

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2023

OR

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

For the transition period from _____ to _____

Commission File Number: 001-38085

Ovid Therapeutics Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware
(State or Other Jurisdiction of
Incorporation or Organization)

46-5270895
(I.R.S. Employer
Identification Number)

441 Ninth Avenue, 14th Floor
New York, New York
(Address of principal executive offices)

10001
(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

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

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

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

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

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

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

As of May 2, 2023, the registrant had 70,521,340 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; 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**

	March 31, 2023	December 31, 2022
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	\$ 63,051,928	\$ 44,867,846
Marketable securities	54,668,093	84,133,565
Prepaid expenses and other current assets	1,799,343	2,379,280
Total current assets	119,519,364	131,380,691
Long-term equity investments	5,982,452	5,622,547
Restricted cash	1,930,253	1,930,753
Right-of-use asset, net	14,669,362	14,922,669
Property and equipment, net	1,053,063	1,147,963
Other assets	212,510	261,191
Total assets	\$ 143,367,004	\$ 155,265,814
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 1,831,490	\$ 1,952,910
Accrued expenses	4,085,369	4,504,669
Current portion, lease liability	804,139	533,946
Total current liabilities	6,720,998	6,991,525
Long-term liabilities:		
Lease liability	15,698,324	16,001,725
Total liabilities	22,419,322	22,993,250
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 March 31, 2023 and December 31, 2022	1	1
Common stock, \$0.001 par value; 125,000,000 shares authorized; 70,491,510 and 70,466,885 shares issued and outstanding at March 31, 2023 and December 31, 2022, respectively	70,492	70,467
Additional paid-in-capital	359,754,310	357,770,825
Accumulated other comprehensive income (loss)	5,630	(42,187)
Accumulated deficit	(238,882,751)	(225,526,542)
Total stockholders' equity	120,947,682	132,272,564
Total liabilities and stockholders' equity	\$ 143,367,004	\$ 155,265,814

See accompanying notes to these unaudited condensed consolidated financial statements

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

	For The Three Months Ended March 31, 2023	For The Three Months Ended March 31, 2022
Revenue:		
License and other revenue	\$ 66,160	\$ 1,445,366
Total revenue	66,160	1,445,366
Operating expenses:		
Research and development	6,614,717	7,832,269
General and administrative	8,343,748	9,880,203
Total operating expenses	14,958,465	17,712,472
Loss from operations	(14,892,305)	(16,267,106)
Other income (expense), net	1,536,095	209,050
Loss before provision for income taxes	(13,356,209)	(16,058,056)
Provision for income taxes	—	50,000
Net loss	\$ (13,356,209)	\$ (16,108,056)
Net loss per share, basic	\$ (0.19)	\$ (0.23)
Net loss per share, diluted	\$ (0.19)	\$ (0.23)
Weighted-average common shares outstanding, basic	70,490,704	70,345,828
Weighted-average common shares outstanding, diluted	70,490,704	70,345,828

See accompanying notes to these unaudited condensed consolidated financial statements

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

	For The Three Months Ended March 31, 2023	For The Three Months Ended March 31, 2022
Net loss	\$ (13,356,209)	\$ (16,108,056)
Other comprehensive income:		
Unrealized gain on marketable securities	47,817	—
Comprehensive loss	<u>\$ (13,308,392)</u>	<u>\$ (16,108,056)</u>

See accompanying notes to these unaudited condensed consolidated financial statements

OVID THERAPEUTICS INC.

Condensed Consolidated Statements of Stockholders' Equity (unaudited)

	Series A Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total
	Shares	Amount	Shares	Amount				
Balance, December 31, 2022	1,250	\$ 1	70,466,885	\$ 70,467	\$ 357,770,825	\$ (42,187)	\$ (225,526,542)	\$ 132,272,564
Issuance of common stock from exercise of stock options and purchases from employee stock purchase plan	—	—	24,625	25	66,968	—	—	66,993
Stock-based compensation expense	—	—	—	—	1,916,518	—	—	1,916,518
Other comprehensive income	—	—	—	—	—	47,817	—	47,817
Net loss	—	—	—	—	—	—	(13,356,209)	(13,356,209)
Balance, March 31, 2023	1,250	\$ 1	70,491,510	\$ 70,492	\$ 359,754,310	\$ 5,630	\$ (238,882,751)	\$ 120,947,682
	Series A Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total
	Shares	Amount	Shares	Amount				
Balance, December 31, 2021	1,250	\$ 1	70,364,912	\$ 70,359	\$ 351,033,589	\$ —	\$ (171,357,513)	\$ 179,746,436
Issuance of common stock from exercise of stock options and purchases from employee stock purchase plan	—	—	52,333	14	33,065	—	—	33,079
Stock-based compensation expense	—	—	—	—	1,324,812	—	—	1,324,812
Net income	—	—	—	—	—	—	(16,108,056)	(16,108,056)
Balance, March 31, 2022	1,250	\$ 1	70,417,245	\$ 70,373	\$ 352,391,466	\$ —	\$ (187,465,569)	\$ 164,996,271

See accompanying notes to these unaudited condensed consolidated financial statements

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

	For The Three Months Ended March 31, 2023	For The Three Months Ended March 31, 2022
Cash flows from operating activities:		
Net loss	\$ (13,356,209)	\$ (16,108,056)
Adjustments to reconcile net loss to cash used in operating activities:		
Non-cash consideration received in licensing agreement transaction	—	(945,366)
Unrealized gain on equity investment	(359,905)	(207,068)
Interest income and accretion of discount on marketable securities	(729,119)	—
Stock-based compensation expense	1,916,518	1,324,812
Depreciation and amortization expense	144,650	73,807
Amortization of right-of-use asset	253,307	—
Accretion of lease liability	288,501	88,291
Change in operating assets and liabilities:		
Prepaid expenses and other current assets	258,228	262,437
Security deposit	12,491	(4,305)
Accounts payable	(121,420)	(4,870,271)
Accrued expenses	(419,302)	299,300
Net cash used in operating activities	<u>(12,112,260)</u>	<u>(20,086,419)</u>
Cash flows from investing activities:		
Purchase of marketable securities	(9,757,591)	—
Sales/maturities of marketable securities	40,000,000	—
Issuance of short-term note receivable	—	(1,000,000)
Purchases of property and equipment	(13,560)	(45,935)
Software development and other costs	—	(30,230)
Net cash provided by (used in) investing activities	<u>30,228,849</u>	<u>(1,076,165)</u>
Cash flows from financing activities:		
Proceeds from exercise of options and purchases from employee stock purchase plan	66,993	33,079
Net cash provided by financing activities	<u>66,993</u>	<u>33,079</u>
Net increase (decrease) in cash, cash equivalents and restricted cash	18,183,582	(21,129,505)
Cash, cash equivalents and restricted cash, at beginning of period	46,798,599	189,728,285
Cash, cash equivalents and restricted cash, at end of period	<u>\$ 64,982,181</u>	<u>\$ 168,598,780</u>
Non-cash investing and financing activities:		
Right-of-use asset in exchange for lease liability	\$ —	\$ 15,779,773

See accompanying notes to these unaudited condensed consolidated financial statements

OID 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 committed to developing medicines that transform the lives of people with epilepsies and seizure-related disorders.

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 and other equity instruments. 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 March 31, 2023, the Company had approximately \$117.7 million in cash, cash equivalents and marketable securities. Since inception, the Company has generated \$222.6 million in revenue, primarily from the Company’s royalty, license and termination agreement (“RLT Agreement”) with Takeda Pharmaceutical Company Limited (“Takeda”). Historically, the Company has incurred recurring losses, has experienced negative operating cash flows and requires significant cash resources to execute its business plans. The Company has an accumulated deficit of \$238.9 million as of March 31, 2023, working capital of \$112.8 million and had cash used in operating activities of \$12.1 million for the three months ended March 31, 2023.

The Company recorded a net loss of \$13.4 million during the three months ended March 31, 2023, 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 March 31, 2023 will be sufficient to fund its current operating plans through at least the next 12 months from the date of filing of the Company’s Quarterly Report on Form 10-Q. Adequate additional funding may not be available to the Company 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. The Company may be required to delay, reduce the scope of or eliminate 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; and obtaining regulatory approval of any of the Company’s product candidates.

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 13, 2023.

(A) Unaudited Interim Condensed Consolidated Financial Statements

The interim condensed consolidated balance sheet at March 31, 2023 and the condensed consolidated statements of operations, comprehensive loss, cash flows, and stockholders’ equity for the three months ended March 31, 2023 and 2022 are unaudited. The accompanying unaudited condensed consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles (“GAAP”) and following the requirements of the SEC for interim reporting. As permitted under those rules, certain footnotes 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 month periods ended March 31, 2023 and 2022 are not necessarily indicative of the results to be expected for the year ending December 31, 2023 or for any other future annual or interim period. The balance sheet as of December 31, 2022 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, 2022 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 subsidiary, Ovid Therapeutics Hong Kong Limited. 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 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 in stockholder's 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 an equity investment in the preferred shares of Gensaic, Inc., formerly M13 Therapeutics, Inc. ("Gensaic"), a privately held corporation. The preferred shares are not considered in-substance common stock, and the investment is accounted for at cost, with adjustments for observable changes in prices or impairments, and is classified within long-term equity investments on our consolidated balance sheets with adjustments recognized in other (expense) income, net on our consolidated statements of operations. The Company has determined that the equity investment does not have a readily determinable fair value and elected the measurement alternative. Therefore, the equity investment's carrying amount 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 investment is impaired. The assessment includes a review of recent operating results and trends, recent sales/acquisitions of the investee's securities, and other publicly available data. If the investment is impaired, the Company writes it down to its estimated fair value. As of March 31, 2023 and December 31, 2022, the equity investment in Gensaic had a carrying value of \$5.1 million.

Long-term equity investments also consist of an equity investment in the common shares of Marinus Pharmaceuticals, Inc. ("Marinus") 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 consolidated balance sheets and other income (expense) on the Company's consolidated statements of operations. As of March 31, 2023 and December 31, 2022, the equity investment in Marinus had a carrying value of approximately \$0.9 million and \$0.5 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 \$61.6 million as of March 31, 2023. The Company's Level 1 assets totaled \$42.5 million as of December 31, 2022.
- 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 \$54.7 million as of March 31, 2023 and \$84.1 million as of December 31, 2022.
- Level 3—Unobservable inputs for the asset or liability. Financial instruments are considered Level 3 when their fair values are determined using pricing models, discounted cash flows or similar techniques and at least one significant model assumption or input is unobservable. There were no Level 3 assets or liabilities as of March 31, 2023 or December 31, 2022.

The carrying amounts reported in the balance sheets for cash and cash equivalents, other current assets, accounts payable and accrued expenses approximate their fair value 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 ASC 842. Operating leases are included in right-of-use "ROU" assets, current liabilities, and long-term lease liability in the Company's 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 award 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) 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.

(M) 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.

(N) 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.

(O) Recent Accounting Pronouncements

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

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 March 31, 2023 and December 31, 2022:

	March 31, 2023			
	Amortized cost	Gross unrealized holding gains	Gross unrealized holding losses	Fair value
Cash	\$ 2,346,359	\$ —	\$ —	\$ 2,346,359
Money market funds	60,705,569	—	—	60,705,569
Marketable securities	54,662,463	5,630	—	54,668,093
Total cash, cash equivalents and marketable securities	<u>\$ 117,714,391</u>	<u>\$ 5,630</u>	<u>\$ —</u>	<u>\$ 117,720,021</u>

	December 31, 2022			
	Amortized cost	Gross unrealized holding gains	Gross unrealized holding losses	Fair value
Cash	\$ 2,853,042	\$ —	\$ —	\$ 2,853,042
Money market funds	42,014,804	—	—	42,014,804
Marketable securities	84,175,752	—	(42,187)	84,133,565
Total cash, cash equivalents and marketable securities	<u>\$ 129,043,598</u>	<u>\$ —</u>	<u>\$ (42,187)</u>	<u>\$ 129,001,411</u>

The Company did not hold any securities that were in an unrealized loss position for more than 12 months as of March 31, 2023 and December 31, 2022.

There were no material realized gains or losses on available-for-sale securities during the three months ended March 31, 2023 and 2022.

NOTE 4 – PROPERTY AND EQUIPMENT AND INTANGIBLE ASSETS

Property and equipment is summarized as follows:

	March 31, 2023	December 31, 2022
Furniture and equipment	\$ 1,436,592	\$ 1,423,032
Leasehold improvements	306,312	306,312
Less accumulated depreciation	(689,841)	(581,381)
Total property and equipment, net	<u>\$ 1,053,063</u>	<u>\$ 1,147,963</u>

Depreciation expense was \$108,460 and \$30,839 for the three months ended March 31, 2023 and 2022, respectively.

Intangible assets, net of accumulated amortization, were \$185,910 and \$222,100 as of March 31, 2023 and December 31, 2022, respectively, and are included in other assets. Amortization expense was \$36,190 and \$32,000 for the three months ended March 31, 2023 and 2022, 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 a 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 10 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 is 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:

	March 31, 2023
Right-of-use asset	\$ 14,669,362
Current lease liability	804,139
Long-term lease liability	\$ 15,698,324

The components of operating lease cost for the three months ended March 31, 2023 were as follows:

	March 31, 2023
Operating lease cost	\$ 541,808
Variable lease cost	—
Short-term lease cost	—

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

2023	1,351,177
2024	2,316,303
2025	2,316,303
2026	2,316,303
2027	2,316,303
Thereafter	12,347,235
	\$ 22,963,624

NOTE 6 – ACCRUED EXPENSES

Accrued expenses consist of the following:

	March 31, 2023	December 31, 2022
Payroll and bonus accrual	\$ 1,895,602	\$ 3,233,802
Research and development accrual	1,343,672	395,247
Professional fees accrual	675,234	682,664
Other	170,861	192,956
Total	\$ 4,085,369	\$ 4,504,669

NOTE 7 – STOCKHOLDERS' EQUITY

The Company's capital structure consists of common stock and convertible 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 1,250 of the 10,000,000 authorized shares of preferred stock as non-voting Series A Convertible Preferred Stock ("Series A Preferred Stock").

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 March 31, 2023 and December 31, 2022. 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.

In November 2020, the Company entered into a sales agreement (the "2020 ATM agreement") with Cowen and Company, LLC ("Cowen"), under which the Company may offer and sell in "at the market offerings," from time to time at its sole discretion, shares of its common stock having an aggregate offering price of up to \$75.0 million through Cowen acting as sales agent. As of March 31, 2023, the Company has not sold any shares of its common stock under the 2020 ATM agreement.

Dividends

Through March 31, 2023, 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, 2023 and January 1, 2022 an additional 3,523,344 and 1,000,000 shares, respectively, were reserved for issuance under the 2017 Plan. As of March 31, 2023, there were 4,243,849 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 March 31, 2023 and 2022, 29,830 and 38,583 shares, respectively, were purchased under the 2017 ESPP, and the Company recorded expense of \$15,154 and \$21,019, 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. The Board acted prior to each of January 1, 2023 and January 1, 2022 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 March 31, 2023, there were 386,777 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 stockholder's 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 March 31, 2023, options to purchase 1,663,597 shares of common stock were outstanding under the 2014 Plan.

Unless specified otherwise in an individual option agreement, stock options granted under the prior 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 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 12 months.

Performance-based option awards generally have similar terms, with vesting commencing on the date the performance condition is achieved and expire in accordance with the specific terms of the agreement. At March 31, 2023, there were 100,000 performance-based options outstanding and unvested that include options to be granted upon the achievement of certain research and development milestones.

The fair value of options granted during the three months ended March 31, 2023 and 2022 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 were 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 was based on the simplified method in accordance with the SEC Staff Accounting Bulletin No. Topic 14D. Beginning in January 1, 2023, the expected volatility was estimated based on historical volatility information of the Company since the Company's initial public offering.

All assumptions used to calculate the grant date fair value of nonemployee options are generally consistent with the assumptions used for options granted to employees. In the event the Company terminates any of its consulting agreements, the unvested options underlying the agreements would also be canceled.

The Company did not grant any stock options to nonemployee consultants for services rendered during the three months ended March 31, 2023 and 2022. There were 125,303 and 175,938 unvested nonemployee options outstanding as of March 31, 2023 and 2022, respectively. Total expense recognized related to nonemployee stock options for the three months ended March 31, 2023 and 2022, was \$108,585 and \$195,000, respectively. Total unrecognized compensation expenses related to the nonemployee stock options was \$518,392 as of March 31, 2023. The Company did not recognize any expense for nonemployee performance-based option awards during the three months ended March 31, 2023 or 2022.

The Company granted 2,660,500 and 2,426,750 stock options to employees during the three months ended March 31, 2023 and 2022, respectively. There were 7,372,384 and 5,954,029 unvested employee options outstanding as of March 31, 2023, and 2022, respectively. Total expense recognized related to the employee stock options for the three months ended March 31, 2023 and 2022 was \$1.8 million and \$1.1 million, respectively. Total unrecognized compensation expense related to employee stock options was \$14.2 million as of March 31, 2023. No expense for employee performance-based options was recognized during the three months ended in March 31, 2023 and 2022.

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

	Three Months Ended March 31,	
	2023	2022
Research and development	\$ 510,261	\$ 395,492
General and administrative	1,406,257	929,320
Total	<u>\$ 1,916,518</u>	<u>\$ 1,324,812</u>

	Three Months Ended March 31,	
	2023	2022
Stock options	\$ 1,901,364	\$ 1,303,793
Employee Stock Purchase Plan	15,154	21,019
Total	\$ 1,916,518	\$ 1,324,812

The fair value of employee options granted during the three months ended March 31, 2023 and 2022 was estimated utilizing the following assumptions:

	Three Months Ended March 31,	
	2023	2022
	Weighted Average	Weighted Average
Volatility	84.61 %	87.17 %
Expected term in years	6.06	6.06
Dividend rate	0.00 %	0.00 %
Risk-free interest rate	4.00 %	1.72 %
Fair value of option on grant date	\$ 1.84	\$ 2.00

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, 2022	12,961,238	\$ 4.13	7.42	\$ 62,158
Granted	2,660,500	2.50	9.91	
Exercised	(9,625)	1.91		
Forfeited or expired	(193,936)	3.38		
Options outstanding March 31, 2023	15,418,177	\$ 3.86	7.57	\$ 1,028,677
Vested and exercisable at March 31, 2023	7,920,490	\$ 4.75	6.23	\$ 694,303

At March 31, 2023 there was approximately \$14.7 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 2.57 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 March 31, 2023, the Company was in a pre-tax loss position, and is anticipated to remain so throughout the year.

For the three months ended March 31, 2023, the Company did not record any tax benefit or expense.

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

including cumulative losses in recent years, the Company continues to maintain a full valuation allowance against its net deferred tax assets as of March 31, 2023.

NOTE 10 – COMMITMENTS AND CONTINGENCIES

License Agreements

Northwestern University License Agreement

In December 2016, the Company entered into a license 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 Patents. 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 Patents, 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 Patents, 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

On December 30, 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. Since the intangibles acquired in the AstraZeneca license agreement do not have an alternative future use, all costs incurred were treated as research and development expense. The Company recorded a total of \$12.3 million as research and development expense related to this agreement during December 2021.

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 (“Collaboration Agreement”) with Gensaic. The Collaboration Agreement involves the research and development of phage-derived particle (“PDP”) products on Gensaic's proprietary platform for certain central nervous system rare disorder targets.

Under the 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.

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 this agreement by providing written notice to Gensaic 90 days in advance of the termination date.

As of March 31, 2023, none of these contingent payments were considered probable.

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.

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

On January 6, 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 were 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 will no longer have any financial obligation to Takeda under the original collaboration agreement, including milestone payments or any future development and commercialization costs. On March 29, 2021 upon the closing of the RLT Agreement, the Company received an upfront payment of \$196.0 million and, if soticlestat is successfully developed, will be eligible to receive up to an additional \$660.0 million upon Takeda achieving developmental, regulatory and sales milestones. In addition, the Company will 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 will be payable on a country-by-country and product-by-product basis for any indications that soticlestat is approved for 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.

The Company identified the following material promises under the RLT Agreement: (1) no later than the second business day prior to the closing of the RLT Agreement (the “Closing Date”), the Company and Takeda were required to agree on an estimate of the development expenses that accrued, or would accrue, under the original collaboration agreement as of March 31, 2021; (2) on the Closing Date, the Company was required to (i) provide and transfer to Takeda the materials, information and data relating to the soticlestat program, including clinical trial data and results, as further set forth in the RLT Agreement, (ii) assign to Takeda certain agreements applicable to the soticlestat program, and (iii) assign to Takeda all of its right, title and interest in, to and under all intellectual property rights developed or created pursuant to the original collaboration agreement and owned jointly by the Company and Takeda as of the Closing Date; (3) within 45 days after March 31, 2021, the Company and Takeda were required to provide a written report to the finance officer designated by the other party setting forth a final total of the development expenses that accrued as of March 31, 2021 and, within 10 business days after receipt of such report, the finance officers shall agree on whether a net settlement payment is due from Takeda to the Company or from the Company to Takeda; and (4) within 75 days after the Closing Date, to the extent not provided on the Closing Date, Ovid shall provide to Takeda (i) any materials, information and data relating to the soticlestat program, including clinical trial data and results, as further set forth in the RLT Agreement, (ii) other documents (including all expired agreements and related data developed thereunder) to the extent relating to the soticlestat program that are necessary for the exploitation, development, commercialization and manufacture of soticlestat, as further set forth in the RLT Agreement and (iii) any tangible embodiment of the intellectual property rights controlled by Ovid that are reasonably necessary for, used in or held for use in Takeda’s exploitation of the soticlestat program.

The Company determined the transaction price is equal to the upfront fee of \$196.0 million and is associated with all four performance obligations identified above. It is noted that the incremental effort associated with performance obligations three and four is negligible and not material in the context of the RLT Agreement since all of the information is related to the collaboration period for which the Company already has the information readily available. Therefore, since they are not material in the context of the RLT Agreement, the full upfront fee was allocated to the two performance obligations satisfied at closing.

During the three months ended March 31, 2023, no expense was recognized pursuant to the RLT Agreement.

Healx License and Option Agreement

On February 1, 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 has secured a one-year option to investigate gaboxadol (“OV101”) as part of a potential combination therapy for Fragile X syndrome in a Phase 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.

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 a 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 this agreement during the three months ended March 31, 2023. During the three months ended March 31, 2022, the Company recorded revenue of \$0.5 million associated with the Healx License and Option Agreement.

Marinus Pharmaceuticals Out-License Agreement

On March 1, 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 consist 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 on March 18, 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 recorded revenue and an associated investment in equity securities of approximately \$0.9 million related to the patent license agreement on March 18, 2022, based on the price of Marinus common stock on March 1, 2022. The Company had unrealized gains on the Marinus common stock of \$0.4 million and \$0.2 million for the three months ended March 31, 2023 and 2022, respectively, which were recorded as unrealized gains on equity securities and are reflected 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.

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 March 31,	
	2023	2022
Net loss	\$ (13,356,209)	\$ (16,108,056)
Net loss attributable to participating securities	—	—
Net loss attributable to common stockholders	\$ (13,356,209)	\$ (16,108,056)

	For the Three Months Ended March 31,	
	2023	2022
Net loss attributable to common stockholders	\$ (13,356,209)	\$ (16,108,056)
Weighted average common shares outstanding used in computing net loss per share - basic	70,490,704	70,345,828
Weighted average common shares outstanding used in computing net loss per share - diluted	70,490,704	70,345,828
Net loss per share, basic	\$ (0.19)	\$ (0.23)
Net loss per share, diluted	\$ (0.19)	\$ (0.23)

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 March 31,	
	2023	2022
Stock options to purchase common stock	15,418,577	12,755,657
Common stock issuable upon conversion of Series A convertible preferred stock	1,250,000	1,250,000

NOTE 14 – SUBSEQUENT EVENT

Graviton License Agreement and Equity Purchase

On April 30, 2023, the Company entered into an agreement with Graviton Bioscience Corporation (“Graviton”), whereby it secured from Graviton an exclusive license to develop and commercialize Graviton's library of ROCK2 inhibitors including their lead program GV101 in rare central nervous system (“CNS”) disorders (excluding amyotrophic lateral sclerosis) worldwide (excluding China, Hong Kong, Macau and Taiwan). Under the agreement, the Company and Graviton plan to investigate 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 collaboration the Company also purchased shared of Graviton's Series A Preferred Stock for \$10 million.

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, 2022, which was filed with the Securities and Exchange Commission ("SEC") on March 13, 2023. 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 that transform the lives of people with epilepsies and seizure-related disorders in a manner that is scientifically driven, patient focused and is coupled with an integrated and disciplined approach to research, clinical development and business development. Our team has significant experience and understanding of rare epilepsies and seizure-related neurological conditions, and we continue to build insight into the way the different molecular mechanisms and pathways underlying these disorders impact the symptoms patients suffer. We have set out to be a leader in the field, and have developed a differentiated pipeline containing three novel mechanisms of action to target different causes of epilepsies and seizures. Our knowledge of epilepsy disease biology and pathology, which was acquired through our small molecule development programs, now contributes to our pursuit of additional relevant genetic targets and molecular pathways. Over time, we have built a scalable scientific platform and efficient development capabilities in epilepsies that focus on clear, clinical endpoints. We are initially pursuing therapeutic assets for rare disorders as they can leverage accelerated development programs. If successfully developed and marketed in rare conditions, we intend to explore these assets for broader neurologic indications, as applicable. Our cohesive focus in rare epilepsies and seizures 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 March 31, 2023, we have raised net proceeds of \$275.4 million from the sale of our convertible preferred stock and our common stock. We have also, in previous periods, generated revenue through license and collaboration agreements. As of March 31, 2023, we had \$117.7 million in cash, cash equivalents and marketable securities. As of March 31, 2023, we had an accumulated deficit of \$238.9 million.

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

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

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

Advancing differentiated epilepsy & seizure programs

First-in-class or best-in-class mechanisms of action

EPILEPSY PROGRAMS	INDICATION/TARGET	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	ANTICIPATED MILESTONES
SOTICLESTAT	Dravet syndrome					Phase 3 data from 2 registrational trials
Cholesterol 24-hydroxylase inhibitor Out-licensed to 	Lennox-Gastaut syndrome					Filing marketing authorization submissions in Takeda's FY 2024
GV101* 	Brainstem cavernous malformations Undisclosed indications					- MAD initiation H2 2023 - Phase 2 FPI 2024
Selective ROCK2 inhibitor Collaboration with 						
OV329	Tuberous sclerosis complex, infantile spasms and focal seizures					Phase 1 target engagement & safety in H1 2024
GABA-aminotransferase inhibitor In-licensed from: 						
DV350 & KCC2 PORTFOLIO	1. Resistant epilepsies and other neuropathologies					IND in 2024
KCC2 transporter activator In-licensed from: 						
UNDISCLOSED ROCK2 INHIBITOR PROGRAMS						

*Graviton is conducting development of GV101 through Phase 2 and such development will be directed by a Joint Development Committee that includes members of both Graviton and Ovid.

In the first quarter of 2023, Takeda provided a corporate update in which it reiterated that the anticipated timeline for regulatory filing of the two pivotal Phase 3 trials evaluating soticlestat for Lennox-Gastaut and Dravet syndromes are expected in Takeda's 2024 fiscal year.

In the fourth quarter of 2022, the FDA cleared our investigational new drug application ("IND") for OV329, a GABA-aminotransferase inhibitor, and we subsequently initiated a Phase 1 trial in healthy volunteers.

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 the ongoing war between Russia and Ukraine 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. Rising 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, the global geopolitical tension as a result of the ongoing war between Russia and Ukraine, 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; 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 unrealized gains (losses) on long-term equity investments and interest income and accretion of discount on investments in marketable securities.

Reclassifications

Certain prior period amounts have been reclassified for consistency with the current period presentation. These reclassifications had no effect on the reported results of operations.

Results of Operations

Comparison of the Three Months Ended March 31, 2023 and 2022

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

	Three Months Ended March 31, 2023	Three Months Ended March 31, 2022	Change \$
	(in thousands)		
Revenue:			
License and other revenue	\$ 66	\$ 1,445	\$ (1,379)
License revenue - related party	—	—	—
Total revenue	66	1,445	(1,379)
Operating expenses:			
Research and development	6,615	7,832	(1,217)
General and administrative	8,344	9,880	(1,536)
Total operating expenses	14,958	17,712	(2,753)
Loss from operations	(14,892)	(16,267)	1,375
Other income (expense), net	1,536	159	1,377
Loss before provision for income taxes	(13,356)	(16,109)	2,753
Provision for income taxes	—	—	—
Net loss	<u>\$ (13,356)</u>	<u>\$ (16,109)</u>	<u>\$ 2,753</u>

Revenue

Royalty revenue of \$66,160 was generated in the three months ended March 31, 2023, compared to revenue of \$1.4 million that was recognized in the same period in 2022 relating to licensing agreements.

Research and Development Expenses

	Three Months Ended March 31, 2023	Three Months Ended March 31, 2022	Change \$
	(in thousands)		
Preclinical and development expenses	\$ 2,983	\$ 2,120	\$ 863
Payroll and payroll-related expenses	2,854	4,936	(2,082)
Other expenses	778	776	2
Total research and development	<u>\$ 6,615</u>	<u>\$ 7,832</u>	<u>\$ (1,217)</u>

During the three months ended March 31, 2023, total research and development expenses were \$6.6 million compared to \$7.8 million for the same period in 2022. The decrease of \$1.2 million was primarily comprised of \$2.0 million reduction in payroll and related expenses following an organizational restructuring in 2022, partially offset by a \$0.6 million increase in preclinical and development expenses relating to the OV329 phase 1 clinical trial.

General and Administrative Expenses

	Three Months Ended March 31, 2023	Three Months Ended March 31, 2022	Change \$
	(in thousands)		
Payroll and payroll-related expenses	\$ 4,746	\$ 4,768	\$ (22)
Legal and professional fees	1,880	3,092	(1,212)
General office expenses	1,717	2,020	(303)
Total general and administrative	\$ 8,344	\$ 9,880	\$ (1,536)

General and administrative expenses were \$8.3 million for the three months ended March 31, 2023 compared to \$9.9 million for the same period in 2022. The decrease of \$1.5 million is primarily due to a reduction in legal and consulting fees and a decrease in general office expenses.

Provision for Income Taxes

There was no tax benefit or expense recorded for the three months ended March 31, 2023 and 2022.

Other Income (Expense), net

Other income (expense), net for the three months ended March 31, 2023 includes unrealized gain (loss) on long-term equity investments and interest earned on marketable securities, and included interest income and other nominal items for the three months ended March 31, 2022.

Liquidity and Capital Resources

Overview

As of March 31, 2023, we had total cash, cash equivalents and marketable securities of \$117.7 million as compared to \$129.0 million as of December 31, 2022. We believe that our cash, cash equivalents and marketable securities as of March 31, 2023 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. With the exception of the three months ended March 31, 2021, when we received a one-time upfront payment of \$196.0 million as part of the RLT Agreement, we have incurred losses and experienced negative operating cash flows since our inception and anticipate that we will continue to incur losses and experience negative operating cash flows for at least the next several years. We recorded net losses of approximately \$13.4 million and \$16.1 million for the three months ended March 31, 2023 and 2022, respectively. As of March 31, 2023, we had an accumulated deficit of \$238.9 million and working capital of \$112.8 million.

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 March 31, 2023, 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 approximately \$1.9 million in the 12 months subsequent to March 31, 2023 and approximately \$23.5 million over the 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, inflation rates have increased recently to levels not seen in decades, contributing to the ongoing economic slowdown. Increased inflation may result in increased 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 is expected to further raise, interest rates. Increases in 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 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.

At-the-Market Offering Program

In November 2020, we filed a shelf registration statement on Form S-3 (Registration No. 333-250054) that allows us to sell up to an aggregate of \$250.0 million of our common stock, preferred stock, debt securities and/or warrants ("S-3 Registration Statement"), 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 March 31, 2023, we had up to \$250.0 million available under our S-3 Registration Statement, including up to \$75.0 million available pursuant to our ATM offering program.

Cash Flows

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

	Three Months Ended March 31, 2023	Three Months Ended March 31, 2022
	(in thousands)	
Net cash (used in) provided by:		
Operating activities	\$ (12,112)	\$ (20,086)
Investing activities	30,229	(1,076)
Financing activities	67	33
Net increase (decrease) in cash, cash equivalents, and restricted cash	<u>\$ 18,184</u>	<u>\$ (21,130)</u>

Net Cash Used In Operating Activities

Net cash used in operating activities was \$12.1 million for the three months ended March 31, 2023, which consisted of net loss of \$13.4 million offset by a net of \$1.2 million of non-cash charges and indirect cash charges,

primarily related to \$1.9 million of stock-based compensation expense. Net cash used in operating activities was \$20.1 million for the three months ended March 31, 2022, which consisted of net loss of \$16.1 million offset by a net of \$2.8 million of non-cash charges and indirect cash changes, primarily related to \$1.3 million of stock-based compensation expense, and decreases in accounts payable and accrued expenses of \$4.6 million.

Net Cash Provided By (Used In) Investing Activities

Net cash provided by investing activities was \$30.2 million for the three months ended March 31, 2023, which was due to sales/maturities of marketable securities during the period. Net cash used in investing activities was \$1.1 million for the three months ended March 31, 2022.

Net Cash Provided By Financing Activities

Net cash provided by financing activities during the three months ended March 31, 2023 and 2022 resulted from proceeds from the exercise of options under the 2017 equity incentive plan and purchases of shares under the 2017 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 months ended March 31, 2023, there were no material changes to our critical accounting policies as reported for the year ended December 31, 2022 as part of our Annual Report on Form 10-K, which was filed with the SEC on March 13, 2023. In addition, see Note 2 of our Condensed Financial Statements under the heading “Recent Accounting Pronouncements” for new accounting pronouncements or changes to the accounting pronouncements during the three months ended March 31, 2023.

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 March 31, 2023, we had cash, cash equivalents and marketable securities totaling \$117.7 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 March 31, 2023, 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 March 31, 2023, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

There have been no changes in our internal control over financial reporting during our most recent quarter ended March 31, 2023 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 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 you may lose all or part of your 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 is subject to numerous 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 continue to incur substantial operating losses for the foreseeable future and may never achieve or maintain profitability.
- Our operating history may make it difficult to evaluate the success of our business to date and to assess our future viability.
- 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 all our drug candidates are in 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.

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 with the exception of net income we reported in 2021 primarily due to a one-time, upfront payment of \$196.0 million received pursuant to the RLT Agreement with Takeda. As of March 31, 2023, we had an accumulated deficit of \$238.9 million. We expect to incur increasing operating losses for the foreseeable 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. All 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 continue to incur significant expenses and operating losses over the next several years, 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 to advance 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 expenses associated with the 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.

As of March 31, 2023, our cash, cash equivalents and marketable securities were \$117.7 million, and we had an accumulated deficit of \$238.9 million. We believe that our 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.

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.

While the long-term economic impacts of the COVID-19 pandemic and the ongoing war between Russia and Ukraine 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 response to concerns about inflation, the U.S. Federal Reserve has raised, and is expected to further raise, interest rates. Increases in 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.

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 incurred in taxable years beginning after December 31, 2020, is limited.

In addition, under Section 382 and Section 383 of the Internal Revenue Code of 1986, as amended (“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 that 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 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 March 31, 2023, we recorded no U.S. federal or state income tax provision, based on a pre-tax loss of approximately \$13.4 million. As of March 31, 2023, we had available approximately \$130.9 million of unused NOL carryforwards for federal income tax purposes, \$13.0 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 a material portion of our NOL carryforwards and certain other tax attributes, which could have a material adverse effect on our 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 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 and all our drug candidates are in preclinical development. If we are unable to successfully develop, receive regulatory approval for and commercialize our drug candidates for these or any other indications, or successfully develop any other drug candidates, or experience significant delays in doing so, our business will be harmed.

We are very early in our development efforts and most of the drug candidates for which we control developmental and commercial responsibility are still in preclinical development. For example, we previously announced that we anticipate filing three IND applications in three years, beginning in 2022; however, we cannot guarantee success of preclinical development to achieve all such IND applications or whether FDA will impose clinical holds on any of these INDs. Following IND acceptance, each of our drug candidates will need to be progressed through clinical development in order to achieve regulatory approval, and we will also need to address issues relating to manufacture and supply, which may involve building our own capacity and expertise. 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.

Soticlestat, the most advanced compound we helped to develop, is continuing to be developed by Takeda and is currently in a pivotal trial program. If the pivotal trials are unsuccessful, or the compound is not approved, we will not receive the milestone payments and royalties from the RLT Agreement. Without those funds, we may need to raise significant additional capital to pursue the development and commercialization of our current and future pipeline.

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. For instance, our NEPTUNE trial of OV101, one of our former drug candidates, did not meet its primary endpoints despite earlier encouraging results from our Phase 2 trial STARS, the first clinical trial evaluating efficacy of OV101 in patients with Angelman syndrome. We closed our OV101 program in Angelman syndrome in early 2021. 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.

It is difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval for our gene therapy candidates.

Our future success depends in part on the successful development of our early-stage gene therapy product candidates. We may experience delays in developing a sustainable, reproducible, and scalable manufacturing process or transferring that process to internal and external commercial manufacturing sites, which may prevent us from initiating or completing our clinical trials or commercializing our product candidates on a timely or profitable basis, if at all.

The regulatory approval process for novel gene therapy products such as ours can be more expensive and take longer than for other product types, which are better known or more extensively studied to date. Regulatory approaches and requirements for gene therapy products continue to evolve, and any changes could create significant delay and unpredictability for product development and approval as compared to technologies with which regulatory authorities have more substantial experience.

Also, before a clinical trial can begin to enroll at a site, each clinical site's Institutional Review Board ("IRB") and its Institutional Biosafety Committee will have to review the proposed clinical trial to assess appropriateness to conduct the clinical trial at that site. In addition, adverse events in clinical trials of gene therapy products conducted by others may

cause the FDA or other regulatory authorities outside the U.S. to change the requirements for human research on or for approval of any of our product candidates.

Negative public opinion and increased regulatory scrutiny of gene therapy and genetic research may damage public perception of our product candidates or adversely affect our ability to conduct our business or obtain marketing approvals for our product candidates.

Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. Trials using early versions of retroviral vectors, which integrate into, and thereby alter, the host cell's DNA, have led to several well-publicized adverse events. The risk of serious adverse events remains a concern for gene therapy and we cannot assure that it will not occur in any of our future clinical trials. In addition, there is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material.

Adverse events in trials or studies conducted by us or other parties, even if not ultimately attributable to our product candidates, and resulting publicity, could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

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 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 a new drug application ("NDA") or Biologics License Application ("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 geopolitical actions, including the ongoing war between Russia and Ukraine, 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 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 rare epilepsies, seizure-related disorders, and rare neurological disorders. However, our business development activities and research activities may present attractive opportunities outside of rare 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. Since 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 or 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 rare 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 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. Since 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 actions, including the ongoing war between Russia and Ukraine, 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. Royalties will be 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.

Under the terms of the RLT Agreement, Takeda now has sole discretion over the conduct of the development and commercialization of soticlestat. 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 such agreement. We are dependent upon Takeda's progression of such development and the resulting payments to fund the regulatory development of our current and future drug candidates. If we are unable to find alternative sources of revenue, our inability

to receive royalty or milestone payments under the RLT Agreement would negatively impact our business and results of operations.

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, or even commercial launch 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 rare 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 rare 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 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, President Trump signed Executive Orders and other directives designed to delay the implementation of certain provisions of the PPACA or otherwise circumvent some of the requirements for health insurance mandated by the PPACA. Concurrently, Congress considered legislation to repeal or repeal and replace all or part of the PPACA. While Congress has not passed comprehensive repeal legislation, it has enacted laws that modify certain provisions of the PPACA such as removing penalties, effective January 1, 2019, for not complying with the PPACA's individual mandate to carry health insurance, delaying the implementation of certain PPACA-mandated fees, and increasing the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D. 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. For example, on August 16, 2022, President Biden signed the Inflation Reduction Act of 2022 (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 2031 unless additional Congressional action is taken. Under current legislation, the actual reduction in Medicare payments will vary from 1% in 2022 to up to 4% in the final fiscal year of this sequester. 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. In November 2019, CMS issued a final rule finalizing the changes to the Quality Payment Program. At this time, the full impact to overall physician reimbursement as a result of the introduction of the Quality Payment Program remains unclear.

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. In addition, the IRA, among other things, (i) directs the Secretary of HHS to negotiate the price of certain high-expenditure, single-source drugs and biologics covered under Medicare Part B and Medicare Part D, and subjects drug manufacturers to civil monetary penalties and a potential excise tax by offering a price that is not equal to or less than the negotiated “maximum fair price” under the law, and (ii) imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation. These provisions will take effect progressively starting in fiscal year 2023, although they may be subject to legal challenges. Further, 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. At the state level, legislatures have increasingly passed and implemented regulations designed to control pharmaceutical and biological product pricing, including 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, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved drug. For example, based on a recent executive order, the Biden administration expressed its intent to pursue certain policy initiatives to reduce drug prices. 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 (the “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 U.S. Patent and Trademark Office (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 March 31, 2023, we had 46 full-time employees. 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.

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

We are exposed to the risk that our employees, consultants, distributors, and collaborators 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 information, including intellectual property, proprietary business information, personal information and other confidential information. It is critical that we do so in a secure manner to maintain the confidentiality, integrity and availability of such sensitive information. 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 confidential information. In addition, many of those third parties in turn subcontract or outsource some of their responsibilities to 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 information stored on those systems, make such systems potentially vulnerable to unintentional or malicious, internal and external attacks on our technology environment. In addition, due to our hybrid work environment, we may be more vulnerable to cyberattacks. Potential vulnerabilities can be exploited from inadvertent or intentional actions of our employees, third-party vendors, business partners, or by malicious third parties. Attacks of this nature are increasing in their frequency, levels of persistence, sophistication and intensity, and are 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. In addition to the extraction of sensitive information, such attacks could include the deployment of harmful malware, ransomware, denial-of-service attacks, social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information. In addition, the prevalent use of mobile devices increases the risk of data security incidents.

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 information, 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.

There is no way of knowing with certainty whether we have experienced any data security incidents that have not been discovered. While we have no reason to believe this to be the case, attackers have become very sophisticated in the way they conceal access to systems, and many companies that have been attacked are not aware that they have been attacked. Any event that leads to unauthorized access, use or disclosure of personal information, including but not limited to personal information regarding our patients or employees, could disrupt our business, harm our reputation, compel us to comply with applicable federal and/or state breach notification laws and foreign law equivalents, subject us to time consuming, distracting and expensive litigation, regulatory investigation and oversight, mandatory corrective action, require us to verify the correctness of database contents, or otherwise subject us 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 information, which could include personally identifiable information, 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 successfully prevent service interruptions or security incidents.

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 are subject to laws and regulations covering data privacy and the protection of personal information including health information. 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 U.S., 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 General Data Protection Regulation, or GDPR, which applies to all EU member states as of May 25, 2018 and replaces the former EU Data Protection Directive. The regulation introduces 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 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 significant penalties against us, 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 (the “CCPA”) legislation that has been dubbed the first “GDPR-like” law in the United States. The CCPA gives California residents expanded rights to access 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. The CCPA may increase our compliance costs and potential liability.

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 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 shares 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 share 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.

Effective as of December 31, 2022, we ceased to be an “emerging growth company”, which will increase our costs and demands on management.

On December 31, 2022, we ceased to be an emerging growth company (“EGC”), as defined in the Jumpstart Our Business Startups Act of 2012. In addition, as a result of the market value of our common stock held by non-affiliates as of June 30, 2022, we also qualified as a smaller reporting company and an accelerated filer.

Due to our exit from EGC status and our qualification as a smaller reporting company that is also classified as an accelerated filer, we are subject to certain disclosure and compliance requirements that apply to other public companies that did not previously apply to us due to our status as an EGC. These requirements include, but are not limited to:

- the requirement that our independent registered public accounting firm attest to the effectiveness of our internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act of 2002 (“Section 404”); and
- the requirement that we hold a non-binding advisory vote on executive compensation, the frequency of such advisory vote on executive compensation, and obtain stockholder approval of any golden parachute payments not previously approved.

We expect that compliance with these additional requirements will increase our legal and financial compliance costs and may cause management and other personnel to divert attention from operational and other business matters to devote increased time to public company reporting requirements. In addition, if we are not able to comply with changing requirements in a timely manner, 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, which would require additional financial and management resources.

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 for 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.

While we were an EGC, our independent registered public accounting firm was not required to attest to the effectiveness of our internal controls over financial reporting pursuant to Section 404. This exemption no longer applied to us as of December 31, 2022. Accordingly, beginning with our Annual Report on Form 10-K for the year ending December 31, 2022, we are required to include and have included an attestation from our independent registered public accounting firm on the effectiveness of our internal control over financial reporting. Our compliance with Section 404 requires 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 and compile the system and process documentation necessary to perform the evaluation needed to 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 identify 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 material weaknesses or significant deficiencies in our internal control over financial reporting in the future. Any failure to maintain internal control over financial reporting could severely inhibit our ability to accurately report our financial condition, results of operations or cash flows. If we are unable to conclude that our internal control over financial reporting is effective, or if our independent registered public accounting firm determines we have a material weakness or significant deficiency 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 any 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. 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, including very recently in connection with the

ongoing COVID-19 pandemic, 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, you may lose all or part of your investment in our common stock since you might be unable to sell your shares at or above the price you paid for the shares. The market price for our common stock may be influenced by many factors, including:

- results of clinical trials of our current and any future drug candidates or those of our competitors;
- the success of competitive drugs or therapies;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to our current and any future drug candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional drug candidates;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- our inability to obtain or delays in obtaining adequate drug supply for any approved drug or inability to do so at acceptable prices;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; 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. For example, the COVID-19 pandemic resulted in widespread unemployment, economic slowdown and extreme volatility in the capital markets. Similarly, the Russia-Ukraine war has created extreme volatility in the global capital markets and is 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 2020, we filed a shelf registration statement on Form S-3 (Registration No. 333-250054) that allows us to sell up to an aggregate of \$250.0 million of our common stock, preferred stock, debt securities and/or warrants (the “S-3 Registration Statement”), 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 December 31, 2022, 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.

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

As of March 31, 2023, we had outstanding options to purchase an aggregate of 15,418,177 shares of our common stock at a weighted average exercise price of \$3.86 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 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 your investment and could negatively affect the market price of our common stock. In addition, you may experience further dilution if we issue common stock, or securities convertible into common stock, in the future. As a result of this dilution, you may receive significantly less than the full purchase price you 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 our shares of our common stock outstanding as of March 31, 2023, our executive officers, directors and stockholders who owned more than 5% of our outstanding common stock, in the aggregate, beneficially own shares representing approximately 49% 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 with Takeda.

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 do currently have research coverage offered by four industry or financial analysts, although two analysts have withdrawn research coverage in the last twelve months. We do not have any control over these analysts. If additional 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 your 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 your 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 you might otherwise receive a premium for your 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;
- 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, 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 you might otherwise receive a premium for your 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 Delaware law 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 Delaware General Corporation Law (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 6. Exhibits.

Exhibit Number	Description
3.1	<u>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).</u>
3.2	<u>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).</u>
3.3	<u>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).</u>
4.1	<u>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).</u>
4.2	<u>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).</u>
4.3	<u>Second Amended and Restated Investors' Rights Agreement, by and among the Company and certain of its stockholders, dated January 6, 2017 (incorporated herein by reference to Exhibit 4.2 to the Company's Registration Statement on Form S-1 (File No. 333-217245), filed with the Commission on April 10, 2017).</u>
31.1	<u>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.</u>
31.2	<u>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.</u>
32.1*	<u>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.</u>
101.INS	Inline XBRL Instance Document
101.SCH	Inline XBRL Taxonomy Extension Schema Document
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained within Exhibit 101)

* 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: May 5, 2023

By: /s/ Jeremy M. Levin

Jeremy M. Levin

Chief Executive Officer

(Principal Executive Officer)

Date: May 5, 2023

By: /s/ Jeffrey Rona

Jeffrey Rona

Chief Business and Financial Officer

(Principal Financial and Accounting Officer)

**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: May 5, 2023

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

**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: May 5, 2023

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

**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 March 31, 2023, 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 Exchange Act; 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: May 5, 2023

/s/ Jeremy M. Levin

Jeremy M. Levin
Chief Executive Officer
(Principal Executive Officer)

/s/ Jeffrey Rona

Jeffrey Rona
Chief Business and Financial Officer
(Principal Financial and Accounting Officer)