockholder action by written consent, thereby requiring all stockholder actions to be taken at a meeting of our stockholders;
- eliminating the ability of stockholders to call a special meeting of stockholders; and
- establishing advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted upon at stockholder meetings.

These provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors, who are responsible for appointing the members of our management. Because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the DGCL, which may discourage, delay or prevent someone from acquiring us or merging with us whether or not it is desired by or beneficial to our stockholders. Under the DGCL, a corporation may not, in general, engage in a business combination with any holder of 15% or more of its capital stock unless the holder has held the stock for three years or, among other things, the board of directors has approved the transaction. Any provision of our Certificate of Incorporation or Bylaws or Delaware law that has the effect of delaying or deterring a change of control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock and could also affect the price that some investors are willing to pay for our common stock.

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

Recent Sales of Unregistered Equity Securities

None.

Use of Proceeds

Not applicable.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers

None.

Item 5. Other Information

Director and Officer Trading Arrangements

During the three months ended September 30, 2025, no director or officer (as defined in Rule 16a-1(f) under the Exchange Act) of the Company adopted or terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement," as each term is defined in Item 408(a) of Regulation S-K.

Item 6. Exhibits.

Exhibit Number	Description
3.1	Amended and Restated Certificate of Incorporation (incorporated herein by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 001-38085), filed with the Commission on May 10, 2017).
3.2	Corrected Amended and Restated Certificate of Designation of Series A Convertible Preferred Stock (incorporated herein by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 001-38085), filed with the Commission on September 24, 2019).
3.3	Amended and Restated Bylaws (incorporated herein by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K (File No. 001-38085), filed with the Commission on May 10, 2017).
4.1	Form of Common Stock Certificate of the Company (incorporated herein by reference to Exhibit 4.1 to the Company's Registration Statement on Form S-1/A (File No. 333-217245), filed with the Commission on April 25, 2017).
4.2	Form of Series A Preferred Stock Certificate (incorporated herein by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K (File No. 001-38085), filed with the Commission on February 21, 2019).
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 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 and Principal 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 formatted as Inline XBRL and contained within Exhibit 101

* Filed herewith.

** Furnished herewith and not deemed to be "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and shall not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.

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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.

OVID THERAPEUTICS INC.

Date: November 12, 2025

By: /s/ Jeremy M. Levin
Jeremy M. Levin
Chief Executive Officer
(Principal Executive Officer)

Date: November 12, 2025

By: /s/ Jeffrey Rona
Jeffrey Rona
Chief Business and Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 31.1

CERTIFICATION 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

I, Jeremy M. Levin, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Ovid Therapeutics 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(s) 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(s) 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 12, 2025

By: /s/ Jeremy M. Levin
Jeremy M. Levin
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION 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

I, Jeffrey Rona, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Ovid Therapeutics 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(s) 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(s) 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 12, 2025

By: /s/ Jeffrey Rona
Jeffrey Rona
Chief Business and Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

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

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, (the "Exchange Act") and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), Jeremy M. Levin, Chief Executive Officer of Ovid Therapeutics Inc. (the "Company"), and Jeffrey Rona, Chief Business and Financial Officer of the Company, each hereby certifies that, to the best of his knowledge:

1. The Company's Quarterly Report on Form 10-Q for the period ended September 30, 2025, to which this Certification is attached as Exhibit 32.1 (the "Periodic Report"), fully complies with the requirements of Section 13(a) or Section 15(d) of the Securities Exchange Act of 1934, as amended; and

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

Date: November 12, 2025

/s/ Jeremy M. Levin	/s/ Jeffrey Rona
Jeremy M. Levin	Jeffrey Rona
Chief Executive Officer *(Principal Executive Officer)*	Chief Business and Financial Officer *(Principal Financial and Accounting Officer)*