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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2024

OR

o 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 x No o

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 x No o

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	o	Accelerated Filer	o
Non-accelerated Filer	x	Smaller Reporting Company	x
Emerging growth company	o		

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 o

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

As of November 8, 2024, the registrant had 71,009,866 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;
- 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 clinical trials and potential regulatory filings;
- our expectations regarding the potential market size 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;
- 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;
- the impact of geopolitical tensions, including war or the perception that hostilities may be imminent, adverse global economic conditions, 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; 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 TM 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, 2024	December 31, 2023
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 15,017	$ 27,042
Marketable securities	47,695	78,792
Prepaid expenses and other current assets	3,115	3,763
Total current assets	65,827	109,598
Long-term equity investments	21,125	17,626
Restricted cash	1,931	1,931
Right-of-use asset, net	13,080	13,894
Property and equipment, net	509	769
Other noncurrent assets	182	210
Total assets	$ 102,654	$ 144,027
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,710	$ 3,703
Accrued expenses	7,607	6,524
Current portion, lease liability	1,313	1,246
Total current liabilities	11,630	11,473
Long-term liabilities:		
Lease liability	13,762	14,756
Royalty monetization liability	972	30,000
Total liabilities	26,364	56,230
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, 2024 and December 31, 2023	—	—
Common stock, $ 0.001 par value; 125,000,000 shares authorized; 71,009,866 and 70,691,992 shares issued and outstanding at September 30, 2024 and December 31, 2023, respectively	71	71
Additional paid-in-capital	371,228	365,591
Accumulated other comprehensive income	36	1
Accumulated deficit	(295,045)	(277,865)
Total stockholders' equity	76,290	87,797
Total liabilities and stockholders' equity	$ 102,654	$ 144,027

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, 2024	For The Three Months Ended September 30, 2023	For The Nine Months Ended September 30, 2024	For The Nine Months Ended September 30, 2023
Revenue:				
License and other revenue	$ 173	$ 109	$ 490	$ 250
Total revenue	173	109	490	250
Operating expenses:				
Research and development	7,855	5,333	30,844	17,946
General and administrative	5,544	6,805	20,809	23,397
Total operating expenses	13,399	12,138	51,653	41,343
Loss from operations	(13,226)	(12,029)	(51,163)	(41,093)
Other income (expense), net	(780)	776	33,983	4,076
Loss before provision for income taxes	(14,006)	(11,253)	(17,180)	(37,017)
Provision for income taxes	—	—	—	—
Net loss	$ (14,006)	$ (11,253)	$ (17,180)	$ (37,017)
Net loss per share, basic	$ (0.20)	$ (0.16)	$ (0.24)	$ (0.52)
Net loss per share, diluted	$ (0.20)	$ (0.16)	$ (0.24)	$ (0.52)
Weighted-average common shares outstanding, basic	70,975,778	70,618,609	70,870,220	70,544,536
Weighted-average common shares outstanding, diluted	70,975,778	70,618,609	70,870,220	70,544,536

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, 2024	For The Three Months Ended September 30, 2023	For The Nine Months Ended September 30, 2024	For The Nine Months Ended September 30, 2023
Net loss	$ (14,006)	$ (11,253)	$ (17,180)	$ (37,017)
Other comprehensive income (loss):				
Unrealized gain (loss) on marketable securities	48	(9)	35	38
Comprehensive loss	$ (13,958)	$ (11,262)	$ (17,145)	$ (36,979)

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 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	—	—	38
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

(in thousands, except shares)	Series A Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive (Loss) Income	Accumulated Deficit	Total
	Shares	Amount	Shares	Amount				
Balance, December 31, 2022	1,250	$ —	70,466,885	$ 70	$ 357,771	$ (42)	$ (225,527)	$ 132,273
Issuance of common stock from exercise of stock options and purchases from employee stock purchase plan	—	—	24,625	—	67	—	—	67
Stock-based compensation expense	—	—	—	—	1,917	—	—	1,917
Other comprehensive income	—	—	—	—	—	48	—	48
Net loss	—	—	—	—	—	—	(13,356)	(13,356)
Balance, March 31, 2023	1,250	$ —	70,491,510	$ 70	$ 359,755	6	$ (238,883)	$ 120,948
Issuance of common stock from exercise of stock options and purchases from employee stock purchase plan	—	—	112,283	—	211	—	—	211
Stock-based compensation expense	—	—	—	—	1,949	—	—	1,949
Other comprehensive loss	—	—	—	—	—	—	—	—
Net loss	—	—	—	—	—	—	(12,408)	(12,408)
Balance, June 30, 2023	1,250	$ —	70,603,793	$ 71	$ 361,915	$ 6	$ (251,291)	$ 110,701
Issuance of common stock from exercise of stock options and purchases from employee stock purchase plan	—	—	76,758	—	224	—	—	224
Stock-based compensation expense	—	—	—	—	1,631	—	—	1,631
Other comprehensive loss	—	—	—	—	—	(9)	—	(9)
Net loss	—	—	—	—	—	—	(11,253)	(11,253)
Balance, September 30, 2023	1,250	$ —	70,680,551	$ 71	$ 363,770	$ (3)	$ (262,544)	$ 101,294

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, 2024	Nine Months Ended September 30, 2023
Cash flows from operating activities:		
Net loss	$ (17,180)	$ (37,017)
Adjustments to reconcile net loss to cash used in operating activities:		
Change in fair value of royalty monetization liability	(29,028)	—
Unrealized gain on equity investment	(3,500)	(502)
Change in accrued interest and accretion of discount on marketable securities	(2,301)	(1,536)
Stock-based compensation expense	5,016	5,496
Depreciation and amortization expense	453	428
Non-cash operating lease expense	815	767
Change in lease liability	(926)	(238)
Change in operating assets and liabilities:		
Prepaid expenses and other current assets	649	(2,262)
Security deposit	—	12
Accounts payable	(993)	(736)
Accrued expenses	1,047	1,718
Net cash used in operating activities	(45,948)	(33,870)
Cash flows from investing activities:		
Purchase of marketable securities	(56,533)	(53,928)
Sales/maturities of marketable securities	90,000	110,000
Purchase of long-term equity investment	—	(10,000)
Purchases of property and equipment	(49)	(27)
Software development and other costs	(116)	(110)
Net cash provided by investing activities	33,302	45,935
Cash flows from financing activities:		
Proceeds from exercise of options and purchases from employee stock purchase plan	622	502
Net cash provided by financing activities	622	502
Net (decrease) increase in cash, cash equivalents and restricted cash	(12,024)	12,568
Cash, cash equivalents and restricted cash, at beginning of period	28,972	46,799
Cash, cash equivalents and restricted cash, at end of period	$ 16,948	$ 59,367

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 improving the lives of people affected by brain conditions with significant unmet need.

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. The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development and regulatory success, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations, and the ability to secure additional capital to fund operations.

The Company's major sources of cash have been licensing revenue, proceeds from various public and private offerings of its capital stock, option exercises and interest income. As of September 30, 2024, the Company had $ 62.7 million in cash, cash equivalents and marketable securities. Since its founding, the Company has generated $ 223.4 million in 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 has an accumulated deficit of $ 295.0 million as of September 30, 2024, working capital of $ 54.2 million and net cash used in operating activities of $ 45.9 million for the nine months ended September 30, 2024.

The Company recorded net losses of $ 14.0 million and $ 17.2 million during the three and nine months ended September 30, 2024, respectively, and expects to incur losses in subsequent periods 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. Management believes that the Company's existing cash, cash equivalents and marketable securities as of September 30, 2024 will be sufficient to fund its current operating plans through at least the next 12 months from the date of filing of this Quarterly Report on Form 10-Q. Adequate additional capital to fund the operations of the Company may not be available on acceptable terms or at all. The failure to raise capital as and when needed could have a negative impact on the Company's financial condition and ability to pursue its business strategy. If unsuccessful in raising additional capital, the Company may be required to delay, reduce the scope of or eliminate additional research and development programs, or obtain funds through arrangements with collaborators or others that may require the Company to relinquish rights to certain drug candidates that the Company might otherwise seek to develop or commercialize independently.

The Company is subject to other challenges and risks specific to its business and its ability to execute on its 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: delays or problems in the supply of the Company's product candidates, loss of single source suppliers or failure to comply with manufacturing regulations; 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; complying with applicable regulatory requirements; obtaining regulatory approval of any of the Company's potential product candidates, among others.

NOTE 2 – SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

The Company's significant accounting policies are described in Note 2, "Summary of Significant Accounting Policies," in the Company's Annual Report on Form 10-K filed with the U.S. Securities and Exchange Commission ("SEC") on March 8, 2024.

(A) Unaudited Interim Condensed Consolidated Financial Statements

The interim condensed consolidated balance sheet at September 30, 2024 and the condensed consolidated statements of operations, comprehensive loss, cash flows, and stockholders' equity for the three and nine months ended September 30, 2024 and 2023 are unaudited. The accompanying unaudited condensed consolidated financial statements

have been prepared in accordance with U.S. generally accepted accounting principles ("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 month periods ended September 30, 2024 and 2023 are not necessarily indicative of the results to be expected for the year ending December 31, 2024 or for any other future annual or interim period. The balance sheet as of December 31, 2023 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, 2023 included in the Company's Annual Report on Form 10-K.

(B) Basis of Presentation and Consolidation

The accompanying 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 non-current 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, 2024 and December 31, 2023, the equity investment in Gensaic had a carrying value of $ 5.1 million. As of September 30, 2024 and December 31, 2023, the equity investment in Graviton had a carrying value of $ 15.8 million and $ 11.2 million, respectively. The initial investment in Graviton was $ 10.0 million, and cumulative measurement adjustments totaling $ 5.8 million have been recognized. The Company's equity investments are assessed quarterly and increases have been based upon change in observable prices.

Long-term equity investments also consist of an equity investment in the common shares of Marinus Pharmaceuticals, Inc. ("Marinus", Nasdaq: MRNS) that were received as non-cash 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 September 30, 2024 and December 31, 2023, the equity investment in Marinus had a carrying value of $ 0.2 million and $ 1.3 million, respectively.

(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 and equity securities totaling $ 13.5 million as of September 30, 2024 and $ 25.7 million as of December 31, 2023.
- 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 $ 47.7 million as of September 30, 2024 and $ 78.8 million as of December 31, 2023.
- 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. The Company's Level 3 liabilities consist of a royalty monetization liability that was valued at $ 972,000 as of September 30, 2024 and $ 30.0 million as of December 31, 2023. There were no Level 3 assets as of September 30, 2024 or December 31, 2023.

The following table presents information about liabilities measured at fair value on a recurring basis and for which the Company utilizes Level 3 inputs to determine fair value.

Royalty Monetization Liability

(in thousands)	**September 30, 2024**	
Balance, January 1, 2024	$	30,000
Change in fair value of royalty monetization liability		(29,028)
Balance, September 30, 2024	$	972

The Company estimated the fair value of the royalty monetization liability using a probability-weighted discounted cash flow valuation based on the estimated future sales of soticlestat. Using this approach, the estimated future sales of soticlestat are calculated over the expected life of the agreement using certain unobservable inputs. The unobservable inputs include: the estimated probability of FDA approval for commercialization of soticlestat, the estimated future sales forecast for soticlestat, and the discount rate used to present value the probability-weighted estimated future sales of soticlestat.

The royalty monetization liability is classified as a Level 3 liability as its valuation requires substantial judgment and estimation of factors that are not observable. If different input assumptions were used for the valuation, the estimated fair value could be significantly higher or lower than the fair value determined. See "Royalty Monetization Liability" in this Note 2 (L) below.

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) 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 to employees 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 significant 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 employee 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 accounts for option awards granted to nonemployee consultants and directors in accordance with ASC 718. The fair value of the option issued or committed to be issued is used to measure the transaction, as this is more reliable than the fair value of the services received. The fair value is measured at the value of the Company’s common stock at the earlier of the date that the commitment for performance by the counterparty has been reached or the counterparty’s performance is complete.

(L) Royalty Monetization Liability

The Company accounted for its sale to Ligand Pharmaceuticals Incorporated (“Ligand”) under the purchase and sale agreement (the “Ligand Agreement”) of a 13 % share of royalties and milestones owed to the Company pursuant to the RLT Agreement with Takeda related to the potential approval and commercialization of soticlestat in accordance with ASC 470, Debt, which addresses situations in which an entity receives cash from an investor in return for an agreement to pay the investor a specified percentage of the revenue from a contractual right. The Company classified 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 in accordance with ASC 825, Financial Instruments, which permits a company to elect the fair value option on an instrument specific basis for a recognized financial liability that is not specifically excluded

If commercialized, the Company will recognize 100 % of the royalties and milestones received for sales of soticlestat as revenue and the 13 % share of royalties payable to Ligand as a cash outflow from financing activities in the condensed consolidated statements of cash flows. Changes in the fair value of the debt will be classified as a component of other income (expense), net in the condensed consolidated statements of operations.

In June 2024, Takeda reported Phase 3 topline 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. Based on the study results, the Company’s management reassessed certain assumptions for soticlestat that factor into the valuation of the royalty monetization liability and determined the probability of potential commercialization related to Dravet syndrome to be 7.5 % and the probability of potential commercialization related to Lennox-Gastaut syndrome to be 0 %. A discount rate

of 15 % was utilized in calculating the change to the royalty monetization liability. The impact on the fair value resulted in a $ 29.0 million reduction in the royalty monetization liability in June 2024 which was recognized as other income (expense), net in the statement of operations during the nine months ended September 30, 2024.

Given that the two Phase 3 Takeda trials evaluating soticlestat for the treatment of Dravet and Lennox-Gastaut syndromes did not meet their primary endpoints, it is uncertain whether Takeda will continue to progress, or elect to terminate the development of soticlestat as contemplated by the RLT Agreement, in which case we may not receive the royalty and milestone payments under the RLT Agreement. See Note 11 – Collaboration and License Agreements for a description of the RLT Agreement.

(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 per Share

Net loss per common share is determined by dividing net loss attributable to common stockholders by the basic and diluted weighted-average common shares outstanding during the period. The Company applies the two-class method to allocate earnings between common stock and participating securities.

When applicable, net 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 using the treasury-stock method and the potential impact of preferred stock using the if-converted method.

(O) 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 if the performance obligations are not satisfied.

(P) Recent Accounting Pronouncements

The Company has reviewed recently issued accounting standards and plans to adopt those that are applicable. The Company will adopt ASU 2023-07, Segment Reporting (Topic 280): “Improvements to Reportable Segment Disclosures” (“ASU 2023-07”), beginning with its fiscal year ended December 31, 2024. ASU 2023-07 introduces a new

requirement to disclose significant segment expenses regularly provided to the chief operating decision maker ("CODM"), extends certain annual disclosures to interim periods, clarifies that single reportable segment entities must apply ASC 280 in its entirety, permits more than one measure of segment profit or loss to be reported under certain conditions, and requires disclosure of the title and position of the CODM. The Company is evaluating the potential impact of these changes and will include the disclosures as required.

The Company adopts new pronouncements relating to generally accepted accounting principles applicable to the Company as they are issued, which may be in advance of their effective date. 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 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, 2024 and December 31, 2023:

	September 30, 2024			
(in thousands)	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Fair Value
Cash	$ 1,780	$ —	$ —	$ 1,780
Cash equivalents	13,237	—	—	13,237
Marketable securities	47,659	36	—	47,695
Total cash, cash equivalents and marketable securities	$ 62,676	$ 36	$ —	$ 62,712

	December 31, 2023			
(in thousands)	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Fair Value
Cash	$ 2,701	$ —	$ —	$ 2,701
Cash equivalents	24,340	—	—	24,340
Marketable securities	78,791	1	—	78,792
Total cash, cash equivalents and marketable securities	$ 105,832	$ 1	$ —	$ 105,833

The Company did not hold any securities that were in an unrealized loss position for more than 12 months as of September 30, 2024 and December 31, 2023.

There were no material realized gains or losses on available-for-sale securities during the nine months ended September 30, 2024 and 2023.

NOTE 4 – PROPERTY AND EQUIPMENT AND INTANGIBLE ASSETS

Property and equipment is summarized as follows:

(in thousands)	September 30, 2024	December 31, 2023
Furniture and equipment	$ 1,513	$ 1,463
Leasehold improvements	306	306
Less accumulated depreciation	(1,310)	(1,000)
Total property and equipment, net	$ 509	$ 769

Depreciation expense was $ 105,000 and $ 104,000 for the three months ended September 30, 2024 and 2023, respectively. Depreciation expense was $ 309,000 and $ 318,000 for the nine months ended September 30, 2024 and 2023, respectively.

Intangible assets, net of accumulated amortization, were $ 155,000 and $ 186,000 as of September 30, 2024 and December 31, 2023, respectively, and are included in other assets. Amortization expense was $ 64,000 and $ 41,000 for the three months ended September 30, 2024 and 2023, respectively. Amortization expense was $ 144,000 and $ 110,000 for the nine months ended September 30, 2024 and 2023, respectively.

NOTE 5 – LEASES

During 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 10 months following the commencement date of the lease, or January 10, 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 nine 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, 2024**
ROU asset, net	$	13,080
Current lease liability		1,313
Long-term lease liability	$	13,762

The components of operating lease cost for the nine months ended September 30, 2024 were as follows:

(in thousands)		**September 30, 2024**
Operating lease cost	$	1,625
Variable lease cost		—
Short-term lease cost		—

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

(in thousands)		
2024	$	579
2025		2,316
2026		2,316
2027		2,316
2028		2,469
Thereafter		9,878
Total lease payments	$	19,874

NOTE 6 – ACCRUED EXPENSES

Accrued expenses consist of the following:

(in thousands)	September 30, 2024	December 31, 2023
Payroll and bonus accrual	$ 4,305	$ 4,277
Research and development accrual	2,634	1,396
Professional fees accrual	339	522
Other	329	329
Total	$ 7,607	$ 6,524

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 of the 10,000,000 authorized shares of preferred stock as non-voting Series A Convertible Preferred Stock ("Series A Preferred Stock"), of which 1,250 are issued and outstanding.

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, 2024 and December 31, 2023. 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.

Dividends

Through September 30, 2024, 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, 2024 and January 1, 2023 an additional 3,534,599 and 3,523,344 shares, respectively, were reserved for issuance under the 2017 Plan. As of September 30, 2024, there were 5,569,161 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, 2024 and 2023, 38,289 and 33,931 shares were purchased under the 2017 ESPP, respectively, and the Company recorded expense of $ 6,000 and $ 14,000 , respectively. During the nine months ended September 30, 2024 and 2023, 69,850 and 63,761 shares were purchased under the 2017 ESPP, respectively, and the Company recorded expense of $ 40,000 and $ 42,000 , 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, 2024 and January 1, 2023 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, 2024, there were 282,996 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, 2024, options to purchase 1,328,715 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, 2024 and 2023 was estimated using the Black-Scholes option valuation model. The inputs for the Black-Scholes option valuation model require significant 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. Beginning January 1, 2023, the expected volatility is estimated based on the historical volatility of the Company since the Company's initial public offering.

The Company granted 1,727,660 and 4,000 stock options to employees during the three months ended September 30, 2024 and 2023, respectively. The Company granted 4,229,810 and 2,934,500 stock options to employees during the nine months ended September 30, 2024 and 2023, respectively. There were 6,161,479 and 5,985,002 unvested employee options outstanding as of September 30, 2024 and 2023, respectively. Total expense recognized related to the employee stock options for the three months ended September 30, 2024 and 2023 was $ 1.2 million and $ 1.5 million, respectively. Total expense recognized related to the employee stock options for the nine months ended September 30, 2024 and 2023 was $ 4.8 million and $ 5.1 million, respectively. Total unrecognized compensation expense related to employee stock options was $ 10.9 million as of September 30, 2024. The Company did not recognize any expense for employee performance-based option awards during the three and nine months ended September 30, 2024 and 2023.

The Company granted 20,000 and zero stock options to nonemployee consultants for services rendered during the three months ended September 30, 2024 and 2023, respectively. The Company granted 270,000 and 70,000 stock options to nonemployee consultants for services rendered during the nine months ended September 30, 2024 and 2023, respectively. There were 59,167 and 99,792 unvested nonemployee options outstanding as of September 30, 2024 and 2023, respectively. Total expense recognized related to nonemployee stock options for the three months ended September 30, 2024 and 2023 was $ 68,000 and $ 106,000 , respectively. Total expense recognized related to nonemployee stock options for the nine months ended September 30, 2024 and 2023 was $ 226,000 and $ 378,000 , respectively. Total unrecognized compensation expenses related to the nonemployee stock options was $ 0.1 million as of September 30, 2024.

The Company did not recognize any expense for nonemployee performance-based option awards during the three and nine months ended September 30, 2024 and 2023.

The Company granted zero and 348,575 restricted stock units to employees during the three and nine months ended September 30, 2024. No restricted stock units were granted by the Company in prior periods. The restricted stock units granted will vest in equal installments over three years , beginning January 1, 2025 and otherwise have similar terms to the Company's stock option grants.

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

	Three Months Ended		Nine Months Ended	
(in thousands)	September 30, 2024	September 30, 2023	September 30, 2024	September 30, 2023
Research and development	$ 241	$ 345	$ 1,307	$ 4,061
General and administrative	1,067	1,286	3,709	1,435
Total	$ 1,308	$ 1,631	$ 5,016	$ 5,496

	Three Months Ended		Nine Months Ended	
(in thousands)	September 30, 2024	September 30, 2023	September 30, 2024	September 30, 2023
Stock options and restricted stock units	$ 1,301	$ 1,617	$ 4,976	$ 5,454
Employee Stock Purchase Plan	7	14	40	42
Total	$ 1,308	$ 1,631	$ 5,016	$ 5,496

The fair value of employee options granted during the three and nine months ended September 30, 2024 and 2023 was estimated utilizing the following assumptions:

	Three Months Ended		Nine Months Ended	
	September 30, 2024	September 30, 2023	September 30, 2024	September 30, 2023
	Weighted Average	Weighted Average	Weighted Average	Weighted Average
Volatility	98.59 %	83.16 %	87.50 %	84.56 %
Expected term in years	5.72	6.08	5.91	6.07
Dividend rate	0.00 %	0.00 %	0.00 %	0.00 %
Risk-free interest rate	4.00 %	4.19 %	4.20 %	3.97 %
Fair value of option on grant date	$ 0.82	$ 2.61	$ 1.88	$ 1.92

The fair value of nonemployee options granted during the three and nine months ended September 30, 2024 and 2023 was estimated utilizing the following assumptions:

	Three Months Ended		Nine Months Ended	
	September 30, 2024	September 30, 2023	September 30, 2024	September 30, 2023
	Weighted Average	Weighted Average	Weighted Average	Weighted Average
Volatility	98.53 %	— %	81.92 %	83.73 %
Expected term in years	5.50	0.00	6.43	5.32
Dividend rate	0.00 %	0.00 %	0.00 %	0.00 %
Risk-free interest rate	3.51 %	— %	4.29 %	3.86 %
Fair value of option on grant date	$ 0.83	$ —	$ 2.57	$ 2.21

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, 2023	15,124,546	$ 3.87	6.90	$ 5,212,286
Granted	4,499,810	2.65	5.75	
Exercised	(248,024)	3.13		
Forfeited or expired	(2,340,187)	4.08		
Options outstanding September 30, 2024	17,036,145	$ 3.51	5.75	$ 234,385
Vested and exercisable at September 30, 2024	10,815,499	$ 4.03	4.48	$ —

At September 30, 2024, there was $ 11.0 million of unrecognized stock–based compensation expense related to employee and nonemployee grants, which is expected to be recognized over a remaining average vesting period of 2.28 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, 2024, the Company was in a pre-tax loss position and is anticipated to remain so throughout the year. For the nine months ended September 30, 2024, the Company did no t 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, 2024.

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 a collaboration and option agreement ("Gensaic Collaboration Agreement") with Gensaic. The Gensaic Collaboration Agreement involves the research and development of phage-derived

particle (“PDP”) products on Gensaic’s proprietary platform for certain rare central nervous system (“CNS”) disorder targets.

Under the Gensaic Collaboration Agreement, Gensaic grants the Company an exclusive option to obtain an exclusive license with respect to certain identified lead PDP products, which are 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 Gensaic’s 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.

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, 2024, 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 partners, which led to a fraudulent transfer totaling $ 1.8 million to a third-party impersonating one of the Company’s development partners. The matter has been reported to the U.S. Secret Service and Federal Bureau of Investigation. Based on the information the Company has received from its banking institution; the Company believes the receiving account has been frozen. The Company has also received information suggesting that the account to which the funds were remitted may have been blocked under U.S. sanctions administered by the U.S. Office of Foreign Asset Control (“OFAC”) and the Company is seeking to confirm, in which case, it will consider submitting an application for release of the blocked funds to OFAC. The Company is working with law enforcement authorities and the banks involved in the funds transfer to pursue recovery of the $ 1.8 million, but at this time the Company does not know whether it will be able to recover such funds and, thus, the Company has recognized a loss of $ 1.8 million in the three and nine months ended September 30, 2024. The Company is also assessing recovery under its insurance policies.

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, would be eligible to receive up to an additional $ 660.0 million upon Takeda achieving developmental, regulatory and sales milestones. In addition, the Company would be entitled to receive tiered royalties beginning in the low double-digits, and up to 20 % on sales of soticlestat if regulatory approval is achieved. Royalties would be payable on a country-by-country and product-by-product basis for any indications for which soticlestat was approved and sold 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.

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. In the event that soticlestat is not approved and commercialized, the Company has no continuing debt or other obligations to Ligand.

In June 2024, Takeda reported the Phase 3 topline study results for soticlestat, noting that soticlestat missed its primary endpoints with respect to Dravet and Lennox-Gastaut syndromes. Based on the study results, management reassessed certain assumptions for soticlestat that factor into the valuation of the royalty monetization liability and determined the probability of potential commercialization related to Dravet syndrome to be 7.5 % and the probability of potential commercialization related to Lennox-Gastaut syndrome to be 0 %. The change in valuation assumptions resulted in a $ 29.0 million reduction in the royalty monetization liability in June 2024 which was recognized as other income in the condensed consolidated statement of operations during the nine months ended September 30, 2024.

During the nine months ended September 30, 2024 and 2023, no revenue or expense was recognized pursuant to the RLT Agreement.

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 our relevant intellectual property rights. At the end of the one-year option period, Healx has 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. On February 1, 2023, the Company granted an extension of the option period for up to four months for Healx to continue to investigate gaboxadol. Royalties 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.

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.

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, or the 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, or the 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 the net profit share 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 a share of all milestone and royalty payments.

No revenue was recognized relating to the Healx License and Option Agreement during the nine months ended September 30, 2024 and 2023.

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 and unrealized gains of $ 0.1 million for the nine months ended September 30, 2024 and 2023, respectively, which were recorded as unrealized gains (losses) on equity securities and are reflected in other income (expense), net in the condensed consolidated statements of operations.

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 Rho-associated coiled-coil containing protein kinase 2 (“ROCK2”) inhibitors including their lead program OV888/GV101 in rare CNS disorders (excluding amyotrophic lateral sclerosis) worldwide (excluding China, Hong Kong, Macau and Taiwan). Under the Graviton Agreement, the Company and Graviton are investigating OV888/GV101 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 observable changes in price and recorded the gains in other income (expense), net, in the condensed consolidated statements of operations.

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

Basic net loss per share is calculated based upon the weighted-average number of common shares outstanding during the period, excluding outstanding stock options that have not yet vested. For any period in which the Company records net income, diluted net income per share is calculated based upon the weighted-average number of common shares outstanding during the period plus the dilutive impact of weighted-average common equivalent shares outstanding during the period resulting from the assumed exercise of outstanding stock options determined under the treasury stock method and the assumed conversion of preferred stock into common shares determined using the if-converted method. Diluted net loss per share is equivalent to the basic net loss per share due to the exclusion of outstanding stock options and convertible preferred stock because the inclusion of these securities would result in an anti-dilutive effect on per share amounts.

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 participating securities.

For any period in which the Company records net income, undistributed earnings allocated to the participating securities are subtracted from net income in determining net income attributable to common stockholders. The undistributed earnings have been allocated based on the participation rights of preferred stock and common shares as if the earnings for the year have been distributed. For periods in which the Company recognizes a net loss, undistributed losses are allocated only to common shares as the participating securities do not contractually participate in the Company's losses. Basic net loss per share is computed by dividing the net loss attributable to common stockholders by the weighted-average number of common shares outstanding during the period. Participating securities are excluded from basic weighted-average common shares outstanding.

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

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
(in thousands)	2024	2023	2024	2023
Net loss	$ (14,006)	$ (11,253)	$ (17,180)	$ (37,017)
Net loss attributable to participating securities	—	—	—	—
Net loss attributable to common stockholders	$ (14,006)	$ (11,253)	$ (17,180)	$ (37,017)

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
(in thousands, except share and per share data)	2024	2023	2024	2023
Net loss attributable to common stockholders	$ (14,006)	$ (11,253)	$ (17,180)	$ (37,017)
Weighted average common shares outstanding used in computing net loss per share - basic	70,975,778	70,618,609	70,870,220	70,544,536
Weighted average common shares outstanding used in computing net loss per share - diluted	70,975,778	70,618,609	70,870,220	70,544,536
Net loss per share, basic	$ (0.20)	$ (0.16)	$ (0.24)	$ (0.52)
Net loss per share, diluted	$ (0.20)	$ (0.16)	$ (0.24)	$ (0.52)

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

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2024	2023	2024	2023
Stock options to purchase common stock	17,036,145	15,164,877	17,036,145	15,164,877
Common stock issuable upon conversion of Series A convertible preferred stock	1,250,000	1,250,000	1,250,000	1,250,000

NOTE 14 – ORGANIZATIONAL RESTRUCTURING

In June 2024, the Company initiated a reduction of its workforce to prioritize its programs and extend its cash runway. The decision, which was approved by the Company's Board of Directors, was precipitated by Takeda's report of Phase 3 topline study results for soticlestat, which are described in Note 11 – Collaboration and License Agreements. The workforce reduction included 17 impacted individuals, or approximately 43 % of the Company's headcount, and was primarily recognized in the three month period ended June 30, 2024. Severance costs of $ 3.7 million related to the organizational restructuring were recognized during the nine months ended September 30, 2024, and included in loss from operations in the line items of the employees' regular compensation in the condensed consolidated statements of operations. Accrued severance costs of $ 2.4 million are included in accrued expenses within the condensed consolidated balance sheet. Cash payments related to these costs are anticipated to be substantially complete in the second quarter of 2025.

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, 2023, which was filed with the Securities and Exchange Commission ("SEC") on March 8, 2024. 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 committed to developing medicines for brain conditions with significant unmet need in a manner that is mechanistically driven, scientifically rigorous and patient focused. Our differentiated pipeline of potential small molecule medicines has produced four unique programs to date, three of which we are actively developing, and the fourth, which we co-developed, was subsequently repurchased by Takeda Company Limited ("Takeda") in 2021. Central to our strategy is identifying and developing differentiated mechanisms of action to interdict unaddressed biological targets in the brain that are fundamental to disease pathology. This strategy has achieved a pipeline inclusive of: OV329, a potential best-in-class next generation GABA aminotransferase inhibitor; a library of direct activators of the potassium chloride co-transporter 2 (KCC2), including the first candidate, OV350; and OV888/GV101, a rho-associated coiled-coil containing protein kinase 2 (ROCK2) inhibitor. This pipeline was curated using an integrated and disciplined approach to business development, research, and clinical development. The development programs in our pipeline act upon factors modulating neuronal hyperexcitability, neuroinflammation and neurovascular anomalies, which we believe underlie seizures, psychoses and other neurological and neurodegenerative conditions.

Our management team has substantial understanding of neurological conditions, drug development and commercialization gained from collective experience and contributions to the development and launch of more than 25 approved medicines in their respective careers prior to joining Ovid. These launches include many approved medicines for disorders of the brain. Our efforts have produced multiple clinical-stage development programs. Two of our three active programs, OV888/GV101 and OV329, have advanced into human studies. We expect to submit an investigational new drug ("IND") application, or the equivalent in jurisdictions outside of the U.S., to begin clinical trials for the third program, OV350, in the fourth quarter of 2024.

For each of our programs we focus on generating data using relevant disease models and thorough toxicology studies to build a replicable and scalable approach to develop small molecule candidates, which begins with conducting animal disease models and toxicology studies in the preclinic to build convincing safety, mechanistic and therapeutic evidence before moving into human studies. While the therapeutic potential of the unique mechanisms of action within each of our pipeline programs is quite broad, we typically seek to implement cost-efficient and accelerated proof-of-concept programs in rare disease populations. This approach provides evidence and de-risking information prior to moving into larger population indications that would require costlier trials. When possible and scientifically relevant, we implement assessments of target engagement and biomarkers into our development programs. Our cohesive focus in brain disorders caused by hyper-excitabilty, neuro-inflammation and neuro-vascular dysfunction reinforces our belief that we can develop and produce multiple novel medicines, scale our infrastructure, and thereby succeed in our mission.

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 operating assets and raising capital. We have historically funded our business primarily through the sale of our capital stock. Through September 30, 2024, we have raised net proceeds of $275.4 million from the sale of our capital stock. We have also, in previous periods, generated revenue through license and collaboration agreements, including $196.0 million from our royalty, license and termination agreement ("RLT Agreement") with Takeda Pharmaceutical Company Limited ("Takeda") and $30.0 million from our purchase and sale agreement ("Ligand Agreement") with Ligand Pharmaceuticals Incorporated ("Ligand"). As of September 30, 2024, we had $62.7 million in cash, cash equivalents and marketable securities and an accumulated deficit of $295.0 million.

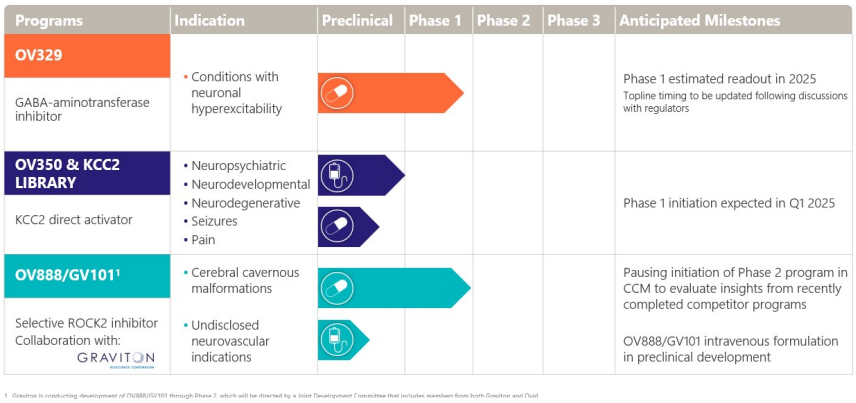
We expect to incur significant expenses and operating losses for at least the next several years. Our net losses may fluctuate significantly from period to period, depending on the timing of our planned clinical trials and expenditures on our other research and development and commercial development 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.

Our Pipeline

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



1 Graviton is conducting development of OV888/GV101 through Phase 2, which will be directed by a Joint Development Committee that includes members from both Graviton and Ovid

OV329, a next-generation GABA-aminotransferase inhibitor

In December 2022, we initiated a Phase 1 SAD/MAD study in healthy volunteers for OV329, a next-generation GABA-aminotransferase inhibitor. Our development of OV329 seeks to achieve a profile that reduces neuronal hyperexcitability with less sedation and without ocular changes that are associated with an older drug in the class, vigabatrin. This profile, supported by our preclinical characterization, suggests that OV329 is more potent, delivers synaptic and extrasynaptic inhibition, and has a lasting pharmacodynamic effect with rapid tissue clearance.

We are executing the multiple ascending dose portion of the Phase 1 study as planned and no serious adverse events have been observed. Based upon supportive human safety data to date, we will explore with regulators adding additional cohorts to increase dosing opportunities for future Phase 2 programs. We anticipate this will delay topline data until 2025, and we will provide more detailed timelines following discussions with regulators. When completed, the Phase 1 results should include results on safety, tolerability, and two biomarkers that may provide a signal for target engagement and clinical effect, using magnetic resonance spectroscopy and transcranial magnetic stimulation, respectively.

To evaluate OV329's profile compared to vigabatrin, a first-generation GABA-AT inhibitor, we conducted additional animal experiments to evaluate the risk of drug accumulation in the eye and causing retinal cell dysregulation. At the Epilepsy Pipeline Conference in September 2024, we presented results from an animal study demonstrating that OV329 did not accumulate in animal eyes in contrast with vigabatrin. OV329 was present in the brain plasma of mice and then rapidly cleared the tissue and remained undetectable in the retina, eye, and brain. In contrast, vigabatrin was demonstrated to accumulate in the eye, retina and brain in less than 48 hours, which is consistent with previously published

independent research. The full results from this head-to-head study will be presented at the American Epilepsy Society conference in December 2024.

KCC2 direct activator library and OV350

We are developing a library of direct activators of the potassium chloride co-transporter 2 (KCC2), a biologic target that is fundamental to hyper-excitabilty and which is implicated in a broad range of neurological, neuropsychiatric, neurodevelopmental and neurodegenerative indications. The library contains multiple compounds, which have unique therapeutic characteristics and are amenable for oral, intramuscular and intravenous formulations to address clinical indications across the care continuum. We are conducting multiple non-clinical studies to characterize the therapeutic potential and safety of these compounds and intend to rapidly advance the most promising candidates successively into human studies. We anticipate the library will generate multiple clinical development programs in the next 2-3 years.

We expect to submit a regulatory application for a Phase 1 trial of the first candidate from the library, OV350, before year end 2024, and we anticipate initiating first-in-human studies for this class of molecule in the first quarter of 2025. We intend to evaluate the safety, tolerability and pharmacokinetic parameters of an IV formulation of OV350 in healthy human volunteers. In preclinical and animal disease models, OV350 has demonstrated anti-psychotic and anticonvulsant effects, indicating it may have broad therapeutic utility.

ROCK2 inhibition programs with Graviton Bioscience

In May 2023, we in-licensed OV888/GV101 and a library of highly selective Rho-associated coiled-coil containing protein kinase 2 (ROCK2) inhibitors from Graviton Bioscience Corporation (“Graviton”) and entered into a research and collaboration agreement with Graviton. Under the collaboration, Graviton is responsible for developing the programs through Phase 2 development and we will then assume responsibility for Phase 3 development and commercialization. The first program from this collaboration is OV888/GV101, a hard gel capsule. With Graviton, we announced positive topline data from a Phase 1 multiple-ascending dose study of OV888/GV101 on July 1, 2024 and originally intended to initiate a signal-finding trial in people living with cerebral cavernous malformations in the second half of 2024.

However, following the recent completion of competitor trials in CCM, and after discussions with key stakeholders, together with Graviton, we are pausing the initiation of the Phase 2 proof-of-concept study of OV888/GV101 in CCM. With Graviton, we received regulatory clearance to initiate a Phase 2 program, but we will take a strategic pause to evaluate clinical design learnings emerging from competitor Phase 2 programs. We will seek to optimize future development approaches with the benefit of further insights on study duration, enrichment strategies, endpoints, and time-to-event measurements. We continue to pursue additional formulations of OV888/GV101 for undisclosed cerebral vascular conditions with high unmet need.

Soticlestat, a cholesterol 24-hydroxylase inhibitor

In 2021, we sold our rights to soticlestat to Takeda, who studied it in two pivotal Phase 3 trials in Lennox-Gastaut syndrome and Dravet syndrome. In June 2024, Takeda announced the two Phase 3 Takeda trials evaluating soticlestat for the treatment of Dravet and Lennox-Gastaut syndromes did not meet their primary endpoints. It is uncertain whether Takeda will continue to progress or elect to terminate the development of soticlestat as contemplated by the RLT Agreement, in which case we may not receive some or all of the royalty and milestone payments under the RLT Agreement. Takeda has publicly announced plans to engage with regulatory authorities to discuss the totality of the data generated by the study in Dravet syndrome to determine next steps.

Significant Risks and Uncertainties

The global economic slowdown, the overall disruption of global healthcare systems and other risks and uncertainties associated with bank failures, public health crises and global geopolitical tensions, like the ongoing war between Russia and Ukraine and the war involving Israel, may have a material adverse effect on our business, financial condition, results of operations and growth prospects. The resulting high 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. Elevated 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 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 the ongoing war between Russia and Ukraine and the war involving

Israel, worsening global macroeconomic conditions and employee availability and wage increases, which may result in additional stress on our working capital resources.

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; and the challenges of protecting and enhancing our intellectual property rights; and complying with applicable regulatory requirements.

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 effort;
- 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 candidate.

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, unrealized gains (losses) on long-term equity investments, an unrealized loss due to a fraudulent funds transfer and change in fair value of royalty monetization liability.

Results of Operations

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

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

(in thousands)	Three Months Ended September 30, 2024	Three Months Ended September 30, 2023	Change $
Revenue:			
License and other revenue	$ 173	$ 109	$ 64
Total revenue	173	109	64
Operating expenses:			
Research and development	7,855	5,333	2,522
General and administrative	5,544	6,805	(1,261)
Total operating expenses	13,399	12,138	1,261
Loss from operations	(13,226)	(12,029)	(1,197)
Other income (expense), net	(780)	776	(1,556)
Loss before provision for income taxes	(14,006)	(11,253)	(2,753)
Provision for income taxes	—	—	—
Net loss	$ (14,006)	$ (11,253)	$ (2,753)

Revenue

Revenue of $173,000 and $109,000 was recorded in the three months ended September 30, 2024 and 2023, respectively, which consisted of royalties on net sales pursuant to an out-licensing agreement.

Research and Development Expenses

(in thousands)	Three Months Ended September 30, 2024	Three Months Ended September 30, 2023	Change $
Preclinical and clinical development expenses	$ 5,526	$ 2,140	$ 3,386
Payroll and payroll-related expenses	1,631	2,297	(666)
Other expenses	698	895	(197)
Total research and development	$ 7,855	$ 5,333	$ 2,522

During the three months ended September 30, 2024, research and development expenses were $7.9 million, compared to $5.3 million for the same period in 2023. The increase of $2.5 million primarily relates to expenses associated with the acceleration of preclinical and clinical research and development in OV350 and OV888/GV101.

General and Administrative Expenses

(in thousands)	Three Months Ended September 30, 2024	Three Months Ended September 30, 2023	Change $
Payroll and payroll-related expenses	$ 2,707	$ 3,567	$ (860)
Legal and professional fees	1,549	1,908	(359)
General office expenses	1,288	1,330	(42)
Total general and administrative	$ 5,544	$ 6,805	$ (1,261)

General and administrative expenses were $5.5 million and $6.8 million for the three months ended September 30, 2024 and 2023, respectively. The decrease in general and administrative expenses is primarily due to the organizational restructuring executed during the second quarter of 2024, inclusive of payroll and related expenses as well as other cost reductions.

Other Income (Expense), net

Other income (expense), net for the three months ended September 30, 2024 and 2023 was a $0.8 million loss and a $0.8 million gain, respectively. The decrease of $1.6 million was primarily due to an unrealized loss of $1.8 million due to a fraudulent funds transfer, offset by unrealized gain (loss) on long-term equity investments and interest earned and accretion of discount on marketable securities.

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

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

	Nine Months Ended September 30, 2024	Nine Months Ended September 30, 2023	Change $
		(in thousands)	
Revenue:			
License and other revenue	$ 490	$ 250	$ 240
Total revenue	490	250	240
Operating expenses:			
Research and development	30,844	17,946	12,898
General and administrative	20,809	23,397	(2,588)
Total operating expenses	51,653	41,343	10,310
Loss from operations	(51,163)	(41,093)	(10,070)
Other income (expense), net	33,983	4,076	29,907
Loss before provision for income taxes	(17,180)	(37,017)	19,837
Provision for income taxes	—	—	—
Net loss	$ (17,180)	$ (37,017)	$ 19,837

Revenue

Revenue of $490,000 was generated in the nine months ended September 30, 2024, compared to revenue of $250,000 recognized in the same period in 2023. Revenue in both periods consisted of royalties on net sales pursuant to an out-licensing agreement.

Research and Development Expenses

	Nine Months Ended September 30, 2024	Nine Months Ended September 30, 2023	Change $
		(in thousands)	
Preclinical and clinical development expenses	$ 20,186	$ 7,754	$ 12,432
Payroll and payroll-related expenses	8,466	7,698	768
Other expenses	2,192	2,494	(302)
Total research and development	$ 30,844	$ 17,946	$ 12,898

During the nine months ended September 30, 2024, research and development expenses were $30.8 million compared to $17.9 million for the same period in 2023. The increase of $12.9 million primarily relates to $10.7 million in expenses associated with the acceleration of preclinical and clinical research and development in OV329, OV350 and OV888/GV101 as well as costs associated with the organizational restructuring announced in June 2024. Payroll and payroll related costs of the organizational restructuring of $1.7 million were recognized during the nine months ended September 30, 2024, but will be paid over an extended period.

General and Administrative Expenses

	Nine Months Ended September 30, 2024	Nine Months Ended September 30, 2023	Change $
	(in thousands)		
Payroll and payroll-related expenses	$ 11,434	$ 13,343	$ (1,909)
Legal and professional fees	5,362	5,404	(42)
General office expenses	4,013	4,650	(637)
Total general and administrative	$ 20,809	$ 23,398	$ (2,588)

General and administrative expenses were $20.8 million for the nine months ended September 30, 2024 compared to $23.4 million for the same period in 2023. The decrease of $2.6 million was primarily due to reduced headcount and other cost reduction strategies during the period. Costs of the organizational restructuring of $2.0 million were recognized during the nine months ended September 30, 2024, but will be paid over an extended period.

Other Income (Expense), net

Other income (expense), net for the nine months ended September 30, 2024 was $34.0 million compared to $4.1 million for the same period in 2023. The increase of $29.9 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 increase resulted from unrealized gain (loss) on long-term equity investments and interest earned and accretion of discount on marketable securities, offset by an unrealized loss due to a fraudulent funds transfer.

Liquidity and Capital Resources

Overview

As of September 30, 2024, we had total cash, cash equivalents and marketable securities of $62.7 million. We believe that our cash, cash equivalents and marketable securities as of September 30, 2024 will fund our projected operating expenses and capital expenditure requirements for at least 12 months from the issuance of 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 $14.0 million and $11.3 million for the three months ended September 30, 2024 and 2023, respectively. As of September 30, 2024, we had an accumulated deficit of $295.0 million and working capital of $54.2 million.

In June 2024, we announced an organizational restructuring which included a reduction of our workforce to prioritize our programs and extend our cash runway.

Future Funding Requirements

We believe that our available cash, and cash equivalents and marketable securities are sufficient to fund existing and planned cash requirements for at least the next 12 months. 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, 2024, 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 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 January 10, 2023 and will continue for 10 years following the rent commencement date. We issued a letter of credit in the amount of $1.9 million in association with the execution of the lease agreement, which is reflected as restricted cash on our condensed consolidated balance sheets. Payment obligations under the lease agreement include $2.3 million in the 12 months subsequent to September 30, 2024 and $19.9 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, increased inflation rates may result in greater operating costs (including labor costs) and may affect our operating budgets. In response to concerns about inflation, the U.S. Federal Reserve has raised, and may further raise, interest rates. Increases in interest rates, or a delay in interest rate reductions, 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. See “Risk Factors” for additional risks associated with our capital requirements.

While we expect that the organizational restructuring in June 2024 and a majority of the related cash payments will be substantially completed by the second quarter of 2025, we may incur other charges or cash expenditures not currently contemplated due to unanticipated events that may occur, including in connection with the implementation of the organizational restructuring. Additionally, we may not achieve the expected benefits of these cost reduction measures and other cost reduction plans on the anticipated timeline, or at all, which could otherwise accelerate our liquidity needs and could force us to further curtail or suspend our operations.

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. As of September 30, 2024, we had $250.0 million available under our S-3 Registration Statement, including $75.0 million available 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, 2024	Nine Months Ended September 30, 2023
Net cash (used in) provided by:		
Operating activities	$ (45,948)	$ (33,870)
Investing activities	33,302	45,935
Financing activities	622	502
Net (decrease) increase in cash, cash equivalents, and restricted cash	$ (12,024)	$ 12,568

Net Cash Used In Operating Activities

Net cash used in operating activities was $45.9 million for the nine months ended September 30, 2024, which consisted of net loss of $17.2 million, primarily due to a non-cash fair value adjustment recorded to the royalty monetization liability under the Ligand Agreement resulting in a $29.0 million gain, partially offset by $5.0 million of noncash stock-based compensation expense, . Net cash used in operating activities was $33.9 million for the nine months ended September 30, 2023, which consisted of net loss of $37.0 million offset by non-cash charges and changes in operating assets and liabilities, primarily related to $5.5 million of stock-based compensation expense.

Net Cash Provided By Investing Activities

Net cash provided by investing activities was $33.3 million and $45.9 million for the nine months ended September 30, 2024 and 2023, respectively, which was primarily due to 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, 2024 and 2023 resulted from proceeds from the exercise of stock options and purchases under the employee stock purchase plan.

Smaller Reporting Company Status

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.

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 months ended September 30, 2024, there were no material changes to our critical accounting policies as reported for the year ended December 31, 2023 as part of our Annual Report on Form 10-K, which was filed with the SEC on March 8, 2024. 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, 2024.

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, 2024, we had cash, cash equivalents and marketable securities totaling $62.7 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, 2024, 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, 2024, our disclosure controls and procedures were not effective.

In light of the material weaknesses described below, management performed additional analysis and other procedures to ensure that our consolidated financial statements were prepared in accordance with U.S. Generally Accepted Accounting Principles (“GAAP”). Accordingly, 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.

Identification of Material Weaknesses

Based on the evaluation of the effectiveness of our disclosure controls and procedures, our principal executive officer and principal financial officer concluded that the Company did not have sufficiently trained resources in emerging and evolving threats related to spear phishing, vendor email compromise, and other cyber attack approaches and designing and implementing controls over changes to vendor payment information. As a result, we were unable to maintain effective risk assessment and information and communication processes, and did not have effective process-level control activities over changes to vendor payment information.

The control deficiencies described above created a reasonable possibility that a material misstatement to the consolidated financial statements would not be prevented or detected on a timely basis. Therefore, we concluded that the deficiencies represent material weaknesses in the Company’s internal control over financial reporting.

During the quarter ended September 30, 2024, the control deficiencies described above resulted in the Company not identifying and preventing a criminal scheme involving a business email compromise at one of its development partners, which led to a fraudulent transfer of $1.8 million to a third-party impersonating one of its development partners. We self-discovered this fraudulent activity and promptly initiated contact with our bank as well as appropriate law

enforcement authorities in an effort to, among other things, recover the transferred funds. To date, we have not found any evidence of additional fraudulent activity.

Remediation Plan

Immediately following the incident, we initiated a reassessment of our processes and developed an action plan to remediate the identified weaknesses. This included replacing personnel on the accounting team with a newly added resource. Additionally, we are executing the following steps until remediation of the material weaknesses is achieved:

- performing additional investigative and confirming procedures;
- enhancing the design of vendor information change controls;
- implementing new controls over the approval requirements and review for ACH and wire transfers;
- increasing internal communication and training to heighten awareness and emphasize the importance of exercising professional skepticism and judgment as it relates to spear phishing, vendor email compromise and other cyber attack approaches; and
- reporting regularly to the Audit Committee of the Board of Directors of the Company on the progress and results of the remediation plan, including the identification, status and resolution of internal control deficiencies.

Changes in Internal Control over Financial Reporting

Except as described above, there have been no other changes in our internal control over financial reporting during our most recent quarter ended September 30, 2024 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 Selected Risks Associated with Our Business

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

- Historically, we have incurred significant operating losses and expect to incur substantial operating losses in the 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.
- 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 may force us to delay, limit or terminate certain of our drug development efforts or other operations.
- We are early in our development efforts of our current drug candidates and most 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, are not able to obtain the required regulatory approvals, we, or our licensees, 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.
- Under the RLT Agreement, we are entitled to receive royalty and milestone payments in connection with the development and commercialization of soticlestat. If Takeda fails to progress, delays, or discontinues the development of soticlestat, we may not receive some or all of such payments, which would materially harm our business.
- 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 identified material weaknesses in our internal control over financial reporting. If we are unable to remedy the material weaknesses, or 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.

Risks Related to Our Financial Position and Need for Additional Capital

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, 2024 was $14.0 million and we had an accumulated deficit of $295.0 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.

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 may 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. We expect our expenses to increase as we advance our current and future drug candidates through preclinical studies and clinical trials, commercialize our drug candidates, and pursue the acquisition or in-licensing of any additional drug candidates. Our expenses could increase beyond expectations if the FDA or other regulatory authorities require us to perform preclinical studies or clinical trials in addition to those that we currently anticipate. In addition, even if we obtain marketing approval for our drug candidates, they may not achieve commercial success. Our revenue, if any, will be derived from sales of drugs that we do not expect to be commercially available for a number of years, if at all. If we obtain marketing approval for any drug candidates that we develop or otherwise acquire, we expect to incur significant expenses related to manufacturing, marketing, sales and distribution.

We will require more capital in order to advance the preclinical and clinical development, obtain regulatory approval, and, following regulatory approval, commercialize our current or future drug candidates. Any additional capital raising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our current and future drug candidates.

As of September 30, 2024, our cash, cash equivalents and marketable securities were $62.7 million, and we had an accumulated deficit of $295.0 million. We believe that our existing cash, cash equivalents and marketable securities will fund our current operating plans through at least 12 months from the filing of this 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. For example, 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. If, for any reason, Takeda fails to progress, or elects to terminate the development of soticlestat as contemplated by the RLT Agreement, or if the development or commercialization of soticlestat is delayed or deprioritized by Takeda, we may not receive some or all of the royalty and milestone payments under the RLT Agreement.

While the long-term economic impacts associated with public health crises and geopolitical tensions, like the ongoing war between Russia and Ukraine and the war involving Israel, are 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. 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. High interest rates, especially if coupled with reduced 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 financial condition and our ability to pursue our business strategy.

If we are unable to raise additional capital when needed, we may 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 may 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. In June 2024 we announced a reduction of our workforce to prioritize our programs and extend our cash runway. We may not achieve the expected benefits of these cost reduction measures and other cost reduction plans on the anticipated timeline, or at all, which could otherwise accelerate our liquidity needs and could force us to further curtail or suspend our operations.

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, 2024, we recorded no U.S. federal or state income tax provision, based on a pre-tax loss of $14.0 million. As of September 30, 2024, we had available $171.4 million of unused NOL carryforwards for U.S. federal income tax purposes, $12.6 million of unused NOL carryforwards for Massachusetts income tax purposes, $164.1 million of unused NOL carryforwards for New York income tax purposes, and $163.9 million of unused NOL carryforwards for New York City 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.

Changes in tax laws or regulations that are applied adversely to us or our customers may have a material adverse effect on our business, cash flow, financial condition, or results of operations.

New tax laws, statutes, rules, regulations, or ordinances could be enacted at any time. For instance, the recently enacted Inflation Reduction Act of 2022 (the "IRA") imposes, among other rules, a 15% minimum tax on the book income of certain large corporations and a 1% excise tax on certain corporate stock repurchases. Further, existing tax laws, statutes, rules, regulations, or ordinances could be interpreted differently, changed, repealed, or modified at any time. Any such enactment, interpretation, change, repeal, or modification could adversely affect us, possibly with retroactive effect. In particular, changes in corporate tax rates, the realization of our net deferred tax assets, the taxation of foreign earnings, and the deductibility of expenses under the Tax Act, as amended by the CARES Act or any future tax reform legislation, could have a material impact on the value of our deferred tax assets, result in significant one-time charges, and increase our future tax expenses.

Risks Related to the Development and Commercialization of Our Drug Candidates

We are very early in our 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 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 current good manufacturing practices ("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 countries.

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, are not able to obtain the required regulatory approvals,

we, or our licensees, 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 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 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. 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.

For example, in June 2024, following encouraging Phase 2 findings, 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. If, for any reason Takeda fails to progress, or elects to terminate the development of soticlestat as contemplated by the RLT Agreement, or if the development or commercialization of soticlestat is delayed or deprioritized by Takeda, we may not receive some or all of the royalty and milestone payments under the RLT Agreement and may need to seek additional funds sooner than we had planned.

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 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. 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 the ongoing war between Russia and Ukraine and war involving Israel, any other war or the perception that hostilities may be imminent, including, terrorism, natural disasters or public health crises.

Further, clinical endpoints for certain diseases we are targeting, such as cerebral cavernous malformations, 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 epilepsies, seizure-related disorders, and rare neurological disorders. However, our business development activities and research activities may present attractive opportunities outside of certain epilepsies and seizure-related disorders 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 leading 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.

Identifying and qualifying patients to participate in our clinical trials is critical to our success. The number of patients suffering from some of the seizure-related disorders and rare neurological disorders we are pursuing 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 seizure-related disorders and 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 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, illnesses, injuries, discomforts and 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. 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. For example, adverse events were reported in certain clinical trials for OV101, our former drug candidate, and soticlestat. Clinical trials may not demonstrate any ocular safety benefits for OV329 relative to vigabatrin. 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 certain epilepsies, seizure-related disorders and rare neurological disorders. Given the small number of patients who have the disorders that we are targeting, 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 the 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 the ongoing war between Russia and Ukraine and the war involving Israel, 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

Under the RLT Agreement, we are entitled to receive royalty and milestone payments in connection with the development and commercialization of soticlestat. If Takeda fails to progress or discontinues the development of soticlestat, we may not receive some or all of such payments, which would materially harm our business.

In March 2021, we entered into the RLT Agreement, pursuant to which Takeda secured rights to our 50% global share in soticlestat, which we had originally licensed from Takeda, and we granted to Takeda an exclusive worldwide license under our 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. All rights in soticlestat are now owned by Takeda or exclusively licensed to Takeda by us. Following the closing date of the RLT Agreement, Takeda assumed all responsibility for, and costs of, both development and commercialization of soticlestat, and we will no longer have any financial obligation to Takeda under the original collaboration agreement, including for milestone payments or any future development and commercialization costs. Upon closing of the RLT Agreement, we received a one-time, upfront payment of $196.0 million and, if soticlestat is successfully developed, we will be eligible to receive up to an additional $660.0 million upon Takeda achieving specified regulatory and sales milestones. In addition, if soticlestat achieves regulatory approval, we will be entitled to receive tiered royalties at percentages ranging from the low double-digits, up to 20% on sales of soticlestat.

Under the terms of the RLT Agreement, Takeda has 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. Takeda has publicly announced plans to engage with regulatory authorities to discuss the totality of the data generated by the study in Dravet syndrome to determine next steps. If for any reason Takeda fails to progress, or elects to terminate the development of soticlestat as contemplated by the RLT Agreement, or if the development or commercialization of soticlestat is delayed or deprioritized by Takeda, we may not receive some or all of the royalty and milestone payments under the RLT Agreement. We are dependent upon Takeda's progression of such development. If we do not receive any payments pursuant to the RLT Agreement and are unable to find alternative sources of financing, our business and results of operations would be negatively impacted, including our ability to continue developing our current and future drug candidates.

Risks associated with the in-licensing or acquisition of drug candidates could cause substantial delays in the preclinical and clinical development of our drug candidates.

We have previously acquired and we may acquire or in-license drug candidates for preclinical or clinical development in the future as we continue to build our pipeline. Such arrangements with third parties may impose diligence, development and commercialization obligations, milestone payments, royalty payments, indemnification and other obligations on us. Our obligations to pay milestone, royalty and other payments to our licensors may be substantial, and the amount and timing of such payments may impact our ability to progress the development and commercialization of our drug candidates. Our rights to use any licensed intellectual property may be subject to the continuation of and our compliance with the terms of any such agreements. Additionally, disputes may arise regarding our rights to intellectual property licensed to us or acquired by us from a third party, including but not limited to:

- the scope of intellectual property rights included in, and rights granted under, any license or other agreement;
- the sublicensing of patent and other rights under such agreements;
- our compliance with our diligence obligations under any license agreement;
- the ownership of inventions and know-how resulting from the creation or use of intellectual property by us, alone or with our licensors and collaborators;
- the scope and duration of our payment obligations, and our ability to make such payments when they are owed;
- our need to acquire additional intellectual property rights from third parties that may impact payments due under such agreements;
- the rights of our licensors to terminate any such agreement;
- our rights and obligations upon termination of such agreement; and
- the scope and duration of exclusivity obligations of each party to the agreement.

Disputes over intellectual property and other rights that we have licensed or acquired, or may license or acquire in the future, from third parties could prevent or impair our ability to maintain any such arrangements on acceptable terms,

result in delays in the commencement or completion of our preclinical studies and clinical trials and impact our ability to successfully develop and commercialize the affected drug candidates. If we fail to comply with our obligations under any future licensing agreements, these agreements may be terminated or the scope of our rights under them may be reduced and we might be unable to develop, manufacture or market any product that is licensed under these agreements.

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. Further, our business development activities and research activities may present attractive opportunities outside of certain epilepsies and seizure-related disorders and we may choose to pursue drug candidates in other areas of interest including other disorders and diseases 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.

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 by the Health Care and Education Reconciliation Act (collectively, the "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;
- the federal Health Insurance Portability and Accountability Act of 1996 ("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 the Health Information Technology for Economic and Clinical Health Act ("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; Other Modifications to HIPAA, 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 ("CMS"), 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 executive, judicial, Congressional and executive branch challenges to certain aspects of the PPACA. For example, on June 17, 2021 the U.S. Supreme Court dismissed a challenge on procedural grounds that argued the PPACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress. Further, there have been a number of health reform measures by the Biden administration that have impacted the PPACA. On August 16, 2022, President Biden signed the IRA into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in 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 Biden administration will impact the PPACA and our business.

Other legislative changes have been proposed and adopted since the PPACA was enacted. These 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. The American Taxpayer Relief Act of 2012, among other things, further reduced Medicare payments to several providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

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, in July 2021, the Biden administration released an executive order, "Promoting Competition in the American Economy," with multiple provisions aimed at prescription drugs. In response to Biden's executive order, on September 9, 2021, the U.S. Department of Health and Human Services ("HHS,") released a Comprehensive Plan for Addressing High Drug Prices that outlines principles for drug pricing reform and sets out a variety of potential legislative policies that Congress could pursue as well as potential administrative actions HHS can take to advance these principles. Further, the IRA, among other things, (i) directs HHS to negotiate the price of certain high-expenditure, single-source drugs and biologics covered under Medicare, and (ii) imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation. These provisions take effect progressively starting in fiscal year 2023. In August 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. HHS will select up to fifteen additional drugs covered under Part D for negotiation in 2025. Additionally, the Biden administration released an additional executive order on October 14, 2022, directing HHS to report on how the Center for Medicare and Medicaid Innovation can be further leveraged to test new models for lowering drug costs for Medicare and Medicaid beneficiaries. In response to the Biden administration's October 2022 executive order, on February 14, 2023, HHS released a report outlining three new models for testing by the Centers for Medicare & Medicaid Services Innovation Center which will be evaluated on their ability to lower the cost of drugs, promote accessibility, and improve quality of care. It is unclear whether the models will be utilized in any health reform measures in the future. Further, on December 7, 2023, the Biden administration announced an initiative to control the price of prescription drugs through the use of march-in rights under the Bayh-Dole Act. 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.

We expect that these and other healthcare reform measures that may be adopted in the future, particularly in light of the upcoming U.S. Presidential and Congressional elections, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved drug. 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.

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.

Recent 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. On December 16, 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 on March 16, 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 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 do not currently have the ability to independently conduct any preclinical studies or clinical trials. We intend to 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 intend to rely upon CROs to monitor and manage data for our clinical programs, as well as the execution of future preclinical studies. We expect 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 good laboratory practices ("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 will have agreements governing their activities, our CROs will not be our employees, and we will 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.

As of September 30, 2024, we had 25 full-time employees. On June 28, 2024, we announced a reduction of our workforce to prioritize our programs and extend our cash runway. As our development and commercialization plans and strategies for our current pipeline of product candidates develop, we expect to need additional managerial, operational, sales, marketing, financial, legal and other resources. 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. 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.

The organizational restructuring and consequent change in our operations may cause additional attrition and affect employee morale. Additionally, as we are operating our business with fewer employees, we face additional risk that we might not be able to execute on our strategic plans, which may have an adverse effect on our business, financial condition and operating results.

Further, our organizational restructuring announced in June 2024 may make retention of our current personnel both more important and more challenging. This organizational restructuring resulted in the loss of certain longer-term employees, the loss of institutional knowledge and expertise and the reallocation and combination of certain roles and responsibilities across the organization, all of which could adversely affect our operations.

Actions that we have taken to restructure our business to prioritize our programs and extend our cash runway may not have the anticipated effects.

In June 2024, we announced an organizational restructuring plan to reduce our workforce by 17 people, or approximately 43% of our then-existing headcount. In connection with this organizational restructuring, our General Counsel and Chief Operating Officer ceased serving in those roles. The decision to reduce our workforce was made in order to prioritize our programs and extend our cash runway. As a result of the organizational restructuring, we have incurred and expect to continue to incur cash expenditures related to employee severance, benefits and related costs. We may incur additional expenses not currently contemplated due to events associated with the organizational restructuring; for example, the organizational restructuring may have a future impact on other areas of our liabilities and obligations, which could result in losses in future periods. Moreover, we may not realize, in full or in part, the anticipated benefits and savings from this reorganization due to unforeseen difficulties, delays or unexpected costs. If we are unable to realize the expected operational efficiencies and cost savings from the restructuring, our operating results and financial condition would be adversely affected. In addition, we may need to undertake additional organization restructurings, workforce reductions or other similar activities in the future.

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, 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 company confidential 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.

Significant disruptions of our information technology systems or data security incidents could result in significant financial, legal, regulatory, business and reputational harm to us.

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 upon which we rely face a variety of evolving threats that could cause security incidents. We have also outsourced elements of our operations (including elements of our information technology infrastructure) to third parties, and as a result, we manage a number of third-party vendors who may or could have access to our computer networks or our sensitive data. 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. Furthermore, our ability to monitor the aforementioned third parties' information security practices is limited, and these third parties may not have adequate information security measures in place. If our third-party service providers experience a security incident or other interruption, we could experience adverse consequences. While we may be entitled to damages if our third-party service providers fail to satisfy their privacy or security-related obligations to us, any award may be insufficient to cover our damages, or we may be unable to recover such award. 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 the ongoing war between Russia and Ukraine and the war involving Israel, among others, are likely to increase the frequency of cybersecurity incidents.

In addition, due to our hybrid-work environment, we may be more vulnerable to cyberattacks as more of our employees utilize network connections, computers, and devices outside our premises or network, including working at home, while in transit and in public locations. 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 found during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

Potential vulnerabilities can be exploited from inadvertent or intentional actions of our employees, third-party vendors, business partners, or by malicious third parties. 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 experience delays in deploying remedial measures and patches designed to address identified vulnerabilities. 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.

We may expend significant resources or modify our business activities to try to protect against security incidents. Additionally, certain data privacy and security obligations may require us to implement and maintain specific security

measures or industry-standard or reasonable security measures to protect our information technology systems and sensitive 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 upon whom we rely) 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.

We 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 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, we may be subject to 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, creating complex compliance issues for us. 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 other countries have, or are developing, laws governing the collection, use and transmission of personal information as well. EU member states and other jurisdictions have adopted data protection laws and regulations, which impose significant compliance obligations. For example, in May 2016, the EU formally adopted the EU's General Data Protection Regulation ("GDPR"), which applies to all EU member states as of May 25, 2018 and replaces the former EU Data Protection Directive. The regulation introduced new data protection requirements in the EU and imposes substantial fines for breaches of the data protection rules. The GDPR must be implemented into national laws by the EU member states and imposes strict obligations and restrictions on the ability to collect, analyze, and transfer personal data, including health data from clinical trials and adverse event reporting. Data protection authorities from different EU member states have interpreted the privacy laws differently, which adds to the complexity of processing personal data in the EU, and guidance on implementation and compliance practices are often updated or otherwise revised. Any failure to comply with the rules arising from the GDPR and related national laws of EU member states could lead to government enforcement actions and fines of up to 20 million Euros or 4% of annual global revenue, whichever is greater, and adversely impact our

operating results. The GDPR will increase our responsibility and liability in relation to personal data that we process and we may be required to put in place additional mechanisms ensuring compliance with EU data protection rules.

Additionally, California enacted the California Consumer Privacy Act of 2018, as amended by the California Privacy Rights Act of 2020 (the "CCPA") which has been dubbed the first "GDPR-like" law in the United States. In the past few years, numerous other U.S. states—including Virginia, Colorado, Connecticut, and Utah—have also 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. The CCPA gives California residents expanded rights to access, correct and delete their personal information, opt out of certain personal information sharing and receive detailed information about how their personal information is used by requiring covered companies to provide new disclosures to California consumers (as that term is broadly defined) and provide such consumers new ways to opt-out of certain sales of personal information. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. 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 in the future.

In addition to data privacy and security laws, we may be bound by other 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 and if these policies, materials, or statements are found to be deficient, lacking in transparency, deceptive, unfair, or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators, or other adverse consequences.

We may at times fail (or be perceived to have failed) in our efforts to comply with our data privacy and security obligations. Moreover, despite our efforts, our personnel or third parties on whom we rely may fail to comply with such obligations, which could negatively impact our business operations. If we or the third parties on which we rely 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.

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 (the "Exchange Act"). 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 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 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. Until we cease to be a smaller reporting company, the scaled-back disclosure in our SEC filings will result in less information about our Company being available than for other public companies. 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 may take advantage of certain of the scaled-back disclosures available to smaller reporting companies, including but not limited to:

- reduced disclosure obligations regarding 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.

We have identified material weaknesses in our internal control over financial reporting. If we are unable to remedy the material weaknesses, or 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.

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 partners, which led to a fraudulent transfer of $1.8 million to a third-party impersonating one of our development partners. The matter has been reported to the U.S. Secret Service and Federal Bureau of Investigation. Based on the information we have received from our banking institution, we believe the receiving account has been frozen. We have also received information suggesting that the account to which the funds were remitted may have been blocked under U.S. sanctions administered by the U.S. Office of Foreign Asset Control ("OFAC"), and we are seeking to confirm, in which case, we will consider submitting an application for release of the blocked funds to OFAC. We do not know whether we will be able to recover such funds.

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 as of September 30, 2024. 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 to remediate this matter, as described further in Part I, Item 4 of this Quarterly Report on Form 10-Q. We continue to evaluate steps to remediate the material weaknesses. These remediation measures may be time consuming and costly and there is no assurance that these initiatives will ultimately have the intended effects.

Our compliance with Section 404 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. During the evaluation and testing process, if we are unable to remedy the material weaknesses or if we identify any new one or more material weaknesses in our internal control over financial reporting, we will be unable to assert that our internal control over financial reporting is effective. We cannot assure you that there will not be any new 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 remedy the material weaknesses, continue to be or in the future 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 new material weaknesses 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 the material weaknesses or any new material weakness 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

The market price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for 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, 2024, the closing price of our common stock on The Nasdaq Global Select Market ranged from $0.70 per share to $4.04 per share. During this time period, 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, results of Takeda's clinical trials of soticlestat, 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; 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.

Unstable market and economic conditions may have serious adverse consequences on our business, financial condition and share price.

The global economy, including credit and financial markets, has experienced extreme volatility and disruptions, including severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates, increases in inflation rates and uncertainty about economic stability. The financial markets and the global economy may also be adversely affected by the current or anticipated impact of presidential and other elections in the United States, military conflicts, terrorism, or other acts of violence or geopolitical events. Global geopolitical tensions have created extreme 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. Any such volatility and disruptions may have adverse consequences on us 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.

There is no public market for our Series A convertible preferred stock .

There is no established public trading market for our Series A convertible 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 convertible preferred stock on any national securities exchange or other nationally recognized trading system. Without an active market, the liquidity of the Series A convertible preferred stock will be limited.

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 your rights as a stockholder. In addition, we may issue equity or debt securities as consideration for obtaining rights to additional compounds.

In November 2023, we filed a shelf registration statement on Form S-3 (Registration No. 333-275307) (the "S-3 Registration Statement") to replace our prior registration statement that was set to expire. The S-3 Registration Statement 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. As of September 30, 2024, we had $250.0 million available under our S-3 Registration Statement, including $75.0 million available pursuant to our ATM program. 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.

Stockholders will be diluted by any conversions of outstanding Series A convertible preferred stock and exercises of outstanding options.

As of September 30, 2024, we had outstanding options to purchase an aggregate of 17,036,145 shares of our common stock at a weighted average exercise price of $3.51 per share and 1,250,000 shares of common stock issuable upon conversion of outstanding Series A convertible preferred stock for no additional consideration. Such Series A convertible 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 convertible 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, 2024, our executive officers, directors and stockholders who owned more than 5% of our outstanding common stock, in the aggregate, beneficially own shares representing approximately 57.3% 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 corporate charter 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 corporate charter and our 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 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 charter 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 charter 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 amended and restated certificate of incorporation and amended and restated 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 amended and restated certificate of incorporation or amended and restated 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.

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.

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, 2024, 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).
10.1*	Consulting Services Agreement, dated July 30, 2024 , between Ovid Therapeutics Inc. and Thomas Perone.
10.2	Amended and Restated Executive Employment Agreement , dated September 9, 2024, between the Company and Margaret Alex ander (incorporated herein by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 001-38085), filed with the Commission on September 11, 2024).
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.

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, 2024

By: /s/ Jeremy M. Levin

Jeremy M. Levin

Chief Executive Officer

(Principal Executive Officer)

Date: November 12, 2024

By: /s/ Jeffrey Rona

Jeffrey Rona

Chief Business and Financial Officer

(Principal Financial and Accounting Officer)

CONSULTING SERVICES AGREEMENT

This Consulting Services Agreement (the "***Agreement***") is made by and between Ovid Therapeutics Inc. ("***Company***") and Thomas Perone ("***Consultant***"), dated July [29], 2024. Company and Consultant may each be referred to in this Agreement individually as a "***Party***" and collectively as the "***Parties***."

WHEREAS, Company wishes to engage Consultant to perform certain services, as further described in the Statement of Work attached hereto as Exhibit A, as the same may be amended from time to time (the "***Services***"); and

WHEREAS, Consultant has expertise in conducting the Services and wishes to accept such engagement to perform the Services, all in accordance with the terms and conditions set forth in this Agreement.

NOW THEREFORE, in consideration of the mutual promises contained herein and other good and valuable consideration, the Parties agree as follows:

1. SERVICES

1.1 **Scope**. Company hereby engages Consultant to provide, and Consultant agrees to undertake and complete the Services set forth in Exhibit A, in accordance with any timeline and on any schedule specified therein. Consultant agrees to provide all Services personally in accordance with this Agreement and may not subcontract or otherwise delegate Consultant's obligations under this Agreement to any other entity, unless otherwise agreed upon in advance by Company in writing. Consultant will use his professional expertise and independent judgment in providing the Services promptly, diligently, and competently, and in accordance with the terms of this Agreement and Company's then-current procedures and standards promulgated from time to time by Company with regard to its business and Consultant's access to and use of Company's property, information, equipment, and facilities. Entering into this Agreement in no way obligates Company to retain Consultant to perform additional services.

1.2 **Change in Scope of Work**. Any change in the details of a Statement of Work or the assumptions upon which the work is based (including, but not limited to, changes in the agreed start or completion dates) shall require an amendment to the Statement of Work ("***Amendment***") to be agreed upon in writing and executed by the Parties. Each Amendment shall detail the changes, responsibility for the changes, and any adjustments to the budget and timeline. The Amendment will become effective upon its execution by both Parties. The Parties agree to act in good faith and respond promptly when considering an Amendment requested by the other Party.

1.3 **Reports and Assistance**. Consultant will provide Company with written reports and/or oral reports, as Company may reasonably request, on the status of its Services under this Agreement. Consultant will have the right to establish Consultant's own hours of work, provided, however, that Consultant will be available from time to time to attend all meetings as reasonably requested by Company, including meetings with other consultants that Company may engage to provide services similar to the Services, and shall cooperate with Company's representatives and third party vendors as reasonably requested by Company. Consultant further agrees to provide

Company with all cooperation and assistance reasonably requested in connection with the Services.

1.4 **Records and Audit Rights**. Consultant shall maintain complete and accurate records in the course of providing Services under this Agreement, including all computerized records and files, in a secure area protected from fire and other natural hazards, theft and destruction. Consultant shall reasonably cooperate in any audit conducted hereunder and shall provide reasonable access to any and all of Consultant's books, records, agreements, and other documents necessary to assess Consultant's compliance with this Agreement.

2. RELATIONSHIP OF PARTIES

2.1 **Independent Contractor**. Consultant's relationship with Company is that of an independent contractor, and nothing in this Agreement is intended to, or should be construed to, create a partnership, agency, joint venture or employment relationship between Company and any of Consultant's employees or agents. Company does not authorize Consultant to make any representation, contract or commitment on behalf of Company. Consultant will not be entitled to any of the benefits, coverages, or privileges, that Company may make available to its employees, including, but not limited to, holiday/vacation pay, group health or life insurance, profit-sharing or retirement benefits. Because Consultant is an independent contractor, Company will not withhold or make payments for social security, make unemployment insurance or disability insurance contributions, or obtain workers' compensation insurance on behalf of Consultant. Consultant is solely responsible for, and will file, on a timely basis, all tax returns and payments required to be filed with, or made to, any federal, state or local tax authority with respect to the performance of Services and receipt of fees under this Agreement. Consultant is solely responsible for, and must maintain adequate records of, expenses incurred in the course of performing Services under this Agreement No part of Consultant's compensation will be subject to withholding by Company for the payment of any social security, federal, state or any other employee payroll taxes. Company will regularly report amounts paid to Consultant by filing Form 1099-NEC with the Internal Revenue Service as required by law. If, notwithstanding the foregoing, Consultant is reclassified as an employee of Company, or any affiliate of Company, by the U.S. Internal Revenue Service, the U.S. Department of Labor, or any other federal or state or foreign agency as the result of any administrative or judicial proceeding, Consultant agrees that Consultant will not, as the result of such reclassification, be entitled to or eligible for, on either a prospective or retrospective basis, any employee benefits under any plans or programs established or maintained by Company.

2.2 **Outside Activities**. Consultant may engage in outside business activities in addition to the Services as long as such activities do not compete with the business activities of the Company, create a conflict of interest, or unreasonably interfere with the Consultant's obligations to Company hereunder, each as determined by the Company in its reasonable and good faith discretion.

2.3 **Non-Solicitation**. Consultant agrees that during the Term and for one (1) year thereafter (the "***Restricted Period***"), Consultant will not, either directly or through others, solicit, recruit, or attempt to solicit or recruit, or otherwise induce or encourage any employee, independent contractor, or consultant of Company or any of its affiliates to terminate his or her relationship with Company or such affiliated in anticipation of an offer to become an employee, an offer to provide consulting services or an offer to perform as an independent contractor, in each case, for such other person or entity.

3. PAYMENT

3.1 **Compensation and Invoicing**.

(a) As sole compensation for the timely performance of the Services, Company will compensate Consultant as (and only as) expressly stated in Exhibit A. Nothing contained in this Agreement will be construed in any manner as an obligation or inducement for Consultant to purchase, use, order, or recommend any products manufactured or distributed by or services provided by Company, nor as a reward for any such purchase, order, prescription, or recommendation. The payments provided for in this Section 3.1, as set forth in the Statement of Work, will constitute Consultant's sole compensation for all Services rendered to Company hereunder. No additional payments shall be due hereunder unless specifically agreed to in writing by Company and Consultant.

(b) Except as provided for in Exhibit A, Company will compensate Consultant to the extent that (i) the Services are performed in accordance with this Agreement, and (ii) Consultant provides Company with fully documented invoice(s) certifying the time incurred in providing the Services hereunder. Consultant shall submit monthly invoices in the form attached hereto as Exhibit B to Company for services rendered under this Agreement. All invoices must be received within ten (10) days of the close of the month for which the Services were rendered. Absent a good faith dispute, payment shall be due within thirty (30) days of receipt of the invoice; provided, that Company may contest any invoice or portion thereof, to the extent that it reasonably believes that the charges reflected therein are inappropriate or lack a clear basis (paying all charges that are appropriate). Once any such issue or concern is resolved, Company shall pay any remaining appropriate charges within thirty (30) days of the date that such resolution occurs.

3.2 **Expenses**. The Consultant agrees that it will not incur any expenses hereunder without the prior written approval of Company. Company will only reimburse pre-approved expenses that are (a) permitted by Company's then-existing travel and expense policy, and (b) reasonably, necessarily and actually incurred in connection with the performance of the Services hereunder (including, for example, pre-approved out-of-pocket expenses relating to air travel, hotel, and meal expenses). Notwithstanding the foregoing, Company will not reimburse any expenses if such expenses are subject to reimbursement by a third party. Consultant shall submit with the applicable invoice written documentation itemizing all expenses, including copies of receipts substantiating the claimed expenses. Consultant shall submit all invoices for expenses in accordance with the procedures described in Section 3.1(b).

3.3 **Disclosure**. Company has the right in its sole discretion to disclose, as required under any applicable law, regulation or otherwise, information about the nature of the Services

Consultant performs under this Agreement and any compensation, expenses or other transfers of value made to the Consultant relating to this Agreement. In its sole discretion, Company may disclose and display such information to anyone and in any medium, including to other entities for which Consultant may be performing services, and/or to Consultant's affiliated institutions.

4. INTELLECTUAL PROPERTY

4.1 **Pre-existing Intellectual Property**. Ownership of inventions, technologies, processes, techniques, algorithms, programs, discoveries, improvements, drugs, pharmaceuticals, biologics, products, concepts, designs, prototypes, samples, models, technical information, materials, drawings, specifications and other works of authorship owned or controlled by either Party and existing as of the Effective Date (as defined herein), and all patents, copyrights, trade secret rights and other intellectual property rights therein (collectively, "***Pre-existing Intellectual Property***"), is not affected by this Agreement, and neither Party shall have any claims to or rights in any Pre-existing Intellectual Property of the other Party, except as set forth in Section 4.7 hereunder.

4.2 **Inventions**. Without limiting the foregoing, any interest in patents, patent applications, inventions, technological innovations, copyrights, copyrightable works, developments, discoveries, designs, processes, formulas, know-how, data and analysis, whether patentable or not which Consultant may create, conceive, develop or reduce to practice or author in the performance of the Services or at Company's expense ("***Inventions***"), shall be the exclusive property of Company.

4.3 **Data and Work Product**. All data, information, reports, results, records, documentation, databases, designs, logos, packaging, formulations, writings, and other work product created, conceived, developed or reduced to practice by Consultant in connection with the Services performed hereunder or at Company's expense (collectively, "***Data***"), shall be the exclusive property of Company. Consultant acknowledges that Company holds the copyright to all such materials provided to Consultant, except where Consultant expressly attributes such materials to third parties, such attribution not to be construed as limiting Consultant's obligations under Section 4.7.

4.4 **Assignment of Ownership**. Consultant shall promptly disclose in writing to Company all Data and Inventions promptly as such Data and Inventions arise, and in consideration for the compensation provided to Consultant under this Agreement, Consultant agrees to assign and hereby irrevocably transfers and assigns to Company in the United States and throughout the world any and all of its right, title, and interest in and to Data and Inventions (including of its right, title, and interest to any intellectual property rights therein). Consultant agrees that all such Data and Inventions shall be treated as "works made for hire" exclusively for the Company, as applicable, and as defined in the Copyright Act of 1976, as amended, 17 U.S.C. §101, et seq.; and (b) all rights of action and claims for damages and benefits arising due to past and present infringement of said rights.

4.5 **Other Rights**. If Consultant has any rights, including without limitation "artist's rights" or "moral rights," in and Data or Invention that cannot be assigned pursuant to Section 4.4, Consultant hereby unconditionally and irrevocably grants to Company an exclusive (even as to

Consultant), worldwide, fully paid and royalty-free, irrevocable, perpetual license, with rights to sublicense through multiple tiers of sublicensees, to use, reproduce, distribute, create derivative works of, publicly perform and publicly display any such Data and Invention in any medium or format, whether now known or later developed. In the event that Consultant has any rights in the Data and Inventions that cannot be assigned or licensed, Consultant unconditionally and irrevocably waives the enforcement of such rights, and all claims and causes of action of any kind against Company or Company's customers.

4.6 **Further Assurances**. Consultant agrees to cooperate with and assist Company to apply for, and to execute any documents reasonably necessary to Company to secure, perfect, effectuate, and preserve Company's rights throughout the world in the Data and Inventions as Company deems appropriate. If, after reasonable efforts, Consultant is unable or unwilling to comply with the foregoing obligation, then Consultant hereby irrevocably appoints Company as Consultant's attorney-in-fact solely for the purpose of executing such documents on Consultant's behalf, which appointment is coupled with an interest. Consultant will deliver any deliverables (which, for clarity, may include Data or Inventions) in accordance with the applicable Statement of Work.

4.7 **Background License**. If any part of the Services or Data or Inventions provided or generated hereunder is based on, incorporates, or is an improvement or derivative of, or cannot be reasonably and fully made, used, reproduced, distributed and otherwise exploited without using the technology or violating any intellectual property rights owned by or licensed to Consultant (including without limitation any Pre-Existing Intellectual Property of Consultant or any third party intellectual property rights) and not assigned to Company hereunder, Consultant hereby grants Company and its successors a perpetual, irrevocable, worldwide royalty-free, non-exclusive, freely sublicensable right and license to exploit and exercise all such technology and intellectual property rights (including Pre-Existing Intellectual Property) in support of Company's exercise or exploitation of the Services, Inventions, Data, work product, deliverables or other work performed or information or provided or generated hereunder.

4.8 **No Implied License**. Except as expressly set forth herein, nothing contained in this Agreement, nor the disclosure or provision to Consultant of any Confidential Information (defined below), shall be deemed to transfer or grant to, Consultant, any right, title, interest, or license in, to or under any intellectual property or other proprietary right of Company.

4.9 **No Reverse Engineering.** Consultant may in providing Services under this Agreement receive a Company product or materials. Consultant shall not during the term of this Agreement or at any time after its expiration reverse engineer, disassemble, modify, manipulate, or reproduce, or aid in the reverse engineering, disassembly, modification, manipulation, or reproduction of any Company product or materials without Company's express written consent.

5. CONFIDENTIAL INFORMATION

5.1 **Confidential Information**. Consultant agrees that during the term of this Agreement and thereafter it shall keep confidential and shall not (a) publish or otherwise disclose any Confidential Information furnished to it other than as provided for in this Agreement or (b) use for any purpose any Confidential Information furnished to it except to the extent such use is

expressly permitted by the terms of this Agreement or is reasonably necessary for the performance of the Services under this Agreement. "***Confidential Information***" as used in this Agreement shall mean all information disclosed by Company to Consultant, whether during or before the term of this Agreement, that is considered confidential or proprietary by Company, as well as information that is otherwise learned by Consultant in connection with the Services or that is created or discovered by Consultant while working on behalf Company in furtherance of the Services, including without limitation any Data or Inventions. Confidential Information shall also include, without limitation: (i) concepts and ideas relating to the development and distribution of content in any medium or to the current, future and proposed products or services of Company or its subsidiaries or affiliates; (ii) trade secrets, drawings, inventions, know-how, software programs, and software source documents, compounds or clinical or non-clinical data; (iii) information regarding plans for research, development, new service offerings or products, marketing and selling, business plans, business forecasts, budgets and unpublished financial statements, licenses and distribution arrangements, prices and costs, suppliers and customers; (iv) existence of any business discussions, negotiations or agreements between the parties; and (v) any information regarding the skills and compensation of employees, contractors or other agents of Company or its subsidiaries or affiliates. Confidential Information also includes proprietary or confidential information of any third party who may disclose such information to Company or Consultant in the course of Company's business.

5.2 **Exceptions**. The obligations of Confidentiality and non-use set forth above shall not apply to with respect to any Confidential Information that Consultant can demonstrate by competent written evidence: (a) was generally available to the public at the time it was disclosed to or generated by Consultant; (b) became generally available to the public subsequent to disclosure to or generation by Consultant, other than by Consultant's breach of this Agreement; (c) was in Consultant's possession, as evidenced by its written records, free of any obligation of confidence at the time it was disclosed to Consultant, and was not obtained by Consultant either directly or indirectly from Company; or (d) was rightfully communicated to Consultant by a third party free of any obligation of confidence.

5.3 **Permitted Disclosures; Return of Confidential Information**. Consultant acknowledges the confidential and secret character of the Confidential Information and agrees that the Confidential Information is the sole, exclusive and extremely valuable property of Company. Accordingly, Consultant agrees not to reproduce, use or disclose any Confidential Information except as expressly authorized hereunder. Notwithstanding the foregoing, Consultant may disclose Confidential Information to Consultant employees on a need to know basis, for the sole purpose of performing its obligations under this Agreement, *provided* that in each case the recipient of such Confidential Information is bound by written obligations of confidentiality and non-use at least as equivalent in scope as those set forth in this Article 5 prior to any such disclosure. Upon termination of this Agreement for any reason, including expiration of the Term, Consultant agrees to cease using and to return to Company all whole and partial copies and derivatives of the Confidential Information, whether in Consultant's possession or under Consultant's direct or indirect control.

5.4 **Compelled Disclosure**. Notwithstanding the above obligations, Consultant may disclose particular Confidential Information without violating the obligations of this Agreement to the extent that such disclosure is required by a valid order of a court or other governmental body

having jurisdiction, or by applicable law or regulation; provided that, in each case, Consultant (a) provides Company with reasonable prior written notice of such disclosure obligation to the extent permitted by law, to provide the Company with the opportunity to seek confidential treatment of any such Confidential Information required to be disclosed and/or to obtain a protective order narrowing the scope of disclosure, (b) reasonably cooperates and assists Company in obtaining such a protective order or other appropriate remedy preventing or limiting the disclosure and/or requiring that the Confidential Information so disclosed be used only for the purposes for which the order was issued or for which the law or regulation requires, and (c) discloses the minimum amount of Confidential Information required to comply with such court or government order, law or regulation. For the avoidance of doubt, any Confidential Information that Consultant is required disclose shall remain otherwise subject to the confidentiality and non-use obligations set forth herein.

5.5 **Third Party Information**. Consultant recognizes that Company has received, and in the future will receive, from third parties their confidential or proprietary information subject to a duty on Company's part to maintain the confidentiality of such information and, in some cases, to use it only for certain limited purposes. Consultant agrees that he/she owes Company and such third parties, both during the term of the Agreement and thereafter, a duty to hold all such confidential or proprietary information in the strictest confidence and not to disclose it to any person, firm or corporation (except in a manner that is consistent with Company's agreement with the third party as confirmed in writing) or use it for the benefit of anyone other than Company or such third party (consistent with Company's agreement with the third party). Consultant further agrees not to disclose to Company or to use in the performance of Services for Company any materials or documents obtained by Consultant from a third party (including any present or former client of Consultant) under an obligation of confidentiality, unless (a) Consultant has obtained such third party's written consent to such disclosure, and (b) Company is first notified that such information is the confidential or proprietary information of a third party and Company nevertheless agrees to receive it.

5.6 **HIPAA**. Consultant shall comply at all times with state and Federal laws regarding the confidentiality of medical and financial records. Consultant shall not release any medical or financial records to any third person, except as required by law or court order, or as approved by Company prior to release, except as allowed by the Healthcare Insurance Portability and Accountability Act of 1996 ("***HIPAA***") regulations and rules regarding the privacy and security of Protected Health Information (as defined in such regulations and rules) and for the transmission of Protected Health Information. Company and Consultant agree, solely to the extent applicable to the terms of this Agreement, to fully comply with HIPAA and its regulations and rules regarding such Protected Health Information.

5.7 **Data Privacy.** Consultant will comply with all applicable requirements of and all its obligations under the Data Protection Legislation which arise in connection with the Agreement. "***Data Protection Legislation***" means (i) the EU General Data Protection Regulation (2016/679): (ii) the California Consumer Privacy Act of 2018; or (iii) other laws, rules and regulations relating to processing of any personal data and/or privacy that are in effect as of the Effective Date or may be enacted after the Effective Date.

6. INDEMNIFICATION

6.1 **Indemnification by Company**. Company agrees to defend, indemnify, and hold harmless Consultant from and against any and all liability, damages, loss or expense (including reasonable attorney fees and expenses of litigation) arising from any third party claim or action against Consultant to the extent resulting from: (a) the negligence or willful misconduct of Company or any of its agents or employees; or (b) Consultant's conduct of specific Services in strict accordance with the express written instructions of Company, except in each case of (a) and (b) to the extent resulting from Consultant's negligence or willful misconduct or breach of this Agreement.

7. TERM AND TERMINATION

7.1 **Term**. This Agreement shall be effective as of July 11, 2024 (the "***Effective Date***") and shall remain in full force and effect until January 31, 2025, unless terminated earlier by either Party pursuant to Section 8.2 (the "**Term**").

7.2 **Termination**. The Company and Consultant may terminate this Agreement at any time by giving the other party thirty (30) days' prior written notice.

7.3 **Effects of Termination**. In the event of termination pursuant to Section 7.2, Consultant shall cease work immediately after giving or receiving such notice or termination. In the event of any expiration or termination of this Agreement, unless otherwise advised by Company, Consultant shall (a) return to Company all Confidential Information, all work product (including Data or Inventions) and all other materials belonging to Company and shall notify Company of costs incurred up to the termination date. No expiration or termination of this Agreement will excuse the nonperformance of either Party's obligations with respect to any unfinished Services or shall relieve either Party of any obligation that has accrued prior to the effective date of termination or expiration.

7.4 **Survival**. The rights and obligations contained in Articles 2 ("***Relationship of the Parties***"), 3 ("***Payment***," solely with respect to any outstanding payment obligations and disclosure rights of Company), 4 ("***Intellectual Property***"), 5 ("***Confidential Information***"), 6 ("***Indemnification***"), 9 ("***Publicity***"), 10 ("***Representations and Warranties***"), 11 ("***Limitation of Liability***"), 12 ("***Notices***"), and Sections 1.4 (for the time periods set forth therein), 2.1, 2.2, 8.3-8.5, 13.1, 13.3-13.8 shall survive any termination or expiration of this Agreement for any reason.

7.5 **Company's Remedies**. Because the Services are personal and unique and Consultant will have access to Company's Confidential Information, Company will have the right to enforce this Agreement and any of its provisions by injunction, specific performance or other equitable relief without prejudice to any other rights and remedies that Company may have for a breach of this Agreement. Consultant hereby acknowledges that its breach of this Agreement may cause irreparable harm and significant injury to Company, which may be difficult to ascertain, and accordingly, Consultant agrees that Company shall be entitled to specific performance against any breach, threatened breach, or attempted breach of such sections, in addition to any other remedies that may be available at law or in equity.

8. PUBLICITY

Consultant shall not, in any way or in any form, (a) publicize that it is performing the Services hereunder, or (c) use the name, marks or indicia of Company for any purpose, in whole or in part, in each case, without the prior written consent of Company, which consent may be granted or withheld by Company in its sole discretion. Consultant hereby grants Company the right to use Consultant's name and the relationship with Company in connection with various reports, brochures or other documents produced by or on behalf of Company.

9. REPRESENTATIONS AND WARRANTIES

9.1 **Performance**. Consultant represents, warrants and covenants that Consultant is skilled and experienced in providing the Services, and will perform the Services in a professional and workmanlike manner customary in the industry. Consultant and Company agree that Consultant shall merely provide the Company with advice and recommendations, but it is the Company's decision whether or not to follow such advice and/or to seek further advice from others.

9.2 **No Debarment or Disqualification**. Consultant represents, warrants and covenants that he/she: (a) is not under investigation by the FDA for debarment or presently debarred by the FDA pursuant to 21 U.S.C. § 335a; (b) has not been disqualified by the FDA and does not have a disqualification hearing pending pursuant to 21 CFR § 312.70 or its successor provisions; and (c) is not engaged in any conduct or activity that could lead to any of the above-mentioned disqualification or debarment actions. If during the term of this Agreement, Consultant (i) comes under investigation by the FDA for a debarment action or disqualification, (ii) is debarred or disqualified, or (iii) engages in any conduct or activity that could lead to any of the above-mentioned disqualification or debarment actions, Consultant will immediately notify Company of same.

9.3 **No Conflicts**. Consultant represents, warrants and covenants that Consultant is not performing work for a third party or is under obligations outside of this Agreement that would prevent Consultant from competently providing Services to Company or create a conflict of interest (including where such third party is competing or is preparing to compete with any business or demonstrably anticipated business of Company). Consultant shall not enter into any agreement to provide services which would in any way materially impair its/his/her ability to complete the Services in a timely fashion or create a conflict of interest.

9.4 **No Discrimination**. Consultant represents, warrants and covenants that he/she will not act in any manner to discriminate against any employee of Company because of the employee's race, color, age, sex, national origin, ancestry, religion, sexual orientation, gender identity, disability, or any other characteristic protected by applicable law.

9.5 **Due Authorization**. Each Party represents and warrants that it is duly authorized to execute and deliver this Agreement and to perform its obligations hereunder.

9.6 **Anti-Corruption Laws**. Consultant represents and warrants that (a) the Services will be performed in accordance with all applicable current government regulatory requirements and all federal, state and local laws, rules, guidelines and regulations, including, but not limited to, all national and transnational anti-bribery statutes including, without limitation, the United States

Foreign Corrupt Practices Act, as amended from time to time, the UK Bribery Act 2010, and the OECD Anti-Bribery Convention (hereafter, "***Anti-Corruption Laws***"). To determine and ensure compliance with the "Anti-Corruption Laws", Consultant shall, upon reasonable advance notice, permit Company and its representatives during normal business hours to inspect and audit Consultant's business records. Consultant shall take such actions that are commercially feasible to adopt any reasonable suggestions of Company to correct any deficiencies identified by any inspection or audit conducted by Company. Consultant acknowledges that Company is committed to complying with all national and transnational anti-bribery statutes including, without limitation, compliance with the Anti-Corruption Laws and agrees that Consultant will comply with their provisions at all times with regard to the Services including, but not limited to, not offering or giving anything of value to a foreign public official in connection with the performance of the official's duties or inducing an official to use their position to influence any acts or decisions of any foreign, state or public international organization.

9.7 **Prohibition Against Insider Trading**. Consultant is aware that securities laws of the United States and other jurisdictions prohibit any person who has material, non-public information about a company from purchasing or selling securities of such company or from communicating such information to any person under circumstances in which it is reasonably foreseeable that such person is likely to purchase or sell such securities.

10. LIMITATION OF LIABILITY

CONSULTANT'S SOLE REMEDY UNDER THIS AGREEMENT SHALL BE AN ACTION AT LAW FOR DIRECT DAMAGES. IN NO EVENT SHALL COMPANY BE LIABLE TO CONSULTANT FOR ANY SPECIAL, INCIDENTAL, CONSEQUENTIAL, EXEMPLARY, PUNITIVE OR INDIRECT DAMAGES ARISING FROM THIS AGREEMENT OR THE SERVICES PROVIDED HEREUNDER (WHETHER IN CONTRACT, TORT, NEGLIGENCE, STRICT LIABILITY, BY STATUTE OR OTHERWISE). THIS LIMITATION SHALL APPLY EVEN IF COMPANY HAS BEEN ADVISED OR IS AWARE OF THE POSSIBILITY OF SUCH DAMAGES.

11. NOTICES

Any notice required or permitted by this Agreement shall be in writing and shall be delivered as follows with notice deemed given as indicated: (a) by personal delivery when delivered personally; (b) by overnight courier upon written verification of receipt; (c) by telecopy or facsimile transmission upon acknowledgment of receipt of electronic transmission; or (d) by certified or registered mail, return receipt requested, upon verification of receipt. Notice shall be sent to the addresses set forth below or such other address as either party may specify in writing:

If to Company:
Ovid Therapeutics Inc.
e-mail: **[***]**

If to Consultant:
Thomas Perone
e-mail: **[***]**

12. MISCELLANEOUS

12.1 **Jurisdiction; Governing Law.** This Agreement has been prepared in the English language, which language shall govern the interpretation of, and any disputes arising out of, this Agreement. Any claim, dispute, or controversy of whatever nature arising out of or relating to this Agreement shall be: (a) governed by and construed under the laws of the State of New York, without giving effect to any choice of law principles that would require the application of the laws of a different state or jurisdiction, and (b) brought exclusively in a court of competent jurisdiction, federal or state, located within New York County, New York and in no other jurisdiction, and each Party hereby consents to personal jurisdiction and venue in, and agrees to service of process issued or authorized by, such courts. Notwithstanding the foregoing, nothing contained in this Agreement shall deny either Party the right to seek injunctive or other equitable relief from any court of competent jurisdiction in the context of a bona fide emergency or prospective irreparable harm.

12.2 **Assignment**. Consultant may not subcontract or otherwise delegate or assign this Agreement or any of its obligations under this Agreement without the Company's prior written consent. Any attempted assignment in violation of the foregoing shall be null and void. Subject to the foregoing, this Agreement will be for the benefit of the Company's successors and assigns, and will be binding on Consultant's assignees.

12.3 **Modification or Amendment**. This Agreement may only be modified or amended in a writing signed by a duly authorized officer of Company and Consultant.

12.4 **Waiver.** Any waiver of the terms and conditions hereof must be explicitly in writing. Either Party's waiver of any breach of any provision hereof by the other Party is not a waiver of any succeeding breach of such provision or a waiver of the provision itself.

12.5 **Severability of Provisions**. Should any section, or portion thereof, of this Agreement be held invalid by reason of any law, statute, or regulation existing now or in the future in any jurisdiction by any court of competent authority or by a legally enforceable directive of any governmental body, such section or portion thereof will be validly reformed so as to approximate the intent of the Parties as nearly as possible and, if not reasonably capable of reform, will be deemed divisible and deleted with respect to such jurisdiction, but the Agreement will not otherwise be affected.

12.6 **Headings**. Section headings are not to be considered a part of this Agreement and are not intended to be a full and accurate description of the contents hereof.

12.7 **Counterparts**. This Agreement and any amendments hereto may be executed in one or more counterparts. All of such counterparts shall constitute one and the same Agreement and shall become effective when a copy signed by each Party has been delivered to the other Party. Delivery of an executed counterpart of a signature page of this Agreement by facsimile transmission, by electronic mail in "portable document format" (".pdf" format), or by any other electronic means intended to preserve the original graphic and pictorial appearance of a document, or by a combination of such means, shall be effective as delivery of a manually executed counterpart of this Agreement.

12.8 **Entire Agreement**. This Agreement and all Exhibits attached hereto constitute the entire agreement between the Parties with respect to the subject matter herein, and supersede all prior agreements, arrangements and understandings. Consultant represents that in executing this Agreement, it has not relied upon any representation or statement not set forth herein.

[*Signature page follows*]

IN WITNESS WHEREOF, the Parties have caused this Consulting Agreement to be executed by their duly authorized representative.

OVID THERAPEUTICS INC.

BY: _____ DocuSigned by: 5A89BA65C80E4E0...

NAME: Jeff Rona

TITLE: CBFO

CONSULTANT

BY: _____ DocuSigned by: Thomas Perone 0FD514EC00F9412...

NAME: Thomas Perone

EXHIBIT A

STATEMENT OF WORK

SERVICES:

To provide general business advice to the CEO as requested from time to time.

FEES AND REIMBURSEMENT:

A. Services Fee.

In consideration for the Services, the Company shall compensate Consultant as follows:

1. From the Effective Date through August 15, 2024, Consultant shall be paid a retainer fee of $20,623 for the Services described herein for up to 2.5 calendar days per week;

2. From the period of August 16, 2024 through September 15, 2024, Consultant shall be paid a retainer fee of $16,498 for the Services described herein for up to 2 calendar days per week;

3. From the period of September 16, 2024 through October 15, 2024, Consultant shall be paid a retainer fee of $8,249 for the Services described herein for up to 1 calendar days per week; and

4. From the period of October 16, 2024 through February 28, 2025, Consultant shall be paid a fee of $500 per hour for the Services described herein with such service and hours to be mutually agreed upon by the parties (collectively, the "**Services Fee**").

The Services Fee under items 1, 2 and 3 above shall be payable on October 15, 2024, as set forth in Section D below.

B. Expenses.

Company will reimburse Consultant for expenses in accordance with Section 3.2 of the Agreement

C. Maximum Compensation:

The maximum amount payable to Consultant (including expenses) in relation to the above Services, including all items in paragraphs A and B above, is $55,000.

D. Invoicing and Payment of Service Fee and Expenses:

Company shall pay Consultant for items 1, 2 and 3 above on October 15, 2024, without an invoice. From October 16, 2024 through February 28, 2024, Consultant shall submit monthly invoices for Services rendered and expenses incurred and shall provide such reasonable receipts or other documentation of expenses as Company might request, all in accordance with Section 3.1 and Section 3.2 of the Agreement. Consultant must address all invoices for Services to Company and send them electronically to **AP@ovidrx.com**.

E. Continuation as a Service Provider

The Parties intend that during the Term of this Agreement, Consultant shall be considered as remaining in Continuous Service for the purposes of the Ovid Therapeutics Inc. 2017 Equity Incentive Plan and other

relevant or successor plans, as applicable. Consultant's options shall continue to be governed by the plan and applicable grant notices and agreements under which the options were granted. As set forth therein, Consultant shall have a set number of days following the end of the Term of this Agreement to exercise the options.

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, 2024

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, 2024

By: /s/ Jeffrey Rona

Jeffrey Rona

Chief Business and Financial Officer
(Principal Financial Officer and Principal 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, 2024, 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.

Dated: November 12, 2024

/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)*