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 June 30, 2025

or

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

For the transition period from _____ to

Commission File Number: 001-36593

Soleno Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**77-0523891**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**

100 Marine Parkway, Suite 400
Redwood City, California
(Address of principal executive offices)

94065
(Zip Code)

(650) 213-8444
(Registrant's telephone number, including area code)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.001 par value	SLNO	NASDAQ

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

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

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

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

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

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

As of August 1, 2025, there were 53,145,009 shares of the registrant's Common Stock, par value $0.001 per share, outstanding.

SOLENO THERAPEUTICS, INC.

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PART I—FINANCIAL INFORMATION

Item 1. Financial Statements

Soleno Therapeutics, Inc.
Condensed Consolidated Balance Sheets
(in thousands, except share and per share data)

	June 30, 2025	December 31, 2024
Assets	**(unaudited)**	
Current assets		
Cash and cash equivalents	$ 76,497	$ 87,928
Marketable securities	210,344	203,509
Accounts receivable, net	24,624	-
Inventory	2,355	-
Prepaid expenses and other current assets	2,969	2,452
Total current assets	316,789	293,889
Long-term assets		
Property and equipment, net	166	186
Operating lease right-of-use assets	2,434	2,798
Intangible assets, net	5,832	6,805
Long-term marketable securities	7,002	27,211
Other long-term assets	83	83
Total assets	$ 332,306	$ 330,972
Liabilities and stockholders' equity		
Current liabilities		
Accounts payable	$ 6,216	$ 8,882
Accrued compensation	6,267	4,776
Accrued clinical trial site costs	1,863	1,826
Operating lease liabilities	676	526
Accrued interest payable	409	-
Other current liabilities	5,512	2,737
Total current liabilities	20,943	18,747
Long-term liabilities		
Contingent liability for Essentialis purchase price	18,859	14,791
Long-term debt, net	49,845	49,828
Long-term lease liabilities	2,248	2,472
Other long-term liabilities	271	21
Total liabilities	92,166	85,859
Commitments and contingencies (Note 6)		
Stockholders' equity		
Preferred stock, $0.001 par value; 10,000,000 shares authorized, no shares issued and outstanding	-	-
Common stock, $0.001 par value, 100,000,000 shares authorized, 50,437,066 and 45,703,811 shares issued and outstanding at June 30, 2025 and December 31, 2024, respectively	50	46
Additional paid-in-capital	740,746	696,966
Accumulated other comprehensive income	85	361
Accumulated deficit	(500,741)	(452,260)
Total stockholders' equity	240,140	245,113
Total liabilities and stockholders' equity	$ 332,306	$ 330,972

See accompanying notes to condensed consolidated financial statements

Soleno Therapeutics, Inc.
Condensed Consolidated Statements of Operations and Comprehensive Loss
(unaudited)
(in thousands, except share and per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Revenue				
Product revenue, net	$ 32,657	$ —	$ 32,657	$ —
Total revenue	32,657	—	32,657	—
Operating expenses				
Cost of goods sold	696	—	696	—
Research and development	9,147	12,342	22,664	26,944
Selling, general and administrative	28,238	10,889	57,497	19,361
Change in fair value of contingent consideration	1,101	1,637	4,068	2,038
Total operating expenses	39,182	24,868	84,925	48,343
Operating loss	(6,525)	(24,868)	(52,268)	(48,343)
Other income (expense), net				
Interest income, net	3,193	3,014	6,524	5,091
Interest expense	(1,376)	-	(2,737)	-
Total other income (expense), net	1,817	3,014	3,787	5,091
Net loss	$ (4,708)	$ (21,854)	$ (48,481)	$ (43,252)
Other comprehensive income (loss)				
Net unrealized loss on marketable securities	(153)	(46)	(292)	(151)
Foreign currency translation adjustment	12	(1)	16	(2)
Total comprehensive loss	$ (4,849)	$ (21,901)	$ (48,757)	$ (43,405)
Net loss per common share, basic and diluted	$ (0.09)	$ (0.57)	$ (1.00)	$ (1.16)
Weighted-average common shares outstanding used to calculate basic and diluted net loss per common share	50,483,281	38,631,565	48,342,928	37,419,968

See accompanying notes to condensed consolidated financial statements

Soleno Therapeutics, Inc.
Condensed Consolidated Statements of Stockholders' Equity
For the Three and Six Months Ended June 30, 2025 and 2024
(unaudited)
(in thousands, except share data)

	Common Stock		Additional Paid-In	Accumulated Other Comprehensive Income	Accumulated	Total Stockholders'
	Shares	Amount	Capital	(Loss)	Deficit	Equity
Balances at January 1, 2025	45,703,811	$ 46	$ 696,966	$ 361	$ (452,260)	$ 245,113
Stock-based compensation	-	-	14,679	-	-	14,679
Issuance of common stock in connection with exercise of stock options and vesting of restricted stock units	943,980	1	13,365	-	-	13,366
Exercise of common stock warrants	1,879,678	2	3,009	-	-	3,011
Unrealized loss on marketable securities	-	-	-	(139)	-	(139)
Foreign currency translation adjustment	-	-	-	4	-	4
Net loss	-	-	-	-	(43,773)	(43,773)
Balances at March 31, 2025	48,527,469	49	728,019	226	(496,033)	232,261
Stock-based compensation	-	-	9,958	-	-	9,958
Issuance of common stock in connection with exercise of stock options and vesting of restricted stock units	1,039,402	1	626	-	-	627
Tax withholding payments for net share-settled equity awards	(433)	-	(33)	-	-	(33)
Exercise of common stock warrants	870,628	-	2,176	-	-	2,176
Unrealized loss on marketable securities	-	-	-	(153)	-	(153)
Foreign currency translation adjustment	-	-	-	12	-	12
Net loss	-	-	-	-	(4,708)	(4,708)
Balances at June 30, 2025	50,437,066	$ 50	$ 740,746	$ 85	$ (500,741)	$ 240,140

	Common Stock		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Loss	Deficit	Equity
Balances at January 1, 2024	31,678,159	$ 32	$ 433,885	$ -	$ (276,410)	$ 157,507
Stock-based compensation	-	-	6,445	-	-	6,445
Issuance of common stock in connection with exercise of stock options and vesting of restricted stock units	14,034	-	15	-	-	15
Exercise of common stock warrants and pre-funded common stock warrants	1,644,886	1	922	-	-	923
Unrealized loss on marketable securities	-	-	-	(105)	-	(105)
Foreign currency translation adjustment	-	-	-	(1)	-	(1)
Net loss	-	-	-	-	(21,398)	(21,398)
Balances at March 31, 2024	33,337,079	33	441,267	(106)	(297,808)	143,386
Stock-based compensation	-	-	7,160	-	-	7,160
Issuance of restricted stock units under equity incentive plans	75,750	-	-	-	-	-
Sale of common stock, net of issuance costs of $9,746	3,450,000	4	148,951	-	-	148,955
Exercise of common stock warrants and pre-funded common stock warrants	1,435,319	1	2,619	-	-	2,620
Exercise of stock options	88,631	-	537	-	-	537
Unrealized loss on marketable securities	-	-	-	(46)	-	(46)
Foreign currency translation adjustment	-	-	-	(1)	-	(1)
Net loss	-	-	-	-	(21,854)	(21,854)
Balances at June 30, 2024	38,386,779	$ 38	$ 600,534	$ (153)	$ (319,662)	$ 280,757

See accompanying notes to condensed consolidated financial statements

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

	Six Months Ended June 30,	
	2025	**2024**
Cash flows from operating activities:		
Net loss	$ (48,481)	$ (43,252)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	1,006	984
Accretion of premium/discount on marketable securities	(1,954)	(1,586)
Non-cash lease expense	364	139
Amortization of debt issuance costs	17	-
Stock-based compensation expense	24,377	13,605
Change in fair value of contingent consideration	4,068	2,038
Other non-cash reconciling items	16	(2)
Change in operating assets and liabilities:		
Accounts receivable	(24,624)	-
Inventory	(2,095)	-
Prepaid expenses, other current assets and other assets	(339)	380
Accounts payable	(2,603)	567
Accrued compensation	1,491	(986)
Accrued clinical trial site costs	37	(1,530)
Operating lease liabilities	(74)	(107)
Other liabilities	3,434	(429)
Net cash used in operating activities	(45,360)	(30,179)
Cash flows from investing activities:		
Purchases of property and equipment	(14)	(19)
Purchases of marketable securities	(104,764)	(261,146)
Maturities of marketable securities	119,800	25,000
Net cash provided by (used in) investing activities	15,022	(236,165)
Cash flows from financing activities:		
Payment of debt issuance costs	(62)	-
Payment of deferred financing costs	(178)	-
Proceeds from the sale of common stock, net of issuance costs	-	148,955
Proceeds from exercise of common stock warrants	5,187	-
Proceeds from exercise of common stock and pre-funded stock warrants	-	3,543
Proceeds received prior to and for the issuance of common stock and pre-funded warrants	-	637
Proceeds from exercise of stock options	13,993	552
Tax withholding payments for net share-settled equity awards	(33)	-
Net cash provided by financing activities	18,907	153,687
Net decrease in cash and cash equivalents	(11,431)	(112,657)
Cash and cash equivalents, beginning of period	87,928	169,681
Cash and cash equivalents, end of period	$ 76,497	$ 57,024
Supplemental disclosure of non-cash operating and financing information		
Cash paid for interest	$ 2,060	$ -

See accompanying notes to condensed consolidated financial statements.

Note 1. Overview

Soleno Therapeutics, Inc. (the Company or Soleno) is a biopharmaceutical company developing novel therapeutics for the treatment of rare diseases. On March 26, 2025, the Company announced that its lead product candidate, VYKAT™ XR (diazoxide choline) extended-release tablets, formerly known as DCCR, had been approved by the U.S. Food and Drug Administration (FDA). VYKAT XR is indicated to treat hyperphagia in adults and pediatric patients four years of age and older with Prader-Willi syndrome (PWS). On April 14, 2025, the Company announced that prescriptions of VYKAT XR had been delivered to the first individuals living with PWS who had been prescribed the medication and began recognizing revenue from the sales of VYKAT XR during the three months ended June 30, 2025. The Company is incorporated in the State of Delaware and is headquartered in Redwood City, California.

Note 2. Liquidity

The Company used $45.4 million of cash in its operating activities, had a net loss of $48.5 million during the six months ended June 30, 2025 and had an accumulated deficit of $500.7 million at June 30, 2025 resulting from having incurred losses since its inception. The Company had $76.5 million of cash and cash equivalents and $217.3 million of marketable securities on June 30, 2025. With FDA approval of VYKAT XR and first prescriptions being delivered in April 2025, the Company recognized revenue during the three months ended June 30, 2025.

As of June 30, 2025, the Company had $50.0 million of debt outstanding under the loan and security agreement with Oxford Financing LLC and its affiliates (collectively, Oxford) entered into in December 2024. Under the terms of the loan agreement with Oxford, following FDA approval of VYKAT XR, an additional $50 million is available through September 30, 2025 and $25 million will become available during the period October 1, 2025 to September 30, 2026. An additional tranche of $25 million may become available upon achievement of certain commercial milestones. A final $50 million may be made available upon the Company's mutual consent with Oxford. The loan carries an interest-only period of 48 months and a total term of 60 months; provided that if specific milestones are achieved prior to September 30, 2026, the interest-only period and maturity date will be extended by 12 months. The term loans accrue interest at a floating rate equal to, subject to certain conditions, (a) 1-month term SOFR plus (b) 5.50%.

Prior to entering into the Oxford loan and security agreement, the Company had historically financed its operations principally through issuance of equity securities. On May 9, 2024, the Company closed an underwritten public offering of 3,450,000 shares of its common stock at a public offering price of $46.00 per share, which included the exercise in full by the underwriters of their option to purchase additional shares. The gross proceeds of the public offering were $158.7 million, before deducting the underwriter discount and other offering expenses, totaling approximately $9.7 million.

On July 19, 2024, the Company entered into an Open Market Sale Agreement℠ (the Sales Agreement) with Jefferies LLC, as sales agent (Jefferies), pursuant to which the Company may offer and sell, from time to time, through Jefferies shares of its common stock having an aggregate offering price of up to $150 million.

In July 2025, the Company closed an underwritten public offering of 2,705,882 shares of its common stock at an offering price of $85.00 per share, which included the exercise in full by the underwriters of their option to purchase additional shares of its common stock. The gross proceeds of the public offering were $230.0 million, before deducting the underwriter discount and other offering expenses, totaling approximately $14.3 million.

The Company expects that its current cash, cash equivalents and marketable securities balances will be sufficient to enable the Company to meet its obligations for at least the next twelve months from the date of this filing.

Note 3. Basis of Presentation and Summary of Significant Accounting Policies

Significant Accounting Policies

There have been no material changes to the significant accounting policies during the three and six months ended June 30, 2025 as compared to the significant accounting policies described in Note 3 of the “Notes to Consolidated Financial Statements” in the Company’s Annual Report on Form 10-K for the year ended December 31, 2024, except as noted below.

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements of the Company have been prepared in accordance with U.S. generally accepted accounting principles (GAAP) for interim financial reporting and as required by Regulation S-X, Rule 10-01. Accordingly, they do not include all of the information and footnotes required by GAAP for complete financial statements. In the opinion of management, all adjustments (including those which are normal and recurring) considered necessary for a fair presentation of the interim financial information have been included. When preparing financial statements in conformity with GAAP, the Company must make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues, expenses and related disclosures at the date of the financial statements. Actual results could differ from those estimates. Additionally, operating results for the three and six months ended June 30, 2025 are not necessarily indicative of the results that may be expected for any other interim period or for the fiscal year ending December 31, 2025. For further information, refer to the financial statements and footnotes included in the Company's annual financial statements for the fiscal year ended December 31, 2024, which are included in the Company's Annual Report on Form 10-K filed with the Securities Exchange Commission (SEC) on February 28, 2025.

Use of Estimates

The preparation of condensed consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities, and reported amounts of expenses in the financial statements and accompanying notes. Actual results could differ from those estimates. Key estimates included in the financial statements include the valuation of deferred income tax assets, the valuation of financial instruments, stock-based compensation, accrued costs for services rendered in connection with third-party contractor clinical trial activities, reserves for product sales, and the valuation of contingent liabilities for the purchase price of assets obtained through acquisition.

Accounts Receivable, net

Accounts receivable, net consists of amounts due from customers, net of customer allowances for cash discounts and any estimated expected credit losses. The Company's measurement of expected credit losses is based on relevant information about past events, including historical experience, current conditions, and reasonable and supportable forecasts that affect the collectability of the reported amount. To date, the Company has not experienced any credit losses. The Company's contract with its customer has customary payment terms that require payment within 45 days. The Company analyzes amounts that are past due for collectability, and periodically evaluates the creditworthiness of its customer. Based on its assessment, as of June 30, 2025, the Company determined that an allowance for credit loss was not required.

Concentration of Major Customers and Off-Balance Sheet Risk

The Company contracts with one customer, its specialty pharmacy, to market and distribute VYKAT XR to patients in the United States. As of June 30, 2025, its accounts receivable, net are solely from sales of VYKAT XR, and are from this sole customer.

The Company's dependency on one customer exposes it to several risks, including the potential for disruptions in its distribution network, changes in this customer's business strategies, or financial difficulties faced by this customer. Any significant disruption or change in the Company's relationship with this customer could materially and adversely affect its ability to effectively reach other potential end users and maintain its market position.

While the Company believes its relationship with this customer is strong and mutually beneficial, there can be no assurance that it will be able to maintain this relationship or that it will be able to replace this specialty pharmacy with alternative specialty pharmacies, if necessary.

The Company relies on sole source suppliers and third-party manufacturers to supply raw materials and manufacture its product. The inability of these suppliers or manufacturers to fulfill supply requirements of the Company could materially impact future operating results. A change in the relationship with these suppliers or manufacturers, or an adverse change in their business, could materially impact future operating results.

Revenue Recognition

The Company recognizes revenue in accordance with ASC Topic 606, *Revenue from Contracts with Customers*, or ASC 606. Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services.

Product Revenue, net

The Company sells its product to one specialty pharmacy. The product is distributed through a third-party logistics distribution agent (3PL) that does not take title to the product. In the Company's agreement with the 3PL, the Company acts as principal because it retains control of the product. Once the product is delivered to the Company's specialty pharmacy, the specialty pharmacy takes title

to the product. The specialty pharmacy then distributes the product to patients. The Company offers returns of product sold to the customer on a limited basis; however, no material returns have been recognized to date.

Revenue from product sales is recognized when the customer obtains control of the Company's product, which occurs at the point in time that there is a transfer of title to the customer. The Company has no other performance obligations besides the sale of product. The Company classifies payments to its customers or other parties in the distribution channel for services that are distinct and priced at fair value as selling, general and administrative expenses in its condensed consolidated statements of operations and comprehensive loss. Otherwise, payments to a customer or other parties in the distribution channel that do not meet those criteria are classified as a reduction of revenue, as discussed further below. Because the Company's payment terms are 45 days or less, the Company concluded there is not a significant financing component. The Company expenses incremental costs of obtaining a contract as and when incurred since the expected amortization period of the asset that the Company would have recognized is one year or less.

Reserves for Variable Consideration

Revenues from product sales are recorded at the net sales price, or the transaction price, which includes estimates of variable consideration for which reserves are established and which result from rebates, discounts, returns, and co-pay assistance that are offered within the contract between the Company and its customer relating to the sale of VYKAT XR. These reserves are based on the amounts earned or to be claimed on the related sales and are classified as reductions of accounts receivable (if the amount is payable to the customer) or a current liability (if the amount is payable to a party other than the customer). Where appropriate, these estimates take into consideration a range of possible outcomes that are weighted for relevant factors such as the Company's historical experience, current contractual and statutory requirements, specific known market events and trends, industry data and forecasted customer buying and payment patterns. Overall, these reserves reflect the Company's best estimates of the amount of consideration to which it is entitled based on the terms of the contract. The amount of variable consideration that is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is considered probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period. Actual amounts of consideration ultimately received may differ from the Company's estimates. If actual results in the future vary from the Company's estimates, it will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known.

The following are the components of variable consideration related to product revenue:

Government rebates: The Company is subject to discount obligations under several government programs, including Medicaid programs, Medicare and TRICARE in the United States. The Company estimates these rebates based upon a range of possible outcomes that are weighted for the estimated payer mix. These reserves are recorded in the same period that the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a liability that is included in accrued expenses and other current liabilities on the Company's condensed consolidated balance sheets. On a quarterly basis, the Company updates its estimates and records any adjustments in the period that it identifies the adjustments.

Trade discounts and allowances: The Company provides discounts on VYKAT XR sales to its customer for prompt payment. This discount is recorded as a reduction of revenue in the period the related product revenue is recognized. In addition, the Company receives and pays for various distribution services from its customer in the distribution channel.

Product returns: The Company's customer has limited return rights related to unexpected instances in which the product is found to be damaged or defective. The Company estimates the amount of product sales that may be returned and records the estimate as a reduction of revenue and a refund liability in the period in which the related product revenue is recognized. Based on the distribution model for VYKAT XR, the Company believes there will be minimal returns as such returns have not been material to date.

Other incentives: Other incentives include co-payment assistance the Company provides to patients with commercial insurance that have coverage and reside in states that allow co-payment assistance. The calculation of the accrual for co-pay assistance is based on an estimate of claims and the cost per claim that the Company expects to receive associated with product that has been recognized as revenue. The estimate is recorded as a reduction of revenue in the same period the related revenue is recognized.

Provisions for trade discounts and allowances are recorded as reductions to accounts receivable, and returns, government rebates, and other incentives are recorded as a component of accrued expenses.

The table below summarizes balances and activity in each of the product revenue allowance and reserve categories as follows (in thousands):

	Rebates	Discounts and Chargebacks	Copay Assistance and Returns	Total
Balance as of December 31, 2024	$ -	$ -	$ -	$ -
Provisions	2,987	919	396	4,302
Credits/payments	-	(288)	(215)	(503)
Balance as of June 30, 2025	$ 2,987	$ 631	$ 181	$ 3,799

Inventory

The Company capitalizes inventory costs associated with products when future economic benefit is expected to be realized. These costs consist of raw materials, manufacturing-related costs, personnel costs including stock-based compensation, facility costs, and other indirect overhead costs. Prior to receiving FDA approval for VYKAT XR in March 2025, the Company expensed costs related to inventory for clinical and pre-commercial purposes directly to research and development expense. Following the FDA's approval of VYKAT XR, the Company began capitalizing inventory related to commercialized products held for sale, in-process of production for sale, and raw materials to be used in the manufacturing of inventory.

The Company values inventory at the lower of cost or estimated net realizable value. The Company determines the cost of inventory, which includes amounts related to materials and manufacturing overhead, on a first-in, first-out basis. Raw materials and work in process includes all inventory costs prior to packaging and labeling, including raw materials, active pharmaceutical ingredient, and drug product. Finished goods include packaged and labeled products. Raw materials and work in process that may be used for either research and development or commercial sale are classified as inventory until the material is consumed or otherwise allocated for research and development. If the material is intended to be used for research and development, it is expensed as research and development once that determination is made. Based on its assessment, as of June 30, 2025, the Company determined that an allowance for excess and obsolete inventory and lower of cost or estimated net realizable value adjustments was not required.

Cost of Goods Sold

Cost of goods sold consists of manufacturing costs, transportation and freight, amortization of capitalized intangibles, royalty payments and indirect overhead costs associated with the manufacturing and distribution of VYKAT XR. Cost of goods sold may also include periodic costs related to certain manufacturing services and inventory adjustment charges. Finally, cost of goods sold may also include costs related to excess or obsolete inventory adjustment charges, abnormal costs, unabsorbed manufacturing and overhead costs, and manufacturing variances.

Recently Adopted Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board (FASB) or other standard setting bodies that are adopted by the Company as of the specified effective date.

In December 2023, the FASB issued ASU 2023-09, *Improvements to Income Tax Disclosures,* which amends the guidance in *ASC 740, Income Taxes*. This ASU is intended to improve the transparency of income tax disclosures by requiring (1) consistent categories and greater disaggregation of information in the rate reconciliation and (2) income taxes paid disaggregated by jurisdiction. It also includes certain other amendments to improve the effectiveness of income tax disclosures. This ASU is effective for fiscal years beginning after December 15, 2024. Adoption is permitted either prospectively or retrospectively. The Company is evaluating the effect that ASU 2023-09 will have on its annual financial statements and disclosures.

In November 2024, the FASB issued ASU 2024-03, *Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expense,* which requires the disclosure of additional information related to certain costs and expenses, including amounts of inventory purchases, employee compensation, and depreciation and amortization included in each income statement line item. The guidance also requires disclosure of the total amount of selling expenses and the Company's definition of selling expenses. The guidance is effective for the Company for fiscal years beginning after December 15, 2026, and for interim periods within fiscal years beginning after December 15, 2027. The Company is evaluating the effect that ASU 2024-03 will have on its financial statements and disclosures.

Other accounting standards that have been issued or proposed by FASB or other standards-setting bodies that do not require adoption until a future date are not currently expected to have a material impact on the Company's financial statements upon adoption.

Note 4. Fair Value of Financial Instruments

The carrying value of the Company's cash, cash equivalents and accounts payable, approximate fair value due to the short-term nature of these items.

Fair value is defined as the exchange price that would be received for an asset or an exit price paid to transfer a liability in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs.

The fair value hierarchy defines a three-level valuation hierarchy for disclosure of fair value measurements as follows:

- Level I — Unadjusted quoted prices in active markets for identical assets or liabilities;
- Level II — Inputs other than quoted prices included within Level I that are observable, unadjusted quoted prices in markets that are not active, or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the related assets or liabilities; and
- Level III — Unobservable inputs that are supported by little or no market activity for the related assets or liabilities.

The categorization of a financial instrument within the valuation hierarchy is based upon the lowest level of input that is significant to the fair value measurement.

The fair value of marketable securities, which are Level 2 financial instruments, is based upon market prices quoted on the last day of the fiscal period or other observable market inputs. The Company obtains pricing information from its investment manager and generally determines the fair value of investment securities using standard observable inputs, including reported trades, broker/dealer quotes, bids and/or offers. Marketable securities, all of which are classified as available-for-sale securities, consisted of the following at June 30, 2025 (in thousands):

	June 30, 2025			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Estimated Fair Value
U.S. Treasury securities	$ 129,691	$ 76	$ (35)	$ 129,732
Corporate debt securities and commercial paper	87,585	37	(8)	87,614
Total	$ 217,276	$ 113	$ (43)	$ 217,346

The following table sets forth the Company's financial instruments that were measured at fair value on a recurring basis by level within the fair value hierarchy (in thousands):

	Fair Value Measurements at June 30, 2025			
	Total	Level 1	Level 2	Level 3
Assets				
Cash equivalents:				
Money market funds	$ 69,792	$ 69,792	$ —	$ —
Total cash equivalents	$ 69,792	$ 69,792	$ —	$ —
Marketable securities:				
U.S. Treasury securities	$ 129,732	$ —	$ 129,732	$ —
Corporate debt securities and commercial paper	87,614	—	87,614	—
Total marketable securities	$ 217,346	$ —	$ 217,346	$ —
Total assets	$ 287,138	$ 69,792	$ 217,346	$ —
Liabilities				
Essentialis purchase price contingency liability	$ 18,859	$ —	$ —	$ 18,859
Total liabilities	$ 18,859	$ —	$ —	$ 18,859

	Fair Value Measurements at December 31, 2024			
	Total	Level 1	Level 2	Level 3
Assets				
Cash equivalents:				
Money market funds	$ 59,885	$ 59,885	$ —	$ —
Total cash equivalents	$ 59,885	$ 59,885	$ —	$ —
Marketable securities:				
U.S. treasury securities	$ 198,065	$ —	$ 198,065	$ —
Corporate debt securities and commercial paper	32,655	—	32,655	—
Total marketable securities	$ 230,720	$ —	$ 230,720	$ —
Total assets	$ 290,605	$ 59,885	$ 230,720	$ —
Liabilities				
Essentialis purchase price contingency liability	$ 14,791	$ —	$ —	$ 14,791
Total liabilities	$ 14,791	$ —	$ —	$ 14,791

Based on the terms of the Company's completed merger with Essentialis on March 7, 2017, the Company is obligated to make cash earnout payments of up to a maximum of $21.2 million to the former Essentialis stockholders. The fair value of the Essentialis purchase price contingent liability has historically been estimated using scenario-based methods based upon the Company's analysis of the likelihood of obtaining specified approvals from the FDA as well as achieving two commercial sales milestones of $100 million and $200 million in cumulative revenue. The Level 3 estimates are based, in part, on subjective assumptions. As of June 30, 2025, following the receipt of FDA approval for VYKAT XR, the Company no longer considers FDA approval as a variable and determined a 100% probability of achieving the remaining two milestones. Prior to receiving FDA approval, management relied on published research relating to clinical development success rates to determine the likelihood of FDA approval occurring. Based on this assessment, an 88% probability of achieving all three milestones was determined to be reasonable as of December 31, 2024. During the periods presented, other than as discussed above regarding the receipt of FDA approval for VYKAT XR, the Company has not changed the manner in which it values its Essentialis purchase price contingent liability.

The Company recognizes transfers between levels of the fair value hierarchy as of the end of the reporting period. There were no transfers between levels within the hierarchy during the periods presented.

The following table sets forth a summary of the changes in the fair value of the Company's Level 3 liabilities for the six months ended June 30, 2025 and 2024 (in thousands):

	Purchase Price Contingent Liability
Balance at January 1, 2025	$ 14,791
Change in value of contingent liability	4,068
Balance at June 30, 2025	$ 18,859

	Purchase Price Contingent Liability
Balance at January 1, 2024	$ 11,549
Change in value of contingent liability	2,038
Balance at June 30, 2024	$ 13,587

Note 5. Inventory

Inventory consisted of the following (in thousands):

	June 30, 2025	December 31, 2024
Raw materials	$ 1,143	$ -
Work in process	950	-
Finished goods	262	-
Total	$ 2,355	$ -

Note 6. Commitments and Contingencies

Facility Leases

On June 13, 2024, the Company entered into a new office lease in Redwood City, California for office space for its headquarters facility. The lease provides office space of approximately 18,026 square feet and for base monthly rent payments beginning at $57,400 that increase annually by approximately 3.0% over the term of five years from the date of occupancy. In addition to base rent, the Company has agreed to reimburse the landlord for certain operating expenses under the terms of the lease. The lease commencement date was September 1, 2024 when the premises became available for occupancy. The Company's operating lease for its predecessor headquarters facility office space in Redwood City, California began on June 1, 2023 and expired in May 2025.

The Company's operating lease right-of-use (ROU) assets, current operating lease liabilities and long-term operating lease liabilities each appear as a separate line within the Company's condensed consolidated balance sheets. In September 2024, the Company recorded an increase to its ROU assets by $2.8 million and an increase to its lease liability of $2.8 million as a result of the June 2024 office lease. As of June 30, 2025 and December 31, 2024, the Company's short-term liabilities were equal to $0.7 million and $0.5 million, respectively, and the long-term operating lease liabilities were equal to $2.2 million and $2.5 million, respectively.

The weighted average discount rate related to the Company's lease liabilities was 8.5% as of June 30, 2025 over a remaining term of 4.2 years, and 8.5% as of December 31, 2024 over a remaining term of 4.7 years. The discount rate was determined based on estimates of the Company's incremental borrowing rate, as the discount rate implicit in the lease cannot be readily determined.

The components of lease expense during the three and six months ended June 30, 2025 and 2024 were as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Operating lease cost:				
Operating lease cost	$ 232	$ 77	$ 490	$ 154
Variable lease cost	2	4	6	8
Short-term lease cost	12	59	26	93
Total operating lease cost	$ 246	$ 140	$ 522	$ 255

Supplemental cash flow information related to leases was as follows (in thousands):

Cash paid for amounts included in the measurement of lease liabilities:

	Six Months Ended June 30,	
	2025	2024
Operating cash flows from operating leases	$ 200	$ 121

The following is a schedule by year of future maturities of the Company's operating lease liabilities as of June 30, 2025 (in thousands):

2025 (remainder of the year)	$	294
2026		751
2027		861
2028		942
2029		665
Total lease payments		3,513
Less interest		(589)
Total	$	2,924

Other Commitments

The Company enters into agreements in the normal course of business, including with contract research organizations for clinical trials, contract manufacturing organizations for certain manufacturing services and supplies, and vendors for preclinical studies as well as other services and products for operating purposes, which are generally cancelable upon written notice. As of June 30, 2025, the Company's non-cancelable other commitments were $15.7 million in the aggregate.

Contingencies

In the normal course of business, the Company enters into contracts and agreements that contain a variety of representations and warranties and provide for general indemnifications. The Company's exposure under these agreements is unknown because it involves claims that may be made against the Company in the future but have not yet been made. The Company accrues a liability for such matters when it is probable that future expenditures will be made, and such expenditures can be reasonably estimated.

Note 7. Long-term Debt

On December 17, 2024, the Company entered into a loan and security agreement for up to $200 million with Oxford. The loan is collateralized by substantially all of the Company's assets, including its intellectual property, subject to certain limitations.

As of June 30, 2025, the Company had $50.0 million outstanding under the Oxford loan agreement. Under the terms of the loan agreement with Oxford, following FDA approval of VYKAT XR, an additional $50 million is available through September 30, 2025 and $25 million will become available during the period October 1, 2025 to September 30, 2026. An additional tranche of $25 million may become available upon achievement of certain commercial milestones. A final $50 million may be made available upon the mutual consent of the Company and Oxford. The loan carries an interest-only period of 48 months and a total term of 60 months; provided that if specific milestones are achieved prior to September 30, 2026, the interest-only period and maturity date will be extended by 12 months. The term loans accrue interest, payable monthly, at a floating rate equal to, subject to certain conditions, (a) 1-month term SOFR plus (b) 5.50%. The principal portion of the loan is due in eleven equal monthly installments beginning February 1, 2029, through December 1, 2029. However, if a specified milestone is achieved on or after December 17, 2025, then the term loan will begin to amortize in equal monthly installments beginning on February 1, 2030, and the maturity date will be extended to December 1, 2030. At maturity, the final principal payment will include a fee of 5% of the total principal borrowed. The $2.5 million final interest payment related to the $50 million borrowed as of June 30, 2025 is accrued over the term of loan as long-term accrued interest payable. However, if a specified milestone is achieved on or after December 17, 2025, then the term loan will begin to amortize in equal monthly installments beginning on February 1, 2030, the maturity date will be extended to December 1, 2030, and the final payment will include a fee of 6.5% of the total principal borrowed. As of June 30, 2025, $271 thousand was accrued as part of other long-term liabilities on the condensed consolidated balance sheet. Loan issuance costs of $174 thousand were recorded as a reduction of the principal loan balance on the condensed consolidated balance sheet and are amortized as interest expense over the term of the loan.

The outstanding long-term debt consisted of the following (in thousands):

		June 30, 2025
Long-term debt	$	50,000
Unamortized debt issuance costs		(155)
Total long-term debt, net	$	49,845

The following table provides the components of interest expense related to the long-term debt (in thousands):

	Three Months Ended June 30, 2025	Six Months Ended June 30, 2025
Interest expense based on contractual loan rate	$ 1,242	$ 2,470
Amortization of debt issuance costs and accretion of final interest payment	134	267
Total interest expense	$ 1,376	$ 2,737

The loan and security agreement provides for both affirmative and negative covenants, including covenants limiting the ability of the Company and their subsidiaries to, among other things, dispose of assets, incur debt, grant liens, pay dividends and distributions on their capital stock, make investments and acquisitions, and enter into transactions with affiliates, in each case subject to customary exceptions for a loan facility of this size and type. In addition, the loan and security agreement contains a minimum revenue covenant commencing on the earlier of the date that more than $50 million principal amount of term loans have been funded under the loan and security agreement and June 30, 2026; provided that such minimum revenue covenant shall not be tested during periods when the Company's market capitalization or unrestricted cash meet certain minimum thresholds. The occurrence of an event of default could result in the acceleration of the Company's obligations under the loan and security agreement, the termination of the lenders' commitments, a 5.0% increase in the applicable rate of interest and the exercise by the lender of other rights and remedies provided for under the loan and security agreement. The Company was in compliance with all applicable debt covenants as of June 30, 2025.

Note 8. Stockholders' Equity

Preferred Stock

The Company is authorized to issue 10,000,000 shares of Preferred Stock.

May 2024 Public Offering of Common Stock

On May 9, 2024, the Company closed an underwritten public offering of 3,450,000 shares of its common stock at a public offering price of $46.00 per share, which included the exercise in full by the underwriters of their option to purchase additional shares. The gross proceeds of the public offering were $158.7 million, before deducting the underwriter discount and other offering expenses, totaling approximately $9.7 million.

October 2023 Public Offering of Common Stock and Concurrent Private Placement of Common Stock and Pre-Funded Warrants

On October 2, 2023, the Company closed an underwritten public offering of 3,450,000 shares of its common stock at a public offering of $20.00 per share, which included the exercise in full by the underwriters of their option to purchase additional shares. The gross proceeds of the public offering were $69.0 million, before deducting the underwriting discount and other offering expenses. Concurrently, the Company also completed the closing of approximately $60.0 million for 1,825,000 shares of its common stock and 1,175,000 pre-funded warrants in a private offering pursuant to a securities purchase agreement with certain investors, including entities affiliated with existing stockholders, at a price per share of common stock equal to the public offering price of $20.00 and a price per pre-funded warrant of $19.99. In aggregate, the Company received $129.0 million of gross proceeds less offering costs of $8.2 million. The Company is not required under any circumstance to settle any of the pre-funded warrants for cash, and therefore classified the pre-funded warrants as permanent equity.

Securities Purchase Agreement

On December 16, 2022, the Company entered into a Securities Purchase Agreement for a private placement (Private Placement) with certain entities and members of management (collectively, Purchasers). Pursuant to the Securities Purchase Agreement, the Company agreed to sell to the Purchasers warrants to purchase up to an aggregate of 22,598,870 shares of the Company's common stock, at a purchase price of $0.4425 per warrant. The closing of the Private Placement occurred on May 8, 2023 (the Issue Date), following the satisfaction of certain closing conditions, including the completion of enrollment in the randomized withdrawal period of Study C602. The Company received gross proceeds of $10.0 million for the sale and issuance of warrants to purchase common stock.

The warrants were separated into two tranches with 8,598,870 Tranche A Warrants with an exercise price of $1.75 per share and aggregate proceeds of up to approximately $15.0 million, and 14,000,000 Tranche B Warrants with an exercise price of $2.50 per share and aggregate proceeds of up to $35.0 million. The Tranche A warrants were immediately exercisable and were required to be exercised within 30 days of announcement of positive top-line data from the randomized withdrawal period of Study C602. On September 26, 2023, the Company announced positive top-line data and subsequently received $15.0 million from the exercise of the

Tranche A warrants. The Tranche B warrants were also immediately exercisable and, following the FDA's approval of VYKAT XR, the remainder of these warrants were exercised. The Company received an aggregate of $35.0 million from the exercise of the Tranche B warrants. As of June 30, 2025, there were no remaining warrants outstanding under the Securities Purchase Agreement.

March 2022 Public Offering of Common Stock

On March 31, 2022, the Company sold 2,666,667 shares of its common stock at a public offering price of $3.75 per share, and for certain investors, in lieu of common stock, pre-funded warrants (the 2022 pre-funded warrants) to purchase 1,333,333 shares of its common stock at a public offering price $3.60 per pre-funded warrant, which represents the per share public offering price for the common stock less the $0.15 per share exercise price for each 2022 pre-funded warrant. The March 2022 pre-funded warrants are immediately exercisable and may be exercised at any time until all of the March 2022 pre-funded warrants are exercised in full. Each share of common stock or March 2022 pre-funded warrant was sold together with one, immediately exercisable, common warrant (the 2022 common warrants) with a five-year term to purchase one share of common stock at an exercise price of $4.50 per share. The net proceeds of the offering were $13.8 million, after deducting the underwriting discount and other offering expenses. The Company is not required under any circumstance to settle any of the 2022 pre-funded warrants or the 2022 common warrants for cash, and therefore classified both types of warrants as permanent equity.

Through June 30, 2025, 2,150,406 of the March 2022 common warrants had been exercised for gross proceeds of $9.7 million and 1,333,329 warrants were exercised using the cashless exercise option with no proceeds to the Company. As of June 30, 2025, 516,265 of the March 2022 common warrants remained outstanding.

At the Market Offering

In July 2024, the Company entered into the Sales Agreement with Jefferies, pursuant to which the Company may offer and sell up to $150.0 million of shares of its common stock, from time to time, through Jefferies.

The Company will pay Jefferies a commission of 3.0% of the aggregate gross proceeds from the sale of shares and has agreed to provide Jefferies with customary indemnification and contribution rights. The Company has also agreed to reimburse Jefferies for certain specified expenses. The Company is not obligated to sell any shares under the Sales Agreement. The offering of the shares pursuant to the Sales Agreement will terminate upon the termination of the Sales Agreement by Jefferies or the Company, as permitted therein.

Common Stock Warrants

As of June 30, 2025 and December 31, 2024, the following table summarizes the Company's outstanding common stock warrants:

	As of June 30, 2025		As of December 31, 2024		
	Number of Common Warrant Shares	Weighted Average Exercise Price per Share	Number of Common Warrant Shares	Weighted Average Exercise Price per Share	Expiration Date
March 2022 Common warrants	516,265	$ 4.50	1,255,346	$ 4.50	March 2027
May 2023 Tranche B warrants	-	$ -	2,065,305	$ 2.50	November 2026
October 2023 pre-funded warrants	250,000	$ 0.01	250,000	$ 0.01	N/A
Total	766,265		3,570,651		

Equity Incentive Plans

2014 Plan

The Company maintains the 2014 Equity Incentive Plan (the 2014 Plan). Under the 2014 Plan the Company may grant stock options, stock appreciation rights, restricted stock, restricted stock units, performance units or performance shares to employees, directors, advisors, and consultants. Options granted under the 2014 Plan may be incentive stock options (ISOs) or nonqualified stock options (NSOs). ISOs may be granted only to Company employees, including officers and directors.

The Board has the authority to determine to whom stock options will be granted, the number of options, the term, and the exercise price. Options are to be granted at an exercise price not less than fair value. For individuals holding more than 10% of the voting rights of all classes of stock, the exercise price of an option will not be less than 110% of fair value. Performance-based grants have vesting contingent upon the achievement of certain performance criteria related to the Company’s commercialization of its therapeutics. The contractual term of an option is no longer than five years for ISOs for which the grantee owns greater than 10% of the voting power of all classes of stock and no longer than ten years for all other options. The terms and conditions governing restricted stock units is at the sole discretion of the Board.

On January 17, 2024, the Company filed a Registration Statement on Form S-8 which registered an additional 1.0 million shares which automatically became available for issuance under the 2014 Plan as of January 1, 2024. On June 6, 2024, the Company's stockholders approved the Amended and Restated 2014 Plan which included an increase of 2.0 million shares, which became immediately available for issuance. On February 28, 2025, the Company filed a Registration Statement on Form S-8 which registered an additional 1.8 million shares which automatically became available for issuance under the 2014 Plan as of January 1, 2025. As of June 30, 2025, a total of 1,104,779 shares were available for future grant under the 2014 Plan.

Inducement Plan

The Company maintains the 2020 Inducement Equity Incentive Plan (the Inducement Plan). The Inducement Plan provides for the grant of equity-based awards, including non-statutory stock options, restricted stock units, restricted stock, stock appreciation rights, performance shares and performance units, and its terms are substantially similar to the 2014 Plan.

In accordance with Rule 5635(c)(4) and Rule 5635(c)(3) of the Nasdaq Listing Rules, awards under the Inducement Plan may only be made to individuals not previously employees or non-employee directors of the Company (or following such individuals' bona fide period of non-employment with the Company), as an inducement material to the individuals' entry into employment with the Company, or, to the extent permitted by Rule 5635(c)(3) of the Nasdaq Listing Rules, in connection with a merger or acquisition. On January 31, 2024, the Company filed a Registration Statement on Form S-8 which registered 500,000 shares available for issuance under the Inducement Plan, which became available for issuance following approval of the Board of Directors on January 24, 2024.

As of June 30, 2025, a total of 8,318 shares were available for future grant under the Inducement Plan.

Stock-based compensation expense

The Company recognizes stock-based compensation expense related to options and restricted stock units granted to employees, directors and consultants. The compensation expense is allocated on a departmental basis, based on the classification of the award holder. No income tax benefits have been recognized in the condensed consolidated statements of operations and comprehensive loss for stock-based compensation arrangements during any of the periods presented.

Stock-based compensation expense was recognized in the condensed consolidated statements of operations and comprehensive loss as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Research and development	$ 2,368	$ 2,705	$ 6,682	$ 5,166
Selling, general and administrative	7,330	4,455	17,695	8,439
Total	$ 9,698	$ 7,160	$ 24,377	$ 13,605

After the FDA approval of VYKAT XR in March 2025, the Company began capitalizing stock-based compensation associated with the allocation of labor costs related to work performed to manufacture VYKAT XR. For the three months ended June 30, 2025, the Company capitalized $0.3 million into inventory.

Stock Options

The Company granted options to purchase 102,242 and 343,700 shares of the Company's common stock to employees during the three months ended June 30, 2025 and 2024, respectively. During the six months ended June 30, 2025, the Company granted options to purchase 689,403 and 3,600 shares of the Company's stock to employees and a consultant, respectively. During the six months ended June 30, 2024, the Company granted options to purchase 1,080,380 of the Company's stock to employees and a consultant. There were no performance-based options granted during the three and six months ended June 30, 2025 and 2024, respectively. The fair value of each award granted was estimated on the date of grant using the Black-Scholes option pricing model with the following assumptions:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Expected life (years)	6.1	6.1	5.9-6.1	5.8-6.1
Risk-free interest rate	3.8%-4.2%	4.3%-4.6%	3.8%-4.7%	4.0%-4.6%
Volatility	117%	122%	117%-120%	122%-124%
Dividend rate	— %	— %	— %	— %

The Black-Scholes option-pricing model requires the use of highly subjective assumptions to estimate the fair value of stock-based awards. These assumptions include the following estimates:

- *Expected life:* The expected life of stock options represents the period of time that the options are expected to be outstanding. Due to the lack of historical exercise history, the expected life of the Company's service-based stock options has been determined utilizing the "simplified method", based on the average of the contractual term of the options and the weighted-average vesting period. The expected life for the performance-based options was determined based on consideration of the contractual term of the stock options, an estimate of the date the performance criteria would be met and expectations of employee behavior.
- *Risk-free interest rate:* The risk-free interest rate is based on the yields of U.S. Treasury securities with maturities similar to the expected life of the stock options.
- *Volatility:* The estimated volatility rate is based on the volatilities of the Company's common stock for a historical period equal to the expected life of the stock options.
- *Dividend rate:* The Company has never declared or paid any cash dividends and does not presently plan to pay cash dividends in the foreseeable future. Consequently, the Company used an expected dividend yield of zero.

The following table summarizes stock option transactions for the six months ended June 30, 2025 which were for awards issued under the 2014 Plan and the Inducement Plan:

	Number of Options Outstanding	Weighted-Average Exercise Price per Share	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Balance at January 1, 2025	3,971,798	$ 26.75	8.48	$ 79,578
Options granted	693,003	53.24		
Options exercised	(1,026,329)	13.63		
Options canceled/forfeited	(170,194)	52.61		
Balance at June 30, 2025	3,468,278	$ 34.66	8.47	$ 170,585
Options exercisable at June 30, 2025	1,007,206	$ 24.22	7.47	$ 60,193
Options vested and expected to vest at June 30, 2025	3,468,278	$ 34.66	8.47	$ 170,585

The weighted-average grant date fair value of options granted was $46.61 and $36.34 per share for the six months ended June 30, 2025 and 2024, respectively. The intrinsic value of the stock options exercised was $55.6 million and $3.9 million for the six months ended June 30, 2025 and 2024, respectively. At June 30, 2025, total unrecognized employee stock-based compensation related to stock options that are likely to vest was $77.2 million, which is expected to be recognized over the weighted-average remaining vesting period of 2.7 years.

Restricted Stock Units

There were 130,955 and 32,500 restricted stock units granted to employees and directors by the Company during the three months ended June 30, 2025 and 2024, respectively, and 353,581 and 391,530 restricted stock units granted to employees and directors by the Company during the six months ended June 30, 2025 and 2024, respectively. During the six months ended June 30, 2024, the Company granted 10,000 performance-based restricted stock units to a consultant. The shares were valued based on the Company's common stock price on the grant date.

The following table summarizes restricted stock unit transactions for the six months ended June 30, 2025 under the 2014 Plan:

	Number of Restricted Stock Units	Weighted-Average Grant-Date Fair Value per Share
Outstanding at January 1, 2025	939,865	$ 48.55
Restricted stock units granted	353,581	$ 57.26
Restricted stock units vested	(819,273)	$ 48.58
Restricted stock units canceled/forfeited	(5,716)	$ 52.82
Outstanding at June 30, 2025	468,457	$ 55.02

The weighted-average grant-date fair value of all restricted stock units granted during the six months ended June 30, 2025 and 2024 was $57.26 and $37.58, respectively. The fair value of all restricted stock units vested during the six months ended June 30, 2025 and 2024 was $40.6 million and $6.8 million, respectively. At June 30, 2025, total unrecognized employee stock-based compensation related to restricted stock units was $19.3 million, which is expected to be recognized over the weighted-average remaining vesting period of 2.2 years.

Certain directors have elected to delay the issuance of certain vested restricted stock units granted under the 2014 Plan. Certain of these RSUs have vested as of June 30, 2025 based on the applicable service periods, but the issuance of common stock has been deferred until a future date when the directors' service to the Company terminates, or there is a change in control of the Company. The grant-date fair value of these 13,000 shares is $0.6 million, which has been recognized as stock-based compensation expense over the requisite service period in the condensed consolidated statements of operations and comprehensive loss.

2014 Employee Stock Purchase Plan

The Company's board of directors and stockholders have adopted the 2014 Employee Stock Purchase Plan (the ESPP). The ESPP has become effective, and the board of directors will implement commencement of offers thereunder in its discretion. A total of 1,864 shares of the Company's common stock has been made available for sale under the ESPP. In addition, the ESPP provides for annual increases in the number of shares available for issuance under the plan on the first day of each year beginning in the year following the initial date that the board of directors authorizes commencement, equal to the least of:

- 1.0% of the outstanding shares of the Company's common stock on the first day of such year;
- 3,729 shares; or
- such amount as determined by the board of directors.

As of June 30, 2025, there were no purchases by employees under this plan.

Note 9. Net loss per share

Basic net loss per share is computed by dividing net loss by the weighted-average number of common stock outstanding during the period. Shares of common stock that are potentially issuable for little or no cash consideration at issuance, such as the Company's pre-funded warrants issued in October 2023 and in connection with the exercise of certain May 2023 Tranche A and Tranche B warrants, are considered outstanding common stock and are included in the calculation of basic and diluted net loss per share in connection with *ASC 260 Earnings Per Shares*. Diluted net loss per share is computed by dividing net loss by the weighted-average number of common stock outstanding and dilutive potential common stock that would be issued upon the exercise or vesting of common stock awards and exercise of common stock warrants that are not pre-funded. The Company applies the two-class method to calculate basic and diluted earnings per share as its warrants issued in March 2022, May 2023 and October 2023 are participating securities. However, the two-class method does not impact the net loss per share of common stock as the March 2022, May 2023 and October 2023 common warrants issued do not participate in losses. For the three and six months ended June 30, 2025 and 2024, the effect of issuing potential common stock is anti-dilutive due to the net losses in those periods and therefore the number of shares used to compute basic and diluted net loss per share are the same in each of those periods.

The following securities are the weighted-average common shares outstanding used to calculate basic and diluted net loss per common share:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Common stock	50,233,281	35,641,878	48,092,928	34,037,201
May 2023 Tranche A pre-funded warrants	-	2,241,352	-	2,325,091
May 2023 Tranche B pre-funded warrants	-	451,632	-	451,632
October 2023 pre-funded warrants	250,000	296,703	250,000	606,044
Total	50,483,281	38,631,565	48,342,928	37,419,968

The following potentially dilutive securities outstanding have been excluded from the computations of diluted weighted-average shares outstanding for the periods presented because such securities have an antidilutive impact due to losses reported (in common stock equivalent shares):

	As of June 30,	
	2025	2024
Warrants issued to 2010/2012 convertible note holders to purchase common stock	-	6,804
Warrants issued to underwriter to purchase common stock	-	1,100
March 2022 common warrants	516,265	1,483,743
May 2023 Tranche B warrants	-	5,775,000
Options to purchase common stock	3,468,278	3,305,259
Outstanding restricted stock units	468,457	256,030
Total	4,453,000	10,827,936

Note 10. Segment Reporting

The Company has one operating and reporting segment focused on the development and commercialization of its lead therapeutic product, VYKAT XR. The Company's chief operating decision maker (CODM) is the chief executive officer who reviews product revenue, net, and cash operating expenses on a consolidated basis to make decisions about allocating resources and assessing performance for the entire Company. The CODM does not review assets at a level or category different than the amounts disclosed in the condensed consolidated balance sheet.

The following table presents selected financial information with respect to the Company's single operating segment for the three and six months ended June 30, 2025 and 2024 (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Revenue				
Product revenue, net	$ 32,657	$ -	$ 32,657	$ -
Net loss	(4,708)	(21,854)	(48,481)	(43,252)
Less total other income, net	1,817	3,014	3,787	5,091
Operating loss	(6,525)	(24,868)	(52,268)	(48,343)
Total operating expenses	39,182	24,868	84,925	48,343
Less non-cash expenses				
Depreciation and amortization	(502)	(494)	(1,006)	(984)
Non-cash lease expense	(169)	(70)	(364)	(139)
Change in fair value of contingent consideration	(1,101)	(1,637)	(4,068)	(2,038)
Stock-based compensation	(9,698)	(7,160)	(24,377)	(13,605)
Cash operating expenses	$ 27,712	$ 15,507	$ 55,110	$ 31,577

Note 11. Subsequent Events

The Company has evaluated its subsequent events from June 30, 2025 through the date these condensed consolidated financial statements were issued and has determined that there are no subsequent events disclosure required, with the exception of the following:

July 2025 Public Offering of Common Stock

In July 2025 the Company closed an underwritten public offering of 2,705,882 shares of its common stock at an offering price of $85.00 per share, which included the exercise in full by the underwriters of their option to purchase additional shares of its common stock. The gross proceeds of the public offering were $230.0 million, before deducting the underwriter discount and other offering expenses, totaling approximately $14.3 million.

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

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, and Section 21E of the Securities Exchange Act of 1934, as amended, that involve substantial risks and uncertainties. Forward-looking statements generally relate to future events or our future financial or operating performance. In some cases, you can identify forward-looking statements because they contain words such as "may," "will," "appears," "should," "expects," "plans," "anticipates," "could," "intends," "target," "projects," "contemplates," "believes," "estimates," "predicts," "potential," or "continue," or the negative of these words or other similar terms or expressions that concern our expectations, strategy, plans, or intentions. Forward-looking statements contained in this Quarterly Report on Form 10-Q include, but are not limited to, any projections of financial information; any statements about the commercialization of VYKAT XR, any statements about historical results that may suggest trends for our business; any statements of the plans, strategies, and objectives of management for future operations; any statements of expectation or belief regarding future events, our products, product sales, expenses, liquidity, cash flow, market growth rates or enforceability of our intellectual property rights and related litigation expenses; and any statements of assumptions underlying any of the foregoing. We have based these forward-looking statements on our current expectations and projections about future events and trends that we believe may affect our business, financial condition, results of operations, prospects, business strategy, and financial needs. The outcome of the events described in these forward-looking statements is subject to known and unknown risks, uncertainties, and other factors described in the section titled "Risk Factors" and elsewhere in this Quarterly Report on Form 10-Q. We operate in a very competitive and rapidly changing environment. New risks and uncertainties emerge from time to time and it is not possible for us to predict all risks and uncertainties that could have an impact on the forward-looking statements contained in this Quarterly Report on Form 10-Q. We cannot assure you that the results, events, and circumstances reflected in the forward-looking statements will be achieved or occur, and actual results, events, or circumstances could differ materially from those described in the forward-looking statements. All forward-looking statements are based on information and estimates available to us at the time of filing this Quarterly Report on Form 10-Q and are not guarantees of future performance. We undertake no obligation to update any forward-looking statements made in this Quarterly Report on Form 10-Q to reflect events or circumstances after the date of this Quarterly Report on Form 10-Q or to reflect new information or the occurrence of unanticipated events, except as required by law.

The interim consolidated financial statements included in this Quarterly Report on Form 10-Q and this Management's Discussion and Analysis of Financial Condition and Results of Operations should be read in conjunction with the financial statements and notes thereto for the year ended December 31, 2024, and the related Management's Discussion and Analysis of Financial Condition and Results of Operations, contained in our Annual Report on Form 10-K for the year ended December 31, 2024.

Business Overview

We are a biopharmaceutical company developing novel therapeutics for the treatment of rare diseases. On March 26, 2025, we announced that our lead product candidate, VYKAT XR (diazoxide choline) extended-release tablets, formerly known as DCCR, had been approved by the U.S. Food and Drug Administration (FDA). VYKAT XR is indicated to treat hyperphagia in adults and pediatric patients four years of age and older with Prader-Willi syndrome (PWS).

We began commercial marketing and sales and recognizing revenue during the three months ended June 30, 2025. The transaction price that we recognize as revenue for VYKAT XR sales includes an estimate of variable consideration, which includes rebates, discounts, returns, and copay assistance that are offered within our contract with our specialty pharmacy. Refer to Note 3 of the notes to the unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q for additional information.

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of financial condition and results of operations are based upon our unaudited condensed consolidated financial statements, which have been prepared in accordance with GAAP. The preparation of these condensed consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenues and expenses. On an on-going basis, we evaluate our critical accounting policies and estimates. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable in the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions and conditions.

Revenue Recognition

After FDA approval of VYKAT XR in March 2025, we began commercial marketing and made our first product sales in the three months ended June 30, 2025. ASC Topic 606, *Revenue from Contracts with Customers*, requires us to make estimates of variable

consideration, included in contracts with customers, to be included in the transaction price. The transaction price that is recognized as revenue for products upon delivery and transfer of title to the customer includes an estimate of variable consideration for reserves, which result from rebates, discounts, returns, and co-pay assistance that are offered within contracts between us and our customer.

Government rebates: We are subject to discount obligations under several government programs, including Medicaid programs, Medicare and TRICARE in the United States. We estimate these rebates based upon a range of possible outcomes that are weighted for the estimated payer mix. These reserves are recorded in the same period that the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a liability that is included in accrued expenses and other current liabilities on our condensed consolidated balance sheets. On a quarterly basis, we update our estimates and record any adjustments in the period that we identify the adjustments.

Trade discounts and allowances: We provide discounts on VYKAT XR sales to our customer for prompt payment. This discount is recorded as a reduction of revenue in the period the related product revenue is recognized. In addition, we receive and pay for various distribution services from our customer in the distribution channel.

Product returns: Our customer has limited return rights related to unexpected instances in which the product is found to be damaged or defective. We estimate the amount of product sales that may be returned and records the estimate as a reduction of revenue and a refund liability in the period in which the related product revenue is recognized. Based on the distribution model for VYKAT XR, we believe there will be minimal returns as such returns have not been material to date.

Other incentives: Other incentives include co-payment assistance we provide to patients with commercial insurance that have coverage and reside in states that allow co-payment assistance. The calculation of the accrual for co-pay assistance is based on an estimate of claims and the cost per claim that we expect to receive associated with product that has been recognized as revenue. The estimate is recorded as a reduction of revenue in the same period the related revenue is recognized.

Variable consideration is estimated and reduces the transaction price to reflect our best estimate of the amount of consideration to which we are entitled based on the terms of the contracts and are recorded in the same period the related product revenues is recognized. The amount of variable consideration that is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is considered probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period. Actual amounts of consideration ultimately received may differ from our estimates. If actual results in the future vary from our estimates, we will adjust these estimates in the period these variances become known.

Aside from revenue recognition as noted above, there have been no significant changes during the three and six months ended June 30, 2025 compared to those previously disclosed in “Critical Accounting Policies and Estimates” in “Management’s Discussion and Analysis of Financial Condition and Results of Operations” included in our Annual Report on Form 10-K for the year ended December 31, 2024. Our significant accounting policies are more fully described in Note 3 of our most recent Annual Report on Form 10-K.

Results of Operations

Comparison of the three months ended June 30, 2025 and 2024 (in thousands)

	Three Months Ended June 30,		Increase (decrease)	
	2025	2024	Amount	Percentage
Revenue				
Product revenue, net	$ 32,657	$ -	$ 32,657	100%
Total revenue	32,657	-	32,657	100%
Operating expenses				
Cost of goods sold	696	-	696	100%
Research and development	9,147	12,342	(3,195)	(26%)
Selling, general and administrative	28,238	10,889	17,349	159%
Change in fair value of contingent consideration	1,101	1,637	(536)	(33%)
Total operating expenses	39,182	24,868	14,314	58%
Operating loss	(6,525)	(24,868)	18,343	(74%)
Other income (expense), net				
Interest income, net	3,193	3,014	179	6%
Interest expense	(1,376)	-	(1,376)	(100%)
Total other income (expense), net	1,817	3,014	(1,197)	(40%)
Net loss	$ (4,708)	$ (21,854)	$ 17,146	(78%)

Product Revenue, Net

Product revenue, net was $32.7 million for the three months ended June 30, 2025, due to sales of VYKAT XR after FDA approval was obtained in March 2025, compared to zero for the three months ended June 30, 2024.

Cost of Goods Sold

Cost of goods sold was $0.7 million for the three months ended June 30, 2025, due to sales of VYKAT XR after FDA approval was obtained in March 2025, compared to zero for the three months ended June 30, 2024. Prior to receiving FDA approval for VYKAT XR in March 2025, costs associated with the manufacturing of VYKAT XR were expensed as research and development expense. As such, a portion of the cost of inventory sold during the period was expensed prior to FDA approval.

Research and development expense

Research and development expense was $9.1 million, which includes $2.4 million of non-cash stock-based compensation, for the three months ended June 30, 2025, compared to $12.3 million, which includes $2.7 million of non-cash stock-based compensation, in the same period of 2024. Costs in support of our June 2024 New Drug Application (NDA) submission, supply chain activities, and clinical activities decreased $2.6 million, $0.3 million, and $0.5 million, respectively, between comparable periods. We incurred $0.6 million in the three months ended June 30, 2025 towards our Marketing Authorization Application (MAA) submission in Europe, which was submitted in the second quarter of 2025. The cadence of our research and development expenditures will fluctuate depending upon the state of our clinical programs, the timing of manufacturing and other projects necessary to support the submission of our regulatory filings and activities for commercial launch. Of the non-cash stock-based compensation recognized in the three months ended June 30, 2025, $0.2 million was due to performance-based RSU grants which vested upon approval of our NDA for VYKAT XR by the FDA in March 2025.

Selling, general and administrative expense

Selling, general and administrative expense was $28.2 million, which includes $7.3 million of non-cash stock-based compensation, for the three months ended June 30, 2025, compared to $10.9 million, which includes $4.5 million of non-cash stock-based compensation, in the same period of 2024. Personnel and associated costs increased $7.6 million as we hired additional employees for commercial launch and in support of our increased business activities. New program costs associated with commercial launch, including disease state education, analytics, other marketing programs, medical affairs activities and patient advocacy activities increased by $6.8 million. We expect selling, general and administrative expenses to continue to increase as we have begun commercialization of VYKAT XR. Of the non-cash stock-based compensation recognized in the three months ended June 30, 2025, $0.4 million was due to performance-based RSU grants which vested upon approval of our NDA for VYKAT XR by the FDA in March 2025.

Change in fair value of contingent consideration

We are obligated to make cash payments up to a maximum of $21.2 million to the former Essentialis stockholders upon the achievement of certain future commercial milestones associated with the sales of VYKAT XR in accordance with the terms of our 2017 merger agreement with Essentialis. The fair value of the liability for the contingent consideration payable by us achieving two commercial sales milestones of $100 million and $200 million in cumulative revenue in future years was estimated to be $18.9 million as of June 30, 2025, a $1.1 million increase from the estimate as of March 31, 2025, primarily due to the approval of our NDA for VYKAT XR by the FDA in March 2025 and recording product revenue from sales of VYKAT XR subsequent to the approval. During the three months ended June 30, 2024, the estimate increased by $1.6 million from the $12.0 million estimate as of March 31, 2024.

Other income (expense), net

We had other income (expense), net of approximately $1.8 million in the three months ended June 30, 2025, compared to approximately $3.0 million during the three months ended June 30, 2024. The decrease was primarily due to interest expense associated with the long-term debt, partially offset by an increase in interest income driven by higher cash and cash equivalents and marketable securities during the three months ended June 30, 2025, compared to the three months ended June 30, 2024.

Comparison of the six months ended June 30, 2025 and 2024 (in thousands)

	Six Months Ended June 30,		Increase (decrease)	
	2025	2024	Amount	Percentage
Revenue				
Product revenue, net	$ 32,657	$ -	$ 32,657	100%
Total revenue	32,657	-	32,657	100%
Operating expenses				
Cost of goods sold	696	-	696	100%
Research and development	22,664	26,944	(4,280)	(16%)
Selling, general and administrative	57,497	19,361	38,136	197%
Change in fair value of contingent consideration	4,068	2,038	2,030	100%
Total operating expenses	84,925	48,343	36,582	76%
Operating loss	(52,268)	(48,343)	(3,925)	8%
Other income (expense), net				
Interest income, net	6,524	5,091	1,433	28%
Interest expense	(2,737)	-	(2,737)	(100%)
Total other income (expense), net	3,787	5,091	(1,304)	(26%)
Net loss	$ (48,481)	$ (43,252)	$ (5,229)	12%

Product Revenue, Net

Product revenue, net was $32.7 million for the six months ended June 30, 2025, due to sales of VYKAT XR after FDA approval was obtained in March 2025, compared to zero for the six months ended June 30, 2024.

Cost of Goods Sold

Cost of goods sold was $0.7 million for the six months ended June 30, 2025, due to sales of VYKAT XR after FDA approval was obtained in March 2025, compared to zero for the six months ended June 30, 2024. Prior to receiving FDA approval for VYKAT XR in March 2025, costs associated with the manufacturing of VYKAT XR were expensed as research and development expense. As such, a portion of the cost of inventory sold during the period was expensed prior to FDA approval.

Research and development expense

Research and development expense was $22.7 million, which includes $6.7 million of non-cash stock-based compensation, for the six months ended June 30, 2025, compared to $26.9 million, which includes $5.2 million of non-cash stock-based compensation, in the same period of 2024. Personnel and other associated costs increased $1.8 million as we hired additional employees in support of our research and development activities while costs in support of our June 2024 NDA submission decreased $3.9 million. Supply chain activities and clinical activities decreased $1.5 million and $3.6 million, respectively, between comparable periods. We incurred

$1.3 million in the six months ended June 30, 2025 towards our MAA submission in Europe, which was submitted in the second quarter of 2025. The cadence of our research and development expenditures will fluctuate depending upon the state of our clinical programs, the timing of manufacturing and other projects necessary to support the submission of our regulatory filings and activities for commercial launch. Of the non-cash stock-based compensation recognized in the six months ended June 30, 2025, $2.6 million was due to performance-based RSU grants which vested upon approval of our NDA for VYKAT XR by the FDA in March 2025.

Selling, general and administrative expense

Selling, general and administrative expense was $57.5 million, which includes $17.7 million of non-cash stock-based compensation, for the six months ended June 30, 2025, compared to $19.4 million, which includes $8.4 million of non-cash stock-based compensation, in the same period of 2024. Personnel and associated costs increased $15.3 million as we hired additional employees for commercial launch and in support of our increased business activities. New program costs associated with commercial launch, including disease state education, analytics, other marketing programs, medical affairs activities and patient advocacy activities increased by $12.6 million. We expect selling, general and administrative expenses to continue to increase as we have begun commercialization of VYKAT XR. Of the non-cash stock-based compensation recognized in the six months ended June 30, 2025, $5.1 million was due to performance-based RSU grants which vested upon approval of our NDA for VYKAT XR by the FDA in March 2025.

Change in fair value of contingent consideration

We are obligated to make cash payments up to a maximum of $21.2 million to the former Essentialis stockholders upon the achievement of certain future commercial milestones associated with the sales of VYKAT XR in accordance with the terms of our 2017 merger agreement with Essentialis. The fair value of the liability for the contingent consideration payable by us achieving two commercial sales milestones of $100 million and $200 million in cumulative revenue in future years was estimated to be $18.9 million as of June 30, 2025, a $4.1 million increase from the estimate as of December 31, 2024, primarily due to the approval of our NDA for VYKAT XR by the FDA in March 2025 and recording product revenue from sales of VYKAT XR subsequent to the approval. During the six months ended June 30, 2024, the estimate increased by $2.0 million from the $11.6 million estimate as of December 31, 2023.

Other income (expense), net

We had other income (expense), net of approximately $3.8 million in the six months ended June 30, 2025, compared to approximately $5.1 million during the six months ended June 30, 2024. The decrease was primarily due to interest expense associated with the long-term debt, partially offset by an increase in interest income driven by higher cash and cash equivalents and marketable securities during the six months ended June 30, 2025, compared to the six months ended June 30, 2024.

Liquidity and Capital Resources

We used $45.4 million of cash in operating activities and had a net loss of $48.5 million during the six months ended June 30, 2025. We had an accumulated deficit of $500.7 million at June 30, 2025 as a result of having incurred losses since our inception. We had $76.5 million in cash and cash equivalents, $217.3 million of marketable securities and $295.8 million of working capital on June 30, 2025 and we had a lease obligation totaling $2.9 million to be paid through August 2029, consisting of one operating lease for office space in Redwood City, California. Our ability to achieve operating profitability is dependent upon the successful commercialization of VYKAT XR.

As of June 30, 2025, we had $50.0 million outstanding under our loan and security agreement with Oxford. Under the terms of the loan agreement with Oxford, following FDA approval of VYKAT XR, an additional $50 million is available through September 30, 2025 and $25 million will become available during the period October 1, 2025 to September 30, 2026. An additional tranche of $25 million may become available upon achievement of certain commercial milestones. A final $50 million may be made available upon mutual consent with Oxford. The loan carries an interest-only period of 48 months and a total term of 60 months; provided that if specific milestones are achieved prior to September 30, 2026, the interest-only period and maturity date will be extended by 12 months. The term loans accrue interest at a floating rate equal to, subject to certain conditions, (a) 1-month term SOFR plus (b) 5.50%.

In July 2024, we entered into an Open Market Sale Agreement^{SM} with Jefferies LLC, as sales agent (Jefferies), pursuant to which we may offer and sell, from time to time, through Jefferies, shares of our common stock having an aggregate offering price of up to $150 million.

In July 2025, we closed an underwritten public offering of 2,705,882 shares of our common stock at a public offering price of $85.00 per share, which included the exercise in full by the underwriters of their option to purchase additional shares of our common stock. The gross proceeds of the public offering were $230.0 million, before deducting the underwriter discount and other offering expenses, totaling approximately $14.3 million.

We believe that our existing cash, cash equivalents and marketable securities will be sufficient to meet the company's working capital needs for the next twelve months. Our long-term capital requirements will depend on several factors, most notably the timing and degree of success of our commercialization of VYKAT XR. We believe that we will continue to have access to capital resources through possible public or private equity offerings, debt financings, corporate collaborations or other means, but the access to such capital resources is uncertain and is not assured.

Cash flows

The following table sets forth the primary sources and uses of cash and cash equivalents for each of the periods presented below (in thousands):

	Six Months Ended June 30,	
	2025	2024
Net cash used in operating activities	$ (45,360)	$ (30,179)
Net cash provided by (used in) investing activities	15,022	(236,165)
Net cash provided by financing activities	18,907	153,687
Net decrease in cash and cash equivalents	$ (11,431)	$ (112,657)

Net cash used in operating activities

During the six months ended June 30, 2025, operating activities used net cash of $45.4 million, which was primarily due to the net loss of $48.5 million, less non-cash expense of $24.4 million for stock-based compensation, $1.0 million for depreciation and amortization, $0.4 million for non-cash lease expense, $4.1 million related to the change in fair value of contingent consideration, and $2.0 million added back for accretion of premium/discount on marketable securities. Additionally, cash used during the six months ended June 30, 2025 decreased by $24.8 million due to changes in operating assets and liabilities.

During the six months ended June 30, 2024, operating activities used net cash of $30.2 million, which was primarily due to the net loss of $43.3 million, less non-cash expense of $13.6 million for stock-based compensation, $1.0 million for depreciation and amortization, $0.1 million for non-cash lease expense, and $2.0 million related to the change in fair value of contingent consideration, and $1.6 million added back for the accretion of premium/discount on marketable securities. Additionally, cash used during the six months ended June 30, 2024 decreased by $2.0 million due to changes in operating assets and liabilities.

Net cash provided by (used in) investing activities

During the six months ended June 30, 2025, we used $104.8 million for purchases of marketable securities. We received proceeds of $119.8 million from maturities of marketable securities.

During the six months ended June 30, 2024, we used $261.1 million for purchases of marketable securities and $19 thousand for purchases of property and equipment. We received proceeds of $25.0 million from maturities of marketable securities.

Net cash provided by financing activities

During the six months ended June 30, 2025, we received $5.2 million from the exercise of common stock warrants. We also received $14.0 million from the exercise of stock options. We paid $0.1 million of debt issuance costs.

During the six months ended June 30, 2024, we received $149.0 million from the sale of common stock, net of issuance costs, and $3.5 million from the exercise of common stock and pre-funded stock warrants. Additionally, we received $0.6 million from the exercise of common stock warrants prior to June 30, 2024, with the issuance of common shares occurring after June 30, 2024. We also received $0.6 million from the exercise of stock options.

Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements.

Recent Accounting Pronouncements

See "Recent Adopted Accounting Pronouncements" described in Note 3, Basis of Presentation and Summary of Significant Accounting Policies within Notes to the Condensed Consolidated Financial Statements.

Recent Developments

On July 4, 2025, President Trump signed H.R. 1, the "One Big Beautiful Bill Act", into law. In accordance with U.S. GAAP, we will account for the tax effects of changes in tax law in the period of enactment, which is expected to be the period of the three months ended September 30, 2025. We are currently in the process of analyzing the tax impacts of the law change, but we do not expect a material impact on our financial statements.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

There have not been any material changes to our exposure to market risk during the six months ended June 30, 2025. For additional information regarding market risk, refer to Part II, Item *7A Qualitative and Quantitative Disclosures About Market Risk* in our Annual Report on Form 10-K for the year ended December 31, 2024.

Item 4. Controls and Procedures

Inherent Limitations on Effectiveness of Controls

In designing and evaluating the disclosure controls and procedures, management recognizes that any controls and procedures, even if determined effective and no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives to prevent or detect misstatements. In addition, the design of disclosure controls and procedures must reflect the fact that there are resource constraints and that management is required to apply judgment in evaluating the benefits of possible controls and procedures relative to their costs. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

(a) Evaluation of Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in reports filed under the Securities Exchange Act of 1934, as amended (Exchange Act), is recorded, processed, summarized and reported within the time periods specified in U.S. Securities and Exchange Commission, or SEC, rules and forms, and that such information is accumulated and communicated to our management to allow timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives.

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, has evaluated the effectiveness of the design and operation of our disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act as of the end of the period covered by this Quarterly Report on Form 10-Q. Based upon that evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that, as of the end of the period covered by this Quarterly Report on Form 10-Q, our disclosure controls were not effective at the reasonable assurance level due to the material weakness described below.

(b) Material Weakness in Internal Control

As previously disclosed in our Annual Report on Form 10-K for the year ended December 31, 2024, we identified a material weakness in internal control related to ineffective design and operation of controls over certain information technology general controls (ITGCs), including segregation of incompatible duties, program change management, and user access controls. As a result, the related business process controls (IT application controls and IT-dependent manual controls) that are dependent on the ineffective ITGCs, or that use data produced from the system impacted by the ineffective ITGCs, were also ineffective.

The material weakness identified above did not result in any material misstatements in our financial statements or disclosures, and there were no changes to previously released financial results. Our management concluded that the unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q, present fairly, in all material respects, our financial position, results of operations, and cash flows for the periods presented in accordance with accounting principles generally accepted in the United States of America (GAAP).

(c) Remediation of Material Weakness

Our management is committed to maintaining a strong internal control environment. Management is taking comprehensive actions to remediate the material weakness in internal control over financial reporting, which include the following:

- Hire additional personnel in the accounting and financial reporting function that would allow for appropriate segregation of duties, both systematically and operationally. We will continue to reassess staffing and add additional resources, as required, with the requisite experience and training, to support our system of internal control;

- Implement a training program for all personnel responsible for internal controls over financial reporting, including educating control owners regarding the requirements of each control. For control owners with IT responsibilities, develop and implement additional training and awareness programs addressing ITGC policy and requirements, with a specific focus on user access and change management processes and controls;

- Continue to enhance, standardize and monitor the ongoing improvements in design and operating effectiveness of our controls and the adherence of our personnel to any enhancements in controls, policies, and procedures. Moreover, within our IT environment, increase the extent of oversight and verification checks included in the operation of user access and program change management controls and processes; and

- We will continue to report regularly to the audit committee of our board of directors on the progress and results of the remediation plan, including the identification, status and resolution of internal control deficiencies.

We believe the foregoing efforts will effectively remediate the identified material weakness in internal controls over financial reporting. Because the reliability of the internal control process requires repeatable execution, the successful remediation will require review and evidence of effectiveness prior to management concluding that our internal controls over financial reporting are effective. We may also conclude that the additional measures may be required to remediate the material weakness which may necessitate additional implementation and evaluation time. We will continue to assess the effectiveness of our internal control over financial reporting and take steps to remediate the material weakness expeditiously.

(d) Changes in Internal Control over Financial Reporting

During the six months ended June 30, 2025, we began to implement certain internal controls in connection with remediation efforts related to the material weakness identified in our Annual Report on Form 10-K for the year ended December 31, 2024.

Except as described above, there have been no changes to our internal control over financial reporting that occurred during the six months ended June 30, 2025, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II – OTHER INFORMATION

Item 1. Legal Proceedings

We may, from time to time, be party to litigation and subject to claims that arise in the ordinary course of business. In addition, third parties may, from time to time, assert claims against us in the form of letters and other communications. We currently believe that these ordinary course matters will not have a material adverse effect on our business; however, the results of litigation and claims are inherently unpredictable. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

Item 1A. Risk Factors

An investment in our securities has a high degree of risk. Before you invest you should carefully consider the risks and uncertainties. Additional risks and uncertainties not currently known to us or that we currently deem to be immaterial also may materially adversely affect our business, financial conditions and/or operating results. If any of these risks actually occur, our business, operating results and financial condition could be harmed, and the value of our stock could go down. This means you could lose all or a part of your investment.

Summary Risk Factor

Our business is subject to numerous risks and uncertainties that you should consider before investing in our company, as fully described below. The principal factors and uncertainties that make investing in our company risky include, among others:

- we have limited commercialization history as a company and have incurred significant losses since our inception;
- we are dependent upon the success of VYKAT XR, our sole FDA-approved product;
- VYKAT XR may fail to achieve the degree of market acceptance by physicians, patients, caregivers, healthcare payers and others in the medical community necessary for commercial success;
- we expect to continue to expand our development, regulatory and sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations;
- our patent rights may prove to be an inadequate barrier to competition;
- we have incurred and will continue to incur significant increased costs as a result of operating as a public company, and our management has devoted and will be required to continue to devote substantial time to new compliance initiatives; and
- we may need additional funds to support our operations, and such funding may not be available to us on acceptable terms, or at all, which would force us to delay, reduce or suspend our research and development programs and other operations or commercialization efforts. Raising additional capital may subject us to unfavorable terms, cause dilution to our existing stockholders, restrict our operations, or require us to relinquish rights to our planned products and technologies.

Risks related to our financial condition and capital requirements

We have limited commercialization history as a company and have incurred significant losses since our inception.

We have historically been a clinical-stage company with no approved therapeutic products or revenues from the sale of therapeutic products. Following FDA approval on March 26, 2025, we have begun to commercialize our first product, VYKAT XR, to treat hyperphagia in patients four years and older who have Prader-Willi syndrome (PWS). Evaluating our performance, viability or future success will be more difficult than if we had a longer operating history or prior approved products for sale on the market. Even following approval of VYKAT XR, we will continue to incur significant research and development and selling, general and administrative expenses and will incur substantial commercial expenses related to our operations.

We expect that our immediate future financial results will depend primarily on our success in launching, selling and supporting VYKAT XR. This will require us to be successful in a range of activities, including manufacturing, marketing and selling our products. We are only in the preliminary stages of some of these activities. We may not succeed in these activities and may never generate revenue that is sufficient to be profitable in the future. Even if we are profitable, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to achieve sustained profitability would depress the value of our company and could impair our ability to raise capital, expand our business, diversify our planned products, market our current and planned products, or continue our operations.

We are dependent upon the success of VYKAT XR, our sole FDA-approved product.

We have invested substantially all of our efforts and financial resources in the development of VYKAT XR for the treatment of PWS, a rare complex genetic neurobehavioral/metabolic disease. Our ability to generate product revenues will depend primarily on the successful commercialization of VYKAT XR.

Further, the success of VYKAT XR will depend on a number of factors, including the following:

- building an infrastructure capable of supporting product sales, marketing, and distribution of VYKAT XR in the U.S. and territories where we pursue commercialization directly;
- the success of commercial manufacturing arrangements with third party manufacturers;
- establishing commercial distribution agreements with third party distributors in geographies outside of the United States;
- launching commercial sales of VYKAT XR whether alone or in collaboration with others;
- acceptance of VYKAT XR by patients, the medical community, and third-party payers;
- completing any post-marketing requirements or post-approval commitments to applicable regulatory authorities;
- obtaining a commercially viable price;
- effectively competing with other therapies;
- receiving marketing approvals in the European Union (E.U.) and other geographies;
- a continued acceptable safety and efficacy profile of VYKAT XR;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity; and
- protecting our rights in our intellectual property portfolio, including any potential challenges to patent listings in the U.S "Approved Drug Products with Therapeutic Equivalence Evaluations" or the "Orange Book".

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize VYKAT XR, which would materially harm our business.

We may need additional funds to support our operations, and such funding may not be available to us on acceptable terms, or at all, which would force us to delay, reduce or suspend our research and development programs and other operations or commercialization efforts. Raising additional capital may subject us to unfavorable terms, cause dilution to our existing stockholders, restrict our operations, or require us to relinquish rights to our planned products and technologies.

We are in the early stages of commercializing VYKAT XR, our current sole novel therapeutic product, and accordingly, through June 30, 2025, have just begun to generate revenue from operations. We had a net loss of $175.9 million during the year ended December 31, 2024 (including non-cash stock-based compensation of $100.0 million) and $4.7 million during the three months ended June 30, 2025 (including non-cash stock-based compensation of $10.0 million) and an accumulated deficit of $500.7 million at June 30, 2025 as a result of having incurred losses since our inception. We had $76.5 million in cash and cash equivalents and $217.3 million in marketable securities at June 30, 2025, used $69.1 million and $12.6 million of cash in operating activities during the year ended December 31, 2024 and three months ended June 30, 2025, respectively.

We may need to raise additional capital, either through debt or equity financings to achieve our business plan objectives, including ongoing expenses related to our ongoing regulatory activities and scaling of a sales and marketing organization to support the launch of VYKAT XR.

Because of the numerous risks and uncertainties associated with our product development and commercialization efforts, we are unable to predict the extent of our future losses or when, or if, we will generate meaningful revenue or become profitable, and it is possible we will never achieve these goals. Our ability to obtain additional financing will depend on a number of factors, including, among others, the other risks described in this "Risk Factors" section. If any one of these risks are realized, we may not be able to obtain additional funding, in which case, our business could be jeopardized and we may not be able to continue our operations or pursue our strategic plans. If we are forced to scale down, limit or cease operations, our stockholders could lose all of their investment. Even if we are successful at raising capital, there is no assurance that any funds raised will be sufficient to enable us to attain profitable operations.

To the extent that we are unsuccessful raising sufficient capital, we may need to curtail or cease our operations and implement a plan to extend payables or reduce overhead until sufficient additional capital is raised to support further operations. There can be no assurance that such a plan will be successful. If adequate funds are not available, we may be required to curtail our operations significantly or to obtain funds on unfavorable terms, through dilutive financings or entering into arrangements with collaborative partners or others that may require us to relinquish rights to certain of our product candidates that we would not otherwise relinquish. If we issue equity or convertible debt securities to raise additional funds, our existing stockholders will experience further dilution,

and the new equity or debt securities may have rights, preferences and privileges senior to those of our existing stockholders. If we incur debt, our fixed payment obligations, liabilities and leverage relative to our equity capitalization would increase, which could increase the cost of future capital. Further, the terms of any debt securities we issue or borrowings we incur, if available, could impose significant restrictions on our operations, such as limitations on our ability to incur additional debt or issue additional equity or other operating restrictions that could adversely affect our ability to conduct our business, and any such debt could be secured by any or all of our assets pledged as collateral. Additionally, we may incur substantial costs in pursuing future capital, including investment banking, legal and accounting fees, printing and distribution expenses and other costs.

We have just begun to generate product revenue and may not become profitable in the near term or maintain profitability once achieved.

We began to generate revenue from the commercialization of VYKAT XR in the three months ended June 30, 2025. Our ability to generate significant revenue from product sales and achieve profitability will depend upon our ability, alone or with any future collaborators, to successfully commercialize products that we may develop, in-license or acquire in the future. Our ability to generate revenue from product sales from VYKAT XR also depends on a number of additional factors, including our ability to:

- develop a commercial organization capable of sales, marketing and distribution of VYKAT XR;
- achieve market acceptance of VYKAT XR;
- set a commercially viable price for VYKAT XR;
- establish and maintain supply and manufacturing relationships with reliable third parties, and ensure adequate and legally compliant manufacturing to maintain that supply;
- obtain coverage and adequate reimbursement from third-party payers, including government and private payers;
- find suitable distribution partners to help us market, sell and distribute VYKAT XR in other markets;
- complete and submit applications to, and obtain regulatory approval from, foreign regulatory authorities;
- complete development activities successfully and on a timely basis;
- establish, maintain and enforce our intellectual property rights and avoid third-party patent interference, intellectual property challenges, including any potential challenges to our Orange Book patent listings in the U.S., or intellectual property infringement claims; and
- attract, hire and retain qualified personnel.

In addition, because of the numerous risks and uncertainties associated with product development and commercialization, including that our planned products may not advance through development, achieve the endpoints of applicable clinical trials or obtain approval, we are unable to predict the timing or amount of increased expenses, or when or if we will be able to achieve or maintain profitability. In addition, our expenses could increase beyond expectations if we decide, or are required by the FDA or foreign regulatory authorities, to perform studies or clinical trials in addition to those that we currently anticipate.

Even if we are able to generate significant revenue from the sale of any of our products that may be approved or commercialized, we may not become profitable and may need to obtain additional funding to continue operations. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or shut down our operations.

We have a significant amount of debt, which may affect our ability to operate our business and secure additional financing in the future.

As of June 30, 2025, we had $50.0 million outstanding under our loan and security agreement with Oxford Financing LLC and its affiliates (collectively, Oxford). Under the terms of the loan agreement with Oxford, following FDA approval of VYKAT XR, an additional $50 million is available through September 30, 2025 and $25 million will become available during the period October 1, 2025 to September 30, 2026. One additional tranche of $25 million may become available upon achievement of certain commercial milestones. A final $50 million may be made available upon mutual consent with Oxford. The loan carries an interest-only period of 48 months and a total term of 60 months; provided that if specific milestones are achieved prior to September 30, 2026, the interest-only period and maturity date will be extended by 12 months. Subject to certain conditions, the term loans accrue interest at a floating rate equal to (a) 1-month term SOFR plus (b) 5.50%. Our debt with Oxford is collateralized by substantially all of our assets and contains customary financial and operating covenants limiting our ability to, among other things, dispose of assets, undergo a change in control, merge or consolidate, enter into certain transactions with affiliates, make acquisitions, incur debt, incur liens, pay dividends, repurchase stock and make investments, in each case subject to certain exceptions. The covenants in our loan agreement with Oxford, as well as in any future financing agreements into which we may enter, may restrict our ability to finance our operations and engage in, expand or otherwise pursue our business activities and strategies. Our ability to comply with these covenants may be

affected by events beyond our control and future breaches of any of these covenants could result in a default under the loan agreement. If not waived, future defaults could cause all of the outstanding indebtedness under the loan agreement to become immediately due and payable and terminate commitments to extend further credit. If we do not have or are unable to generate sufficient cash to repay our debt obligations when they become due and payable, either upon maturity or in the event of a default, we may not be able to obtain additional debt or equity financing on favorable terms, if at all, which may negatively impact our ability to operate and continue our business as a going concern.

Our operating results may fluctuate significantly, which makes our future operating results difficult to predict and could cause our operating results to fall below expectations or below our guidance.

Our quarterly and annual operating results may fluctuate significantly in the future, which makes it difficult for us to predict our future operating results. In addition to the risks related to a company launching its first commercial drug described elsewhere in this "Risk Factors" section, the success of a new drug product is inherently difficult to predict and we may not recognize revenue as quickly, consistently or in the amounts that we, analysts or investors anticipate.

Additionally, from time to time, we may enter into collaboration agreements with other companies that include development funding and significant upfront and milestone payments or royalties, which may become an important source of our revenue. Accordingly, our revenue may depend on development funding and the achievement of development and clinical milestones under any potential future collaboration and license agreements and sales of our products, if approved. These upfront and milestone payments may vary significantly from period to period, and any such variance could cause a significant fluctuation in our operating results from one period to the next.

In addition, we measure compensation cost for stock-based awards made to employees at the grant date of the award, based on the fair value of the award as determined by our Board, and recognize the cost as an expense over the employee's requisite service period. As the variables that we use as a basis for valuing these awards change over time, including our underlying stock price and stock price volatility, the magnitude of the expense that we must recognize may vary significantly. Furthermore, our operating results may fluctuate due to a variety of other factors, many of which are outside of our control and may be difficult to predict, including the following:

- the cost and risk of initiating sales and marketing activities;
- the cost of manufacturing our products may vary depending on FDA and other regulatory requirements, the quantity of production and the terms of our agreements with manufacturers;
- expenditures that we will or may incur to acquire or develop additional planned products and technologies;
- changes in the competitive landscape of our industry, including consolidation among our competitors or potential partners;
- the level of demand for our products may fluctuate significantly and be difficult to predict;
- the risk/benefit profile, cost and reimbursement policies with respect to our future products, if approved, and existing and potential future drugs that compete with our planned products;
- competition from existing and potential future offerings that compete with our products;
- our ability to commercialize our products inside and outside of the U.S., either independently or working with third parties;
- our ability to enroll patients in clinical trials and the timing of enrollment;
- the design, timing and outcomes of clinical trials;
- any delays in regulatory review or approval in the U.S. or globally, of any of our planned products;
- the timing and cost of, and level of investment in, research and development activities relating to our planned products, which will change from time to time;
- our ability to establish and maintain collaborations, licensing or other arrangements;
- our ability to adequately support future growth;
- potential unforeseen business disruptions that increase our costs or expenses;
- future accounting pronouncements or changes in our accounting policies; and
- the changing and volatile global economic environment.

The cumulative effects of these factors could result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, comparing our operating results on a period-to-period basis may not be meaningful. Investors should not

rely on our past results as an indication of our future performance. This variability and unpredictability could also result in our failing to meet the expectations of industry or financial analysts or investors for any period. If our revenue or operating results fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if the forecasts we provide to the market are below the expectations of analysts or investors, the price of our common stock could decline substantially. Such a stock price decline could occur even when we have met any previously publicly stated revenue or earnings guidance we may provide.

We may engage in strategic transactions that could impact our liquidity, increase our expenses and present significant distractions to our management.

From time to time we may consider strategic transactions, such as acquisitions, asset purchases and sales, and out-licensing or in-licensing of products, product candidates or technologies. Additional potential transactions that we may consider include a variety of different business arrangements, including spin-offs, strategic partnerships, joint ventures, restructurings, divestitures, business combinations and investments. Any such transaction may require us to incur non-recurring or other charges, may increase our near and long-term expenditures, could not result in perceived benefits that were contemplated upon entering into the transaction, and may pose significant integration challenges or disrupt our management or business, which could adversely affect our operations, solvency and financial results. For example, these transactions may entail numerous operational and financial risks, including:

- exposure to unknown and contingent liabilities;
- disruption of our business and diversion of our management's time and attention in order to develop acquired products, product candidates or technologies;
- incurrence of substantial debt or dilutive issuances of equity securities to pay for acquisitions;
- higher than expected acquisition and integration costs;
- the timing and likelihood of payment of milestones or royalties;
- write-downs of assets or goodwill or impairment charges;
- increased operating expenditures, including additional research, development and sales and marketing expenses;
- increased amortization expenses;
- difficulty and cost in combining the operations and personnel of any acquired businesses with our operations and personnel; and
- impairment of relationships with key suppliers or customers of any acquired businesses due to changes in management and ownership.

Accordingly, although there can be no assurance that we will undertake or successfully complete any additional transactions of the nature described above or that we will achieve an economic benefit that justifies such transactions, any additional transactions that we do complete could have a material adverse effect on our business, results of operations, financial condition and prospects.

We may not be able to enter into strategic transactions on a timely basis or on acceptable terms, which may impact our development and commercialization plans.

We have relied, and expect to continue to rely, on strategic transactions, which include in-licensing, out-licensing, purchases and sales of assets, and other ventures. The terms of any additional strategic transaction that we may enter into may not be favorable to us, and the contracts governing such strategic transaction may be subject to differing interpretations exposing us to potential litigation. We may also be restricted under existing collaboration or licensing arrangements from entering into future agreements on certain terms with potential strategic partners. We may not be able to negotiate additional strategic transactions on a timely basis, on acceptable terms, or at all. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our products or bring them to market and generate product revenue. Furthermore, there is no assurance that any such transaction will be successful or that we will derive an economic benefit as a result.

Risks related to the commercialization of VYKAT XR

VYKAT XR may fail to achieve the degree of market acceptance by physicians, patients, caregivers, healthcare payers and others in the medical community necessary for commercial success.

VYKAT XR may fail to gain sufficient market acceptance by physicians, hospital administrators, patients, healthcare payers and others in the medical community. The degree of market acceptance of VYKAT XR will depend on a number of factors, including the following:

- the incidence and severity of any side effects;
- its effectiveness and potential advantages compared to alternative treatments;
- the price we charge for VYKAT XR;
- the willingness of physicians to change their current treatment practices;
- convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- patients' or caregivers' perception of the efficacy of VYKAT XR and interest in remaining on-drug long-term;
- the strength or effectiveness of marketing and distribution support of partners; and
- the availability of third-party coverage or reimbursement.

If the market opportunity for VYKAT XR is smaller than we believe it is, then our revenues may be adversely affected, and our business may suffer.

PWS is a rare disease, and as such, our projections of both the number of people who have this disease, as well as the subset of people with PWS who could be prescribed VYKAT XR, are estimates based on data analysis of the reported patient population. If our estimates of the prevalence of PWS, the number of patients who may benefit from treatment with VYKAT XR, or the number of patients willing to stay on treatment indefinitely prove to be incorrect, the market opportunity for VYKAT XR may be smaller than we believe it is, our prospects for generating revenue may be adversely affected and our business may suffer.

If we are unable to execute our sales and marketing strategy for VYKAT XR or are unable to gain acceptance in the market, we may be unable to generate sufficient revenue to sustain our business.

Although we believe that VYKAT XR represents a promising commercial opportunity, VYKAT XR may never gain significant acceptance in the marketplace and therefore may never generate substantial revenue or profits for us. We will need to establish a market for VYKAT XR globally and build these markets through physician education, awareness programs, and other marketing efforts. Gaining acceptance in medical communities depends on a variety of factors, including clinical data published or reported in reputable contexts, the provisions of the approved label for VYKAT XR, and word-of-mouth between physicians. The process of publication in leading medical journals is subject to a peer review process and peer reviewers may not consider the results of our studies sufficiently novel or worthy of publication. Failure to have our trials published in peer-reviewed journals may limit the adoption of our products. Our ability to successfully market our products will depend on numerous factors, including:

- the outcomes of clinical utility trials of such products in collaboration with key thought leaders to demonstrate our products' value in informing important medical decisions such as treatment selection;
- the success of our distribution partners;
- whether healthcare providers believe VYKAT XR provides clinical utility; and
- whether health insurers, government health programs and other payers will cover and pay for VYKAT XR and, if so, whether they will adequately reimburse us.

We may rely on third parties, who we do not control, to distribute and sell VYKAT XR. If these distributors are not committed to VYKAT XR or otherwise run into their own financial or other difficulties, it may result in failure to achieve widespread market acceptance of VYKAT XR, and would materially harm our business, financial condition and results of operations.

If we are unable to implement our sales, marketing, distribution, training and support strategies or enter into agreements with third parties to perform these functions, we will not be able to effectively commercialize VYKAT XR and may not reach profitability.

To achieve commercial success for VYKAT XR, we will need to establish a robust sales and marketing organization. We have built a targeted sales, marketing, training and support infrastructure to market VYKAT XR in the U.S. and to establish relationships with collaborations to market, distribute and support VYKAT XR outside of the U.S. There are risks involved with establishing our own sales, marketing, distribution, training and support capabilities. For example, recruiting and training sales and marketing personnel is expensive and time-consuming and could impact any product launch. Additionally, commercialization of therapeutic products is subject to a variety of regulations regarding the manner in which potential customers may be engaged, the manner in which products may be lawfully advertised, and the claims that can be made for the benefits of the product, among other things. Our lack of experience with product launches may expose us to a higher than usual level of risk of non-compliance with these regulations, with consequences that may include fines or the removal of our approved products from the marketplace by regulatory authorities.

Factors that may inhibit our efforts to commercialize VYKAT XR on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to or persuade adequate numbers of physicians to prescribe VYKAT XR or any future products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines;
- unforeseen costs and expenses associated with creating an independent sales and marketing organization; and
- efforts by our competitors to commercialize products at or about the time when our product candidates would be coming to market.

If we are unable to successfully establish our own sales, marketing, distribution, training and support capabilities and instead enter into arrangements with third parties to perform these services, our product revenues and our profitability, if any, are likely to be lower than if we were to market, sell and distribute VYKAT XR ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute VYKAT XR or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to commercialize VYKAT XR effectively. If we do not establish sales, marketing, distribution, training and support capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing VYKAT XR and achieving profitability, and our business would be harmed.

If physicians decide not to prescribe VYKAT XR, we may be unable to generate sufficient revenue to sustain our business.

To generate demand for VYKAT XR, we will need to educate physicians and other health care professionals on the clinical utility, benefits and value of the tests we provide through published papers, presentations at scientific conferences, educational programs and one-on-one education sessions by members of our sales force. In addition, we will need support of physicians, hospital administrators, patients, healthcare payers and others in the medical community that the clinical and economic utility of our products justifies payment for VYKAT XR at adequate pricing levels. We will need to hire additional commercial, scientific, technical and other personnel to support this process.

We may attempt to form partnerships with respect to VYKAT XR, but we may not be able to do so, which may cause us to alter our development and commercialization plans and may cause us to terminate any such programs.

We may form strategic alliances, create joint ventures or collaborations, or enter into licensing agreements with third parties that we believe will more effectively provide resources to develop and commercialize VYKAT XR.

If we attempt to seek appropriate strategic partners, we may face significant competition, and the negotiation process to secure favorable terms is time-consuming and complex. We may not be successful in our efforts to establish such a strategic partnership for any future products and programs on terms that are acceptable to us, or at all.

Any delays in identifying suitable collaborators and entering into agreements to develop or commercialize VYKAT XR could negatively impact the development or commercialization of our products, particularly in geographic regions like the Europe, where we do not currently have development and commercialization infrastructure. Absent a partner or collaborator, we would need to undertake development or commercialization activities at our own expense. If we elect to fund and undertake development and commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we are unable to do so, we may not be able to develop our products or bring them to market, and our business may be materially and adversely affected.

VYKAT XR may cause serious adverse side effects or have other properties that could limit the commercial desirability or result in significant negative consequences following commercialization.

Undesirable side effects caused by VYKAT XR could result in a number of potentially significant negative consequences could result, including:

- we may be forced to recall VYKAT XR and suspend the marketing of VYKAT XR;
- regulatory authorities may withdraw their approvals of VYKAT XR;
- regulatory authorities may require additional warnings on the label that could diminish the usage or otherwise limit the commercial success of VYKAT XR;
- the FDA or other regulatory bodies may issue safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warnings about VYKAT XR;

- the FDA may require the establishment of Risk Evaluation Mitigation Strategy or a comparable foreign regulatory authority may require a similar strategy that may, for instance, restrict distribution of our products and impose burdensome implementation requirements on us;
- we may be required to change the way VYKAT XR is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to subjects or patients;
- we may be subject to litigation or product liability claims; or
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of VYKAT XR. We currently hold $10.0 million in product liability insurance coverage, which may not be adequate to cover all liabilities that we may incur. 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.

We face competition, which may result in others discovering, developing or commercializing products more successfully than we do.

Alternatives product candidates are being developed by our competitors and we will likely face competition with respect to any planned products that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies, medical device companies, and biotechnology companies worldwide. These companies may reduce prices for their competing drugs in an effort to gain or retain market share and undermine the value our products might otherwise be able to offer to payers. 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. Many of these competitors are attempting to develop therapeutics for our target indications.

Smaller or 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 technical and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

There has recently been increased activity in the development of drugs to treat the symptoms of PWS. We are aware of at least nine other current or proposed clinical trials evaluating PWS therapies, including with glucagon-like peptide-1 (GLP-1) receptors in patients with PWS.

Even if we are able to engage partners in commercializing VYKAT XR, they may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, thereby harming our business.

The regulations that govern marketing approvals, pricing and reimbursement for new products vary widely from country to country. Some countries require approval of the sale price of a product before it can be marketed. In many countries, the pricing review period begins after marketing approval is granted. In some foreign markets, pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain regulatory approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product and negatively impact the revenue we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more planned products, even if our planned products obtain regulatory approval.

Our ability to commercialize our products successfully also will depend in part on the extent to which reimbursement for these products and related treatments becomes available from government health administration authorities, private health insurers and other organizations. Government authorities and third-party payers, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and these third-party payers have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. We cannot be sure that reimbursement will be available for any product that we commercialize and, if reimbursement is available, what the level of reimbursement will be. Reimbursement may impact the demand for, or the price of, any product for which we obtain marketing approval. Obtaining reimbursement for our products may be particularly difficult because of the higher prices often associated with products administered under the supervision of a physician. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize our products.

In the U.S., eligibility for reimbursement does not imply that any product will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim payments for new products, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Payment rates may vary according to the use of the product and the clinical setting in which it is used, may be based on payments allowed for lower cost products that are already reimbursed and may be incorporated into existing payments for other services. Net prices for products may be reduced by mandatory discounts or rebates

required by government healthcare programs or private payers and by any future relaxation of laws that presently restrict imports of products from countries where they may be sold at lower prices than in the U.S. Third-party payers often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies.

Our inability to promptly obtain coverage and profitable payment rates from both government funded and private payers for new products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition. In some foreign countries, including major markets in Europe and Japan, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take longer than twelve months after the receipt of regulatory marketing approval for a product in many cases. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product to other available therapies. Our business could be materially harmed if reimbursement of our products, if any, is unavailable or limited in scope or amount or if pricing is set at unsatisfactory levels.

International expansion of our business will expose us to business, regulatory, political, operational, financial and economic risks associated with doing business outside of the U.S.

Our business strategy contemplates international expansion, including partnering with distributors, and introducing VYKAT XR and other planned products outside the U.S. In May 2025, we announced that our E.U. marketing authorization application (MAA) for obtaining regulatory approval of diazoxide choline prolonged-release tablets (which we market in the U.S. as VYKAT XR) for the treatment of adults and children four years and older with PWS who have hyperphagia had been validated by the European Medicines Agency (EMA). If we are successful in obtaining E.U. marketing authorization, the expansion of our commercial activities in the E.U. will increase our compliance costs and exposure to potential liabilities under E.U. laws. Additionally, doing business internationally involves a number of risks, including:

- multiple, conflicting and changing laws and regulations such as tax laws, export and import restrictions, tariffs, employment laws, regulatory requirements and other governmental approvals, permits and licenses;
- potential failure by us or our distributors to obtain regulatory approvals for the sale or use of our current products and our planned future products in various countries;
- difficulties in managing foreign operations, including hiring and maintaining a foreign employee base;
- complexities associated with managing government payer systems, multiple payer-reimbursement regimes or self-pay systems;
- logistics and regulations associated with shipping products, including infrastructure conditions and transportation delays;
- limits on our ability to penetrate international markets if our distributors do not execute successfully;
- financial risks, such as longer payment cycles, difficulty enforcing contracts and collecting accounts receivable, and exposure to foreign currency exchange rate fluctuations;
- reduced protection for intellectual property rights, or lack of them in certain jurisdictions, forcing more reliance on our trade secrets, if available;
- natural disasters, political and economic instability, including wars, terrorism and political unrest, including the outbreak of hostilities in the Ukraine and the Middle East, outbreak of disease, boycotts, curtailment of trade and other business restrictions; and
- failure to comply with the Foreign Corrupt Practices Act, including its books and records provisions and its anti-bribery provisions, by maintaining accurate information and control over sales activities and distributors' activities.

Any of these risks, if encountered, could significantly harm our future international expansion and operations and, consequently, have a material adverse effect on our financial condition, results of operations and cash flows.

Risks related to the operation of our business

We expect to continue to expand our development, regulatory and sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

As of June 30, 2025, we had 133 employees, up from 92 and 33 full-time employees as of December 31, 2024 and December 31, 2023, respectively. We have experienced significant growth in the number of our employees and the scope of our operations, particularly in the areas of sales and marketing, quality assurance, product development, regulatory affairs and drug development. To manage this growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such growth, we may not be able to effectively manage the

expansion of our operations or recruit and train additional qualified personnel. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Future growth would impose significant added responsibilities on members of management, including:

- identifying, recruiting, maintaining, motivating and integrating additional employees with the expertise and experience we will require;
- managing our internal development efforts effectively while complying with our contractual obligations to licensors, licensees, contractors and other third parties;
- managing additional relationships with various strategic partners, suppliers and other third parties;
- improving our managerial, development, operational and finance reporting systems and procedures;
- managing our clinical trials effectively, which we anticipate being conducted at numerous clinical sites; and
- expanding our facilities.

Our failure to accomplish any of these tasks could prevent us from successfully growing. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

The loss of key members of our executive management team could adversely affect our business.

Our success in implementing our business strategy depends largely on the skills, experience and performance of key members of our executive management team and others in key management positions. The collective efforts of each of these persons, and others working with them as a team, are critical to us as we continue to develop our technologies, tests and research and development and sales programs. As a result of the difficulty in locating qualified new management, the loss or incapacity of existing members of our executive management team could adversely affect our operations. If we were to lose one or more of these key employees, we could experience difficulties in finding qualified successors, competing effectively, developing our technologies and implementing our business strategy. Our executive officers all have employment agreements; however, the existence of an employment agreement does not guarantee retention of members of our executive management team and we may not be able to retain those individuals for the duration of or beyond the end of their respective terms. We do not currently maintain “key person” life insurance on any of our employees.

In addition, we rely on collaborators, consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our collaborators, consultants and advisors are generally employed by employers other than us and may have commitments under agreements with other entities that may limit their availability to us.

There is a scarcity of experienced professionals in our industry. If we are not able to retain and recruit personnel with the requisite technical skills, we may be unable to successfully execute our business strategy.

The specialized nature of our industry results in an inherent scarcity of experienced personnel in the field. Our future success depends upon our ability to attract and retain highly skilled personnel, including scientific, technical, commercial, business, regulatory and administrative personnel, necessary to support our anticipated growth, develop our business and perform certain contractual obligations. Given the scarcity of professionals with the scientific knowledge that we require and the competition for qualified personnel among biotechnology businesses, we may not succeed in attracting or retaining the personnel we require to continue and grow our operations.

Any future development or commercialization agreements we may enter into for our products may place the development or distribution of these products outside our control, may require us to relinquish important rights, or may otherwise be on terms unfavorable to us.

We may enter into distribution or commercialization agreements with third parties with respect to our products. Our likely collaborators for any distribution, marketing, licensing or other collaboration arrangements include large and mid-size companies, regional and national companies, and distribution or group purchasing organizations. We will have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our products. Our ability to generate revenue from these arrangements will depend in part on our collaborators’ abilities to successfully perform the functions assigned to them in these arrangements.

Collaborations involving our products are subject to numerous risks, which may include the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to any such collaborations;
- collaborators may not pursue development and commercialization of our products, or may elect not to continue or renew efforts based on clinical trial results, changes in their strategic focus for a variety of reasons, potentially including the

acquisition of competitive products, availability of funding, and mergers or acquisitions that divert resources or create competing priorities;

- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial, abandon a product, repeat or conduct new clinical trials or require a new engineering iteration of a product for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products;
- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to their marketing and distribution;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;
- disputes may arise between us and a collaborator that causes the delay or termination of the research, development or commercialization of our products or that results in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable products; and
- collaborators may own or co-own intellectual property covering our products that results from our collaborating with them, and in such cases, we would not have the exclusive right to commercialize such intellectual property.

Any termination or disruption of collaborations could result in delays in the development of products, increases in our costs to develop the products or the termination of development of a product.

Because we intend to do business outside the U.S., we will be subject to additional risks.

A variety of risks associated with international operations could materially adversely affect our business, including:

- different regulatory requirements for drug approvals in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade wars, trade barriers, trade restrictions, export or import sanctions, 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 taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the U.S.;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism, including the outbreak of hostilities in the Ukraine, the Middle East, outbreaks of disease, or natural disasters including earthquakes, typhoons, floods and fires.

In particular, there is currently significant uncertainty about the future relationship between the United States and various other countries with respect to trade policies, treaties, tariffs, taxes and other limitations on cross-border operations. Since beginning his second term in office, President Trump has made and continues to make significant additional changes in U.S. trade policy and may continue to take future actions that could negatively impact U.S. trade. Such trade policies and tariff implementations, and any related retaliatory trade policies and tariff implementations by foreign governments, may result in any materials that we import to the U.S. from countries subject to tariffs becoming more expensive or increase the price of VYKAT XR in other countries, which could have a material adverse impact on our business, financial condition and results of operations. We cannot predict what actions may ultimately be taken with respect to trade relations between the United States and other countries, what products and services may be subject to such actions or what actions may be taken by the other countries in retaliation. If we are unable to obtain or use services from existing

service providers or become unable to export or sell our products to any of our customers or service providers, our business, liquidity, financial condition, and results of operations would be materially and adversely affected.

We have incurred and will continue to incur significant increased costs as a result of operating as a public company, and our management has devoted and will be required to continue to devote substantial time to new compliance initiatives.

We have incurred and will continue to incur significant legal, accounting and other expenses as a public company. We are subject to the reporting requirements of the Securities Exchange Act of 1934, as amended, the other rules and regulations of the SEC, and the rules and regulations of Nasdaq. The expenses of being a public company are material, and compliance with the various reporting and other requirements applicable to public companies requires considerable time and attention of management. For example, the Sarbanes-Oxley Act (SOX) and the rules of the SEC and national securities exchanges have imposed various requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls. Our management and other personnel need to devote a substantial amount of time to these compliance initiatives. These rules and regulations will continue to increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, these rules and regulations may make it difficult and expensive for us to obtain adequate director and officer liability insurance, and we may be required to accept reduced policy limits on coverage or incur substantial costs to maintain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified personnel to serve on our Board, our Board committees, or as executive officers.

SOX requires, among other things, that we maintain effective internal control over financial reporting and disclosure controls and procedures. In particular, we must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting, as required by Section 404 of SOX (Section 404). We currently do not have an internal audit group, and we will need to hire additional accounting and financial staff with appropriate public company experience and technical accounting knowledge.

If we are not able to comply with the requirements of Section 404 in a timely manner the market price of our stock could decline and we could be subject to sanctions or investigations by Nasdaq, the SEC or other regulatory authorities, which would require additional financial and management resources. Our ability to successfully implement our business plan and comply with Section 404 requires us to be able to prepare timely and accurate financial statements. We expect that we will need to continue to improve existing, and implement new operational and financial systems, procedures and controls to manage our business effectively. Any delay in the implementation of, or disruption in the transition to, new or enhanced systems, procedures or controls, may cause our operations to suffer and we may be unable to conclude that our internal control over financial reporting is effective. This, in turn, could have an adverse impact on trading prices for our common stock, and could adversely affect our ability to access the capital markets.

We are required by Section 404 to evaluate the effectiveness of our internal control over financial reporting. If we are unable to achieve and maintain effective internal controls, our operating results and financial condition could be harmed and the market price of our common stock may be negatively affected.

As a public company with SEC reporting obligations, we are required to document and test our internal control procedures to satisfy the requirements of Section 404, which requires annual assessments by management of the effectiveness of our internal control over financial reporting. Effective December 31, 2024, we no longer qualify as a smaller reporting company and the reduced compliance requirements to smaller reporting companies no longer apply to us. As such, our auditor was required to attest to the effectiveness of our internal control over financial reporting beginning with our annual report on Form 10-K for the year ended December 31, 2024. We must implement and maintain substantial internal control systems and procedures to satisfy the reporting requirements under the Securities Exchange Act of 1934. During our assessments, we may identify deficiencies that we are unable to remediate in a timely manner. Testing and maintaining our internal control over financial reporting may also divert management's attention from other matters that are important to the operation of our business. We may not be able to conclude on an ongoing basis that we have effective internal control over financial reporting in accordance with Section 404. If we conclude that our internal control over financial reporting is not effective, the cost and scope of remediation actions and their effect on our operations may be significant. Moreover, any material weaknesses or other deficiencies in our internal control over financial reporting may impede our ability to file timely and accurate reports with the SEC. Any of the above could cause investors to lose confidence in our reported financial information or our common stock listing on Nasdaq to be suspended or terminated, which could have a negative effect on the trading price of our common stock.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results in a timely manner or prevent fraud, which would harm our business.

Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation, could cause us to fail to meet our reporting obligations in a timely manner, or at all. In addition, any testing by us conducted in connection with Section 404 or any subsequent testing by our independent registered public accounting firm in connection with Section 404, may reveal deficiencies in our internal controls over

financial reporting that are deemed to be significant deficiencies or material weaknesses or that may require prospective or retroactive changes to our consolidated financial statements or identify other areas for further attention or improvement. We are also required to disclose material changes made in our internal controls over financing reporting and procedures on a quarterly basis and our management is required to assess the effectiveness of these controls annually. Ineffective internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our common stock. To achieve compliance with Section 404 within the prescribed period, we have engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a plan to assess and document the adequacy of our internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are designed and operating effectively and implement a continuous reporting and improvement process for internal control over financial reporting. An independent assessment of the effectiveness of our internal controls could detect problems that our management's assessment might not identify. Undetected material weaknesses in our internal controls could lead to financial statement restatements and require us to incur the expense of remediation.

If our estimates or judgments relating to our critical accounting policies are based on assumptions that change or prove to be incorrect, our results of operation could fall below our publicly announced guidance or the expectations of securities analysts and investors, resulting in a decline in the market price of our common stock.

The preparation of consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the amounts reported in our financial statements and accompanying notes. We base our estimates on historical experience and estimates and on various other assumptions that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets, liabilities, equity, revenue and expenses that are not readily apparent from other sources. For example, in connection with the implementation of the new revenue accounting standard if and when we have product sales, management makes judgments and assumptions based on our interpretation of the new standard. The new revenue standard is principle-based and interpretation of those principles may vary from company to company based on their unique circumstances. It is possible that interpretation, industry practice and guidance may evolve as we apply the new standard. If our assumptions underlying our estimates and judgments relating to our critical accounting policies change or if actual circumstances differ from our assumptions, estimates or judgments, our operating results may be adversely affected and could fall below our publicly announced guidance or the expectations of securities analysts and investors, resulting in a decline in the market price of our common stock.

In the future we may identify additional material weaknesses, fail to remediate our current material weakness or otherwise fail to maintain an effective system of internal controls, which may result in material misstatements of our financial statements or cause us to fail to meet our periodic reporting obligations.

As discussed in Item 4 of this Quarterly Report on Form 10-Q, in preparing our consolidated financial statements as of and for the year ended December 31, 2024, our management concluded that our disclosure controls and procedures and our internal control over financial reporting were not effective at the reasonable assurance level. To address this material weakness, we plan to take actions designed to improve our internal control over financial reporting and remediate the control deficiencies that led to the material weakness. If we discover additional weaknesses in our system of internal financial and accounting controls and procedures, our consolidated financial statements may contain material misstatements, and we could be required to restate our financial results. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

Any failure to implement and maintain effective internal control over financial reporting could cause investors to lose confidence in our reported financial and other information, adversely impact our stock price, cause us to incur increased costs to remediate any deficiencies, and attract regulatory scrutiny or lawsuits that could be costly to resolve and distract management's attention, limit our ability to access the capital markets, or cause our stock to be delisted from The Nasdaq Capital Market or any other securities exchange on which it is then listed. 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.

If we experience an interruption in supply from a material sole source supplier, our business may be harmed.

We do not currently have nor do we plan to acquire the infrastructure or capability internally to manufacture or distribute VYKAT XR. We are currently dependent on sole source suppliers to produce raw materials, active pharmaceutical ingredients (APIs), the finished drug product and the associated packaging for VYKAT XR. If there is an interruption in supply from these sole source suppliers, for any reason, there can be no assurance that we will be able to obtain adequate quantities of the raw materials within a reasonable time or at commercially reasonable prices. Interruptions in supplies due to pricing, timing, availability, or other issues with

our sole source suppliers could have a negative impact on our contract manufacturer's ability to manufacture VYKAT XR, which in turn could adversely affect the commercialization of VYKAT XR.

Regardless of whether a sole source supplier enters into a written supply arrangement with us, such supplier could still delay, suspend, or terminate supply of raw materials to us for a number of reasons, including manufacturing or quality issues, payment disputes with us, or bankruptcy or insolvency situations.

Manufacturing or quality assurance difficulties at our contractors and suppliers, the failure or refusal of a supplier or contract manufacturer to supply contracted quantities, or increases in demand on a supplier with constrained capacity could result in delays and disruptions in the manufacturing, distribution, and sale of VYKAT XR, leading to lost revenue or reduced market opportunities. Supply constraints may also lead to pauses, discontinuations, or other product availability issues, which could have a material adverse effect on our consolidated results of operations and cash flows. Further, cost inflation and transportation and logistics challenges in the future may cause delays in, and increase costs related to, procurement activity, and supplier or contract manufacturer arrangements.

If a sole source supplier ceases supply of drug substance, drug product or labeled finished product, there is no guarantee that we will find an alternative supplier on terms acceptable to us, or at all. Finding alternative suppliers may not be feasible or could take a significant amount of time and involve significant expense due to the nature of VYKAT XR. Further the qualification process for a new vendor could take months or years, and any such delay in qualification could significantly harm our business.

In the United States, we intend to rely on one or more specialty pharmacies to dispense VYKAT XR, deliver customer support, and provide us with related services, and our business could be harmed and we could be subject to liabilities if these services are performed inadequately or in a manner that does not comply with applicable laws and regulations.

Our VYKAT XR sales in the United States will initially be through a single specialty pharmacy and therefore our sales of VYKAT XR will be highly dependent on the performance of this specialty pharmacy. A specialty pharmacy is a pharmacy that specializes in the dispensing of medications which often require a high level of patient education and ongoing management. The use of a specialty pharmacy involves risks, including, but not limited to, risks that a specialty pharmacy:

- does not effectively dispense or support VYKAT XR;
- fails to properly administer copay mitigation programs;
- does not provide us with accurate or timely information regarding their inventories or the number of patients who are using VYKAT XR;
- fails to provide timely and accurate information regarding product adverse events or product complaints;
- does not devote the resources necessary to dispense VYKAT XR in a manner that meets patient needs;
- is unable to satisfy financial obligations to us or others;
- loses the required licenses to distribute VYKAT XR; or
- ceases operations.

If a specialty pharmacy partner does not fulfill its contractual obligations to us or fails to adequately dispense VYKAT XR and deliver customer support, our product sales and business could be harmed or we could be subject to legal or regulatory liabilities or sanctions. Furthermore, arrangements between manufacturers and specialty pharmacies can be subject to government scrutiny and challenge under fraud and abuse laws if not structured properly.

We rely on third parties to conduct certain components of our clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such studies.

We rely on third parties, such as contract research organizations, investigational product packaging, labeling and distribution, laboratories, medical institutions and clinical investigators and staff, to perform various functions for our clinical trials. Our reliance on these third parties for clinical development activities reduces our control over these activities but does not relieve us of our responsibilities. We remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the study. Moreover, the FDA requires us and third parties involved in the set-up, conduct, analysis and reporting of the clinical trials to comply with regulations and with standards, commonly referred to as good clinical practices (GCPs), to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of patients in clinical trials are protected. Our clinical investigators are also required to comply with GCPs. Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, regulatory approvals for our planned products and will not be able to, or may be delayed in our efforts to, successfully commercialize our planned products.

If we use biological and hazardous materials in a manner that causes injury, we could be liable for damages.

Our manufacturing processes currently require the controlled use of potentially harmful chemicals. We cannot eliminate the risk of accidental contamination or injury to employees or third parties from the use, storage, handling or disposal of these materials. In the event of contamination or injury, we could be held liable for any resulting damages, and any liability could exceed our resources or any applicable insurance coverage we may have. Additionally, we are subject to, on an ongoing basis, federal, state and local laws and regulations governing the use, storage, handling and disposal of these materials and specified waste products. The cost of compliance with these laws and regulations may become significant and could have a material adverse effect on our financial condition, results of operations and cash flows. In the event of an accident or if we otherwise fail to comply with applicable regulations, we could lose our permits or approvals or be held liable for damages or penalized with fines.

Risks related to intellectual property

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 material adverse effect on the success of our business.

Our commercial success depends in part on our avoiding infringement and other violations of the patents and proprietary rights of third parties. Patent and other intellectual property litigation is prevalent in our sectors. There is a substantial amount of litigation, both within and outside the U.S., involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, derivation and administrative law proceedings, inter partes review and post-grant review before the U.S. Patent and Trademark Office (USPTO), as well as oppositions and similar processes in foreign jurisdictions. Our commercial success depends upon our ability and the ability of our distributors, contract manufacturers, and suppliers to manufacture, market, and sell our planned products, and to use our proprietary technologies without infringing, misappropriating or otherwise violating the proprietary rights or intellectual property of third parties. As the biotechnology and pharmaceutical industries expand and more patents are issued, and as we gain greater visibility and market exposure, the risk increases that our commercialization of VYKAT XR or other business activities may be subject to claims of infringement of the patent and other proprietary rights of third parties. Third parties may assert that we are infringing their patents or employing their proprietary technology without authorization. We may become party to, or be threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our products. Third parties may assert infringement claims against us based on existing or future intellectual property rights. If we are found to infringe a third-party's intellectual property rights, we could be required to obtain a license from such third-party to continue developing and marketing our products. We may also elect to enter into such a license in order to settle pending or threatened litigation. 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 access to the same intellectual property licensed to us and could require us to pay significant royalties and other fees.

Also, there may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of VYKAT XR. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product may infringe.

In addition, third parties may obtain patent rights in the future and claim that use of our products infringes upon these rights. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of VYKAT XR, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize VYKAT XR unless we obtained a license under the applicable patents, or until such patents expire. Similarly, if any third-party patent were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patent may be able to block our ability to develop and commercialize our product unless we obtained a license or until such patent expires. In either case, such a license may not be available on commercially reasonable terms or at all. In addition, we may be subject to claims that we are infringing other intellectual property rights, such as trademarks or copyrights, or misappropriating the trade secrets of others, and to the extent that our employees, consultants or contractors use intellectual property or proprietary information owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

We could be forced, including by court order, to cease commercializing the infringing product. In addition, we could be found liable for monetary damages. A finding of infringement could prevent us from commercializing our planned products or force us to cease some of our business operations, which could materially harm our business. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful infringement or other intellectual property claim against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms.

Even if we are successful in defending against intellectual property claims, litigation or other legal proceedings relating to such claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal

responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce our resources available for development activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their substantially greater financial resources. Uncertainties resulting from the initiation and continuation of litigation or other intellectual property related proceedings could have a material adverse effect on our ability to compete in the marketplace.

Our ability to successfully commercialize our products may be materially adversely affected if we are unable to obtain and maintain effective intellectual property rights for our planned products, or if the scope of the intellectual property protection is not sufficiently broad.

Our success depends in large part on our ability to obtain and maintain patent and other intellectual property protection in the U.S. and in other countries with respect to our proprietary products.

The patent position of pharmaceutical companies generally is highly uncertain and involves complex legal and factual questions for which legal principles remain unresolved. In recent years patent rights have been the subject of significant litigation. As a result, the issuance, scope, validity, enforceability and commercial value of the patent rights we rely on are highly uncertain. Pending and future patent applications may not result in patents being issued which protect our products or which effectively prevent others from commercializing competitive products. The laws of foreign countries may not protect our rights to the same extent as the laws of the U.S. For example, many countries restrict the patentability of methods of treatment of the human body. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the U.S. and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in our patents or pending patent applications, or that we or were the first to file for patent protection of such inventions. As a result of these and other factors, 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 products, in whole or in part, or which effectively prevent others from commercializing competitive products. Changes in either the patent laws or interpretation of the patent laws in the U.S. and other countries may diminish the value of the patents we rely on or narrow the scope of our patent protection.

Moreover, we may be subject to a third-party pre-issuance submission of prior art to the USPTO or become involved in opposition, derivation, reexamination, inter partes review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. The costs of defending our patents or enforcing our proprietary rights in post-issuance administrative proceedings and litigation can be substantial and the outcome can be uncertain. 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 products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. 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 VYKAT XR.

Even if the patent applications we rely on issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. The issuance of a patent is not conclusive as to its scope, validity or enforceability, and the patents we rely on may be challenged in the courts or patent offices in the U.S. and abroad.

Such challenges may result in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop or prevent us from stopping others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Generally, issued patents are granted a term of 20 years from the earliest claimed non-provisional filing date. In certain instances, patent term can be adjusted to recapture a portion of delay by the USPTO in examining the patent application (patent term adjustment) or extended to account for term effectively lost as a result of the FDA regulatory review period (patent term extension), or both. The scope of patent protection may also be limited. Without patent protection for VYKAT XR, we may be open to competition from generic versions of VYKAT XR. Given the amount of time required for the development, testing and regulatory review of new planned products, patents protecting such products might expire before or shortly after such products are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours or otherwise provide us with a competitive advantage.

The scope, validity, enforceability, and commercial value of trademark rights are also uncertain. Pending and future trademark applications may not be successful.

We may become involved in legal proceedings to protect or enforce our intellectual property rights, which could be expensive, time-consuming, or unsuccessful.

Competitors may infringe or otherwise violate the patents we rely on, or our other intellectual property rights including trademarks. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming, and there can be no assurance that such efforts will be successful. Our ability to enforce our patents and other intellectual property rights also depends on the laws of individual countries and each country's practices regarding the enforcement of intellectual property rights and may also be impacted by regulatory actions taken by governmental authorities that affect our ability to use and maintain our intellectual property rights. The loss of patent protection or regulatory exclusivity on VYKAT XR or future products, including potential challenges to our Orange Book patent listings in the U.S., could materially impact our business, results of operations, financial condition and prospects. Any claims that we assert against perceived infringers could also provoke these parties to assert counterclaims against us alleging that we infringe their intellectual property rights. In addition, in an infringement proceeding, a court may decide that a patent we are asserting is invalid or unenforceable or may refuse to stop the other party from using the technology at issue on the grounds that the patents we are asserting do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing. In patent litigation in the U.S., 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, written description, or lack of patentable 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 U.S., 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 vaccine candidates. Such a loss of patent protection could harm our business.

We may not be able to prevent misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the U.S. 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.

Interference or derivation proceedings provoked by third parties or brought by the USPTO, or any foreign patent authority may be necessary to determine the priority of inventions or other matters of inventorship with respect to patents and patent applications. We may become involved in proceedings, including oppositions, interferences, derivation proceedings interparty reviews, patent nullification proceedings, or re-examinations, challenging our patent rights or the patent rights of others, and the outcome of any such proceedings are highly uncertain. An adverse determination in any such proceeding could reduce the scope of, or invalidate, important patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. Our business also could be harmed if a prevailing party does not offer us a license on commercially reasonable terms, if any license is offered at all. Litigation or other proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may also become involved in disputes with others regarding the ownership of intellectual property rights. If we are unable to resolve these disputes, we could lose valuable intellectual property rights.

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.

Additionally, the U.S. Congress and certain state legislatures in the U.S. have also passed, or have proposed passing, legislation that could adversely impact our ability to settle patent litigations. For example, the State of California has enacted legislation that creates a presumption, with certain exceptions and safe harbors, that various types of patent litigation settlements are anti-competitive, and imposes substantial monetary penalties on companies and individuals who do not comply. The enforcement of this law has been preliminarily enjoined on constitutional grounds, but such legislation still creates a risk of significant potential exposure for settling patent litigations and, in turn, makes it more difficult to settle in the first place, which could have a material adverse effect on our business. VYKAT XR is listed in the Orange Book and can be cited by potential generic competitors in support of approval of an abbreviated new drug application (ANDA). An ANDA provides for the marketing of a drug product that has the same active ingredients in the same strengths and dosage form as the listed drug and has been shown to be bioequivalent to the listed drug. Other than the requirement for bioequivalence testing, ANDA applicants are not required to conduct, or submit results of, pre-clinical or clinical tests to prove the safety or effectiveness of their drug product. Drugs approved in this way are commonly referred to as "generic equivalents" to the listed drug and can often be substituted by pharmacists under prescriptions written for the original listed drug.

The ANDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA's Orange Book. Specifically, the applicant must certify that: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired but will expire on a particular date and approval is sought after patent expiration; or

(iv) the listed patent is invalid or will not be infringed by the new product. The ANDA applicant may also elect to submit a section viii statement certifying that its proposed ANDA label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent. If the ANDA applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

We may receive a Paragraph IV certification asserting that the new product will not infringe our listed patents for VYKAT XR, or that such patents are invalid. Upon receipt of such a Paragraph IV certification, we would have 45 days from the receipt of such a certification to initiate a patent infringement lawsuit. The filing of a patent infringement lawsuit within 45 days of the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit, or a decision in the infringement case that is favorable to the ANDA applicant.

The ANDA application also will not be approved until any applicable non-patent exclusivity, such as Orphan Exclusivity, listed in the Orange Book for the referenced product has expired.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims, including potential challenges to our Orange Book patent listings in the U.S., may cause us to incur significant expenses and could distract our technical or management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the market price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. Uncertainties resulting from the initiation and continuation of intellectual property litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be materially adversely affected, harming our business and competitive position.

In addition to our patented technology and products, we rely upon confidential proprietary information, including trade secrets, unpatented know-how, technology and other proprietary information, to develop and maintain our competitive position. Any disclosure to or misappropriation by third parties of our confidential proprietary information could enable competitors to quickly duplicate or surpass our technological achievements, thus eroding our competitive position in the market. We seek to protect our confidential proprietary information, in part, by confidentiality agreements with our employees and our collaborators and consultants. We also have agreements with our employees and selected consultants that obligate them to assign their inventions to us. These agreements are designed to protect our proprietary information; however, we cannot be certain that our trade secrets and other confidential information will not be disclosed or that competitors will not otherwise gain access to our trade secrets, or that technology relevant to our business will not be independently developed by a person that is not a party to such an agreement. Furthermore, if the employees, consultants or collaborators that are parties to these agreements breach or violate the terms of these agreements, we may not have adequate remedies for any such breach or violation, and we could lose our trade secrets through such breaches or violations. Further, our trade secrets could be disclosed, misappropriated or otherwise become known or be independently discovered by our competitors. In addition, intellectual property laws in foreign countries may not protect trade secrets and confidential information to the same extent as the laws of the U.S. If we are unable to prevent disclosure of the intellectual property related to our technologies to third parties, we may not be able to establish or maintain a competitive advantage in our market, which would harm our ability to protect our rights and have a material adverse effect on our business.

We may not be able to protect or enforce our intellectual property rights throughout the world.

Filing, prosecuting and defending patents and trademarks on all of our planned products throughout the world would be prohibitively expensive to us. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection but where enforcement is not as strong as in the U.S. These products may compete with our products in jurisdictions where we do not have any issued patents and our patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so competing. Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to biopharmaceuticals, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business.

The ongoing conflict in Ukraine and related sanctions could significantly devalue our Russian, Belarusian, and Eurasian patents and/or patent applications. Recent Russian decrees may also significantly limit our ability to enforce Russian patents. We cannot predict when or how this situation will change.

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

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. The following examples are illustrative:

- others may be able to make products that are similar to our current and planned products, but that are not covered by claims in our patents;
- the original filers of our patents that we developed or purchased might not have been the first to make the inventions covered by the claims contained in such patents;
- we might not have been the first to file patent applications covering an invention;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- pending patent applications may not lead to issued patents;
- issued patents may not provide us with any competitive advantages, or may be held invalid, unenforceable or not properly listable in the Orange Book in the U.S., as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop or in-license additional proprietary technologies that are patentable; and
- the patents of others may have an adverse effect on our business.

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

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

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents or applications will be due to be paid by us to the USPTO and various governmental patent agencies outside of the U.S. in several stages over the lifetime of the patents or applications. The USPTO and various non-U.S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. 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. However, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, our competitors might be able to use our technologies and this circumstance would have a material adverse effect on our business.

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 products.

The U.S. has 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 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. For example, recent decisions raise questions regarding the award of patent term adjustment (PTA) for patents in families where related patents have issued without PTA. Thus, it cannot be said with certainty how PTA will be viewed in future and whether patent expiration dates may be impacted. 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. For example, the complexity and uncertainty of European patent laws have also increased in recent years. In Europe, a new unitary patent system took effect June 1, 2023, which has significantly impacted European patents, including those granted before the introduction of such a system. Under the unitary patent system, European applications have the option, upon grant of a patent, of becoming a Unitary Patent which will be subject to the jurisdiction of the Unitary Patent Court (UPC). Patents granted before the implementation of the UPC have the ability to opt out of the jurisdiction of the UPC and remain as national patents in the UPC countries. Patents that remain under the jurisdiction of the UPC will be potentially vulnerable to a single UPC-based revocation challenge that, if successful, could invalidate the patent in all countries who are signatories to the UPC. We cannot predict with certainty the long-term effects of any potential changes.

Additionally, recent reforms and changes at government agencies of the U.S. and those of non-U.S. jurisdictions could increase the uncertainties and costs surrounding the prosecution or maintenance of our patent applications, and the maintenance, enforcement, or defense of our issued patents. For example, the ability of the USPTO and other applicable patent authorities to properly administer their functions is highly dependent on the levels of funding available to the agency and their ability to retain key personnel and fill key leadership appointments, among various factors. Termination of employees or delays in replacing or hiring for key positions could significantly impact the ability of the USPTO and other applicable patent authorities to fulfill their functions and could greatly impact our ability to timely and adequately prosecute or maintain our patent applications, and our ability to timely and adequately maintain, enforce, or defend our issued patents.

If we do not obtain a patent term extension in the U.S. under the Hatch-Waxman Act and in foreign countries under similar legislation, thereby potentially extending the term of our marketing exclusivity for our planned products, our business may be materially harmed.

Depending upon the timing, duration and specifics of FDA marketing approval of our products, if any, one or more of the U.S. patents covering any such approved product(s) or the use thereof may be eligible for up to five years of patent term restoration under the Hatch-Waxman Act. The Hatch-Waxman Act allows a maximum of one patent to be extended per FDA approved product. Patent term extension also may be available in certain foreign countries upon regulatory approval of our planned products. Nevertheless, we may not be granted patent term extension either in the U.S. or in any foreign country because of, for example, our failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents, or otherwise failing to satisfy applicable requirements. Moreover, the term of extension, as well as the scope of patent protection during any such extension, afforded by the governmental authority could be less than we request.

If we are unable to obtain patent term extension or restoration, or the term of any such extension is less than requested, the period during which we will have the right to exclusively market our product will be shortened and our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced, possibly materially.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of their former employers or other third parties.

From time to time we may employ individuals who 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 do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee's former employer. Additionally, while we seek to protect our ownership of intellectual property rights by ensuring that our agreements with our employees, collaborators and other third parties with whom we do business include provisions requiring such parties to assign rights in inventions to us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed confidential information of our employees' former employers or other third parties. These and other claims that we have misappropriated the confidential information or trade secrets of third parties can have a similar negative impact on our business to the infringement claims discussed above.

Additionally, we may also be subject to claims that former employers or other third parties have an ownership interest in our patents. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and if we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Even if we are successful, litigation could result in substantial cost and be a distraction to our management and other employees.

Risks related to government regulation

The regulatory approval process is expensive, time consuming and uncertain, and we may not be successful in obtaining approvals for our planned products.

The research, testing, manufacturing, labeling, approval, selling, import, export, marketing and distribution of our products are subject to extensive regulation by the FDA in the U.S. and other regulatory authorities in other countries, and these regulations differ from country to country. We are not permitted to market our planned products until we have received the requisite approval or clearance from the appropriate authorities. In March 2025, we announced that the FDA has approved VYKAT XR in the U.S. for the treatment of hyperphagia in adults and pediatric patients 4 years of age and older with PWS. In May 2025, we announced that our MAA for obtaining regulatory approval of diazoxide choline prolonged-release tablets (which we market in the U.S. as VYKAT XR) for the treatment of adults and children four years and older with PWS who have hyperphagia had been validated by the EMA. Obtaining approvals from regulatory authorities can be a lengthy, expensive and uncertain process. In addition, failure to comply with FDA and other applicable U.S. and foreign regulatory requirements may subject us to administrative or judicially imposed sanctions, including the following:

- warning letters;

- civil or criminal penalties and fines;
- injunctions;
- suspension or withdrawal of regulatory approval;
- suspension of any ongoing clinical studies;
- voluntary or mandatory product recalls and publicity requirements;
- refusal to accept or approve applications for marketing approval of new drugs or biologics or supplements to approved applications filed by us;
- restrictions on operations, including costly new manufacturing requirements; or
- seizure or detention of our products or import bans.

Prior to receiving approval to commercialize any of our planned products in the U.S. or abroad, we will be required to demonstrate with substantial evidence from well-controlled clinical trials, and to the satisfaction of the FDA and other regulatory authorities abroad, that such planned products are safe and effective for their intended uses. Results from preclinical studies and clinical trials can be interpreted in different ways. Even if we believe the preclinical or clinical data for our planned products are promising, such data may not be sufficient to support approval by the FDA and other regulatory authorities. Administering any of our planned products to humans may produce undesirable side effects, which could interrupt, delay or cause suspension of clinical trials of our planned products and result in the FDA or other regulatory authorities denying approval of our planned products for any or all targeted indications.

Regulatory approval from the FDA or a comparable foreign regulatory authority is not guaranteed, and the approval process is expensive and may take several years. The FDA and comparable regulatory authorities also have substantial discretion in the approval process. Despite the time and expense exerted, failure can occur at any stage, and we could encounter problems that cause us to abandon or repeat clinical trials or perform additional preclinical studies and clinical trials. The number of preclinical studies and clinical trials that will be required for FDA approval or approval from comparable regulatory authorities varies depending on the planned product, the disease or condition that the planned product is designed to address and the regulations applicable to any particular planned product. The FDA and comparable regulatory authorities can delay, limit or deny approval of a planned product for many reasons, including, but not limited to, the following:

- a planned product may not be deemed safe or effective;
- FDA officials may not find the data from preclinical studies and clinical trials sufficient;
- the FDA might not approve our or our third-party manufacturer’s processes or facilities; or
- the FDA may change its approval policies or adopt new regulations.

If any planned products fail to demonstrate safety and effectiveness in clinical trials or do not gain regulatory approval, our business and results of operations will be materially and adversely harmed.

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

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

- the regulatory authorities may disagree with the design, implementation or results of our clinical trials;
- the regulatory authorities may reject some or all of our data from clinical trials due to concerns related to bias, unblinding before statistical analysis plan is finalized, and/or reliability of data when the analysis is considered exploratory and not planned prospectively;
- the regulatory authorities may not accept data pooled from different studies, especially if the studies features are not sufficiently similar;
- the regulatory authorities finds that our data are not adequate to support the safety and efficacy of our product candidate for the proposed indication;
- the regulatory authorities may disagree with our statistical analysis plans;

- the regulatory authorities may determine that our product candidates are not safe and effective or have undesirable or unintended side effects, toxicities or other characteristics that preclude our obtaining marketing approval or prevent or limit commercial use;
- the population studied in the clinical trial may not be sufficiently broad or representative to assure efficacy and safety in the full population for which we seek approval;
- the regulatory authorities may disagree with our interpretation of data from nonclinical studies or clinical trials;
- our clinical trials may not meet the statutory standard for substantial evidence of effectiveness or may fail to demonstrate statistical significance on the primary endpoint;
- we may be unable to demonstrate to the regulatory authorities that our product candidate's risk-benefit ratio for its proposed indication is acceptable;
- changes in priorities, reduction in staffing, large staff turnover or inadequate funding for the regulatory authorities could hinder those agencies from performing normal business functions and increase the time necessary for regulatory submissions to be reviewed and approved, or decrease the likelihood of an approval;
- the regulatory authorities may fail to approve the manufacturing processes, test procedures and specifications or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval or resulting in delays in our regulatory approval.

In addition, even if we obtain approval of a product candidate, regulatory authorities may approve a product candidate for fewer or more limited indications than we initially request, or may impose significant limitations in the form of narrow indications, warnings, contraindications, or a risk evaluation and mitigation strategy (REMS). Regulatory authorities may not approve the price we intend to charge for a product candidate, may grant approval contingent on the performance of costly post-marketing clinical trials or other post-marketing studies, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of a product candidate. Any of the foregoing scenarios could seriously harm our business.

Further, under the current administration and new leadership at the Department of Health and Human Services (HHS) and the FDA, executive orders and layoffs due to the reduction in force initiative and other measures implemented by the Department of Government Efficiency may impact the normal operations of the FDA as well as other federal agencies. The FDA may lack adequate staff and resources to meet current review, approval, and inspection schedules, which could delay our anticipated timelines. In January 2025, an executive order entitled "Unleashing Prosperity Through Deregulation", was issued which calls for at least 10 existing regulations to be repealed whenever an executive department or agency publicly proposes for notice and comment or otherwise promulgates a new regulation. Recent developments at the FDA include announcement of a new Commissioner's National Priority Voucher program for companies supporting certain U.S. national health priorities and interests. To the extent our competitors are selected for this new voucher pilot program and obtain faster approvals, our competitive position may be harmed. It is unclear how our industry and our clinical programs and operations will be impacted by policies and regulations implemented under the current administration and FDA leadership, or other executive orders. There is significant uncertainty in the industry and how federal agencies like the FDA will change in the coming years under the current administration. To the extent new policies and other agency changes lead to disruptions in the FDA's operations, our correspondence and regulatory review processes with the FDA may be materially delayed.

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

From time to time, we may publicly disclose preliminary or topline data from our nonclinical studies and clinical trials, which is based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or clinical trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the topline or preliminary results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Topline data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, topline data should be viewed with caution until the final data become available.

Furthermore, regulatory agencies may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability

or commercialization of the particular product candidate or product and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and investors or regulatory authorities may not agree with what we determine is material or otherwise appropriate information to include in our disclosure. If the interim, topline, or preliminary data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our product candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

Even if we receive marketing approval for a planned product, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense and subject us to penalties if we fail to comply with applicable regulatory requirements.

Once marketing approval has been obtained, the approved product and its manufacturer are subject to continual review by the FDA or non-U.S. regulatory authorities, including, among other things, recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. The drug name will also be subject to review and approval by the FDA and other non-U.S. regulatory authorities, and may not be the same in all geographies, which may cause confusion.

Future approvals may contain requirements for potentially costly post-marketing follow-up studies to monitor the safety and effectiveness of the approved product. In addition, we are subject to extensive and ongoing regulatory requirements by the FDA, EMA and other regulatory authorities with regard to the labeling, packaging, adverse event reporting, storage, advertising, promotion and recordkeeping for our products.

In addition, we are required to comply with cGMP and foreign regulations regarding the manufacture of our drugs, which include requirements related to quality control and quality assurance as well as the corresponding maintenance of records and documentation. Further, regulatory authorities must approve these manufacturing facilities before they can be used to manufacture drug products, and these facilities are subject to continual review and periodic inspections by the FDA, EMA and other regulatory authorities for compliance with cGMP and foreign regulations. If we or a third party discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory authority may impose restrictions on that product, the manufacturer or us, including requiring withdrawal of the product from the market or suspension of manufacturing. Changes to the manufacturing process are strictly regulated and generally require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance. In addition, in June 2024, the U.S. Supreme Court in Loper Bright Enterprises v. Raimondo reversed its longstanding approach under the Chevron doctrine, which gave deference to regulatory agencies in litigation against the FDA and other agencies. As a result, more companies may bring lawsuits against the FDA to challenge longstanding decisions and policies of the FDA, which could undermine the FDA's authority, lead to uncertainties in the industry, and disrupt the FDA's normal operations, which could delay the FDA's review of our future marketing applications.

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market, though the FDA must provide an application holder with notice and an opportunity for a hearing in order to withdraw its approval of an application. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products; and
- injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates the marketing, labeling, advertising and promotion of drug and device products that are placed on the market. While physicians may prescribe drugs and devices for off label uses, manufacturers may only promote for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off label uses, and a company that is found to have improperly promoted off label uses may be subject to significant liability.

For example, drugs that are developed for rare diseases can be designated as Orphan Drugs. In the U.S., the disease or condition has an incidence of less than 200,000 persons and in the E.U. the prevalence of the condition must be not more than 5 in 10,000

persons. In the U.S., orphan-designated drugs are granted up to 7-year market exclusivity. In the E.U., products granted orphan designation are subject to reduced fees for protocol assistance, marketing authorization applications, inspections before authorization, applications for changes to marketing authorizations, and annual fees, and potentially benefit from 10 years of market exclusivity (which period of market exclusivity may be extended by two years for orphan medicinal products that have also complied with an agreed pediatric investigation plan), during which time the EMA is generally precluded from accepting a MAA for a similar medicinal product. Applicants must first satisfy the orphan designation criteria and apply for orphan designation before making the application for marketing authorization. The applicant must then successfully maintain the orphan designation at the time of the MAA in order to qualify for 10 years of orphan market exclusivity. It is important to note that the regulatory protection afforded to medicinal products such as data exclusivity, marketing protection, market exclusivity for orphan indications and pediatric extension are currently under review at the E.U. level. The protection currently afforded in the E.U. could be reduced in the future. On April 26, 2023, the European Commission adopted a proposal for a new Directive and a new Regulation. If enacted into law, this proposal will revise and replace the existing general pharmaceutical legislation and will affect the existing period of regulatory protections afforded to medicinal products. The proposal is now undergoing the legislative process and the text of the initial proposal is likely to be amended by the European Parliament and the European Council.

While the Catalyst Pharms., Inc. v. Becerra, 14 F.4th 1299 (11th Cir. 2021) decision created uncertainty in the application of the orphan drug exclusivity, on January 24, 2023, the FDA published a notice in the Federal Register to clarify that while the agency complies with the court's order in Catalyst, the FDA intends to continue to apply its longstanding interpretation of the regulations to matters outside of the scope of the Catalyst order - that is, the agency will continue tying the scope of orphan-drug exclusivity to the uses or indications for which a drug is approved, which permits other sponsors to obtain approval of a drug for new uses or indications within the same orphan designated disease or condition that have not yet been approved. It is unclear how future litigation, legislation, agency decisions, and administrative actions will impact the scope of the orphan drug exclusivity.

Further, orphan exclusivity in the U.S. and other jurisdictions may be lost if the FDA or comparable foreign regulatory authorities later determines that the request for designation was materially defective or if we are unable to ensure that we will be able to manufacture sufficient quantities of the product to meet the needs of patients with the rare disease or condition. In the U.S. and other jurisdictions, orphan drug exclusivity may not effectively protect an approved product from competition because different drugs with different active moieties may be approved for the same condition. Even after an orphan drug is approved, the regulatory authorities can subsequently approve the same drug with the same active moiety for the same condition if the applicable regulatory authorities conclude that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care or the manufacturer of the product with orphan exclusivity is unable to maintain sufficient product quantity.

Failure to obtain marketing approvals in foreign jurisdictions will prevent us from marketing our products internationally.

We may seek distribution and marketing partners for our current products outside the U.S. and may market planned products in international markets. In May 2025, we announced that our MMA for obtaining regulatory approval of diazoxide choline prolonged-release tablets (which we market in the U.S. as VYKAT XR) for the treatment of adults and children four years and older with PWS who have hyperphagia had been validated by the EMA. If we obtain E.U. marketing authorization, the expansion of our commercial activities in the E.U. will increase our compliance costs and exposure to potential liabilities under E.U. laws.

Drugs are also subject to extensive regulation outside of the U.S., including in the E.U. In the E.U., there is a centralized approval procedure that allows for authorization of marketing of a product by the EMA in all 27 Member States of the E.U. (which includes most major countries in the E.U.) and is mandatory for medicinal products for rare disease. After receiving regulatory approval through any of the E.U. registration procedures, separate pricing and reimbursement approvals are also required in most countries. During the time of MAA assessment and following approval of an MAA by the EMA, the applicant is subject to oversight and inspection in pharmacovigilance (Good Vigilance Practice - GVP), clinical trials (Good Clinical Practice - GCP), manufacturing and distribution (Good Manufacturing and Good Distribution Practices- GMP & GDP) and as a Marketing Authorisation Holder.

We have had limited interactions with foreign regulatory authorities. The approval procedures vary among countries and can involve additional clinical testing, and the time required to obtain approval may differ from that required to obtain FDA approval. Moreover, clinical trials or manufacturing processes conducted in one country may not be accepted by regulatory authorities in other countries. Approval by the FDA does not ensure approval by regulatory authorities in other countries or regions, and approval by one or more foreign regulatory authorities does not ensure approval by regulatory authorities in other foreign countries or by the FDA. However, a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory process in others. The foreign regulatory approval process may include all of the risks associated with obtaining FDA approval. We may not obtain foreign regulatory approvals on a timely basis, if at all. We may not be able to file for regulatory approvals and even if we file we may not receive necessary approvals to commercialize our products in any market.

Healthcare reform measures could hinder or prevent our commercial success.

In the U.S., there have been, and we expect there will continue to be, a number of legislative and regulatory changes to the healthcare system in ways that could affect our future revenue and profitability and the future revenue and profitability of our potential

customers. Federal and state lawmakers regularly propose and, at times, enact legislation that would result in significant changes to the healthcare system, some of which are intended to contain or reduce the costs of medical products and services. For example, one of the most significant healthcare reform measures in decades, the Patient Protection and Affordable Care Act of 2010 (PPACA), was enacted in 2010. The PPACA contains a number of provisions, including those governing enrollments in federal healthcare programs, reimbursement changes and fraud and abuse measures, all of which will impact existing government healthcare programs and will result in the development of new programs. The PPACA, among other things:

- could result in the imposition of injunctions;
- requires collection of rebates for drugs paid by Medicaid managed care organizations; and
- requires manufacturers to participate in a coverage gap discount program, under which they must agree to offer 50% point-of-sale discounts off negotiated prices of applicable branded drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D.

Since its enactment, there have been judicial, executive and Congressional challenges to certain aspects of the PPACA. In June 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the PPACA without specifically ruling on the constitutionality of the ACA. Thus, the ACA remains in force in its current form. Any changes to the PPACA are likely to have an impact on our results of operations and may have a material adverse effect on our results of operations. We cannot predict what other health care programs and regulations will ultimately be implemented at the federal or state level or the effect of any future legislation or regulation in the U.S. may have on our business.

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. For example, the Budget Control Act of 2011, among other things, created the Joint Select Committee on Deficit Reduction to recommend proposals for spending reductions to Congress. The Joint Select Committee did not achieve a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, which triggered the legislation's automatic reduction to several government programs, including aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, starting in 2013. In January 2013, former President Obama signed into law the American Taxpayer Relief Act of 2012 (ATRA), which delayed for another two months the budget cuts mandated by the sequestration provisions of the Budget Control Act of 2011. The ATRA, among other things, also reduced Medicare payments to several providers, including hospitals, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. In March 2013, the President signed an executive order implementing sequestration, and in April 2013, the 2% Medicare reductions went into effect. We cannot predict how any additional legislative changes or changes in presidential administrations will affect our business.

There likely will continue to be legislative and regulatory proposals at the federal and state levels directed at containing or lowering the cost of health care. We cannot predict the initiatives that may be adopted in the future or their full impact. The continuing efforts of the government, insurance companies, managed care organizations and other payers of healthcare services to contain or reduce costs of health care may adversely affect:

- our ability to set a price that we believe is fair for our products;
- our ability to generate revenue and achieve or maintain profitability; and
- the availability of capital.

There has recently been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted legislation designed, among other things, to bring more transparency to product pricing, to review the relationship between pricing and manufacturer patient programs, and to reform government program reimbursement methodologies for pharmaceutical products. For example, in August 2022, Congress passed the Inflation Reduction Act of 2022 (IRA), which includes prescription drug provisions that have significant implications for the pharmaceutical industry and Medicare beneficiaries, including allowing the federal government to negotiate a maximum fair price for certain high-priced single source Medicare drugs, imposing penalties and excise tax for manufacturers that fail to comply with the drug price negotiation requirements, requiring inflation rebates for all Medicare Part B and Part D drugs, with limited exceptions, if their drug prices increase faster than inflation, and redesigning Medicare Part D to reduce out-of-pocket prescription drug costs for beneficiaries, among other changes. Only high-expenditure single-source drugs that have been approved for at least 7 years (11 years for single-source biologics) can qualify for negotiation, with the negotiated price taking effect two years after the selection year. For 2026, the first year in which negotiated prices become effective, CMS selected 10 high-cost Medicare Part D drugs in 2023, negotiations began in 2024, and the negotiated maximum fair price for each drug has been announced. CMS has selected 15 additional Medicare Part D drugs for negotiated maximum fair pricing in 2027. For 2028, up to an additional 15 drugs, which may be covered under either Medicare Part B or Part D, will be selected, and for 2029 and subsequent years, up to 20 additional Part B or Part D drugs will be selected. Various industry stakeholders, including certain pharmaceutical companies and the Pharmaceutical Research and Manufacturers of America, have initiated lawsuits against the federal government asserting that the price negotiation provisions of the IRA are unconstitutional. The current administration has issued executive orders focused on decreasing prescription drug prices, including directing the Secretary of HHS to establish a mechanism through which American patients can buy drugs directly from

manufacturers who sell at a most-favored-nation price and directing the U.S. Trade Representative and Secretary of Commerce to take action to ensure foreign countries are not engaged in practices that purposefully and unfairly undercut market prices and drive price hikes in the U.S. If HHS begins to set most-favored-nation pricing targets for prescription drugs, including the use of international pricing reference to set drug prices in the U.S., or increases generic and biosimilar drug entry sooner than expected, that can have a material adverse effect on our industry, ability to set adequate pricing for new drugs to recover R&D costs, ability to attract potential investors and potential buyers in the future. We cannot predict the full impact of the executive orders focused on reducing prescription drug prices or increasing domestic drug manufacturing capacity, or other measures that may be implemented by the current administration related to drug pricing, drug supply chain and manufacturing in the U.S. Such cost containment policies and executive orders could substantially and negatively impact the prices we may charge for any approved products, which could harm our valuation, ability to generate revenue and achieve and sustain profitability. In addition, the One Big Beautiful Bill Act (OBBBA), which was signed into law in July 2025, includes provisions that will impact the United States healthcare system in various ways, including budget cuts to Medicaid and introducing new participant work and eligibility requirements for Medicaid coverage, which are expected to significantly change the administration and applicability of Medicaid coverage. The OBBBA also expanded exemptions for orphan designated drugs for Medicare drug price negotiations, which is expected to incentivize development of orphan designated drugs or increase competition for drug development in orphan diseases or conditions. Although the full impact of the OBBBA on the healthcare system and our business is uncertain, the resulting changes may increase the cost and complexity of completing clinical development of and launching any product candidates for which we may receive regulatory approval or increase our competition in the marketplace, any of which could adversely affect our business and prospects. The impact of ongoing and future judicial challenges, as well as future legislative, executive, and administrative actions and healthcare measures and agency rules implemented by the government on us and the pharmaceutical industry as a whole is unclear. To the extent changes lead to disruptions in federal agencies, greater uncertainty in the industry, or impose more constraints on drug pricing, such as the introduction of the most-favored nation pricing or international reference pricing, our business may be materially impacted. The implementation of cost containment measures or other healthcare reforms may negatively impact the valuation of our company or prevent us from being able to generate revenue, attain profitability, or commercialize our product candidates if approved.

In addition, individual states in the U.S. have also become increasingly active in implementing regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures and, in some cases, mechanisms to encourage importation from other countries and bulk purchasing. A number of states are considering or have recently enacted state drug price transparency and reporting laws that could substantially increase our compliance burdens and expose us to greater liability under such state laws. Further, the FDA has authorized the State of Florida to develop a program to import certain prescription drugs from Canada for a limited time-period to help reduce drug costs, provided that Florida's Agency for Health Care Administration meets the requirements set forth by the FDA. Other states may follow Florida. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our products or product candidates.

Further, changes in regulatory requirements and guidance may occur and we may need to amend clinical trial protocols of ongoing studies to reflect these changes. Amendments may require us to resubmit our clinical trial protocols IRBs for reexamination, which may impact the costs, timing or successful completion of a clinical trial. In light of widely publicized events concerning the safety risk of certain drug products, regulatory authorities, members of Congress, the Governmental Accounting Office, medical professionals and the general public have raised concerns about potential drug safety issues. These events have resulted in the recall and withdrawal of drug products, revisions to drug labeling that further limit use of the drug products and establishment of risk management programs that may, for instance, restrict distribution of drug products or require safety surveillance or patient education. The increased attention to drug safety issues may result in a more cautious approach by the FDA to clinical trials and the drug approval process. Data from clinical trials may receive greater scrutiny with respect to safety, which may make the FDA or other regulatory authorities more likely to terminate or suspend clinical trials before completion or require longer or additional clinical trials that may result in substantial additional expense and a delay or failure in obtaining approval or approval for a more limited indication than originally sought.

Given the serious public health risks of high-profile adverse safety events with certain drug products, the FDA may require, as a condition of approval, costly risk evaluation and mitigation strategies, which may include safety surveillance, restricted distribution and use, patient education, enhanced labeling, special packaging or labeling, expedited reporting of certain adverse events, preapproval of promotional materials and restrictions on direct-to-consumer advertising.

If we fail to comply with healthcare regulations, we could face substantial penalties and our business, operations and financial condition could be adversely affected.

Even though we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payers, certain federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights are and will be applicable to our business. We could be subject to healthcare fraud and abuse and patient privacy regulation by both the federal government and the states in which we conduct our business. The regulations that may affect our ability to operate include, without limitation:

- the federal healthcare program Anti-Kickback Statute, which prohibits, among other things, any person from knowingly and willfully offering, soliciting, receiving or providing remuneration, directly or indirectly, in exchange for or to induce either the referral of an individual for, or the purchase, order or recommendation of, any good or service for which payment may be made under federal healthcare programs, such as the Medicare and Medicaid programs;
- indirectly, to induce either the referral of an individual, for an item or service or the purchasing or ordering of a good or service, for which payment may be made under federal healthcare programs, such as the Medicare and Medicaid programs;
- the federal False Claims Act, which prohibits, among other things, individuals or entities from knowingly presenting, or causing to be presented, false claims, or knowingly using false statements, to obtain payment from the federal government, and which may apply to entities like us which provide coding and billing advice to customers;
- federal criminal laws that prohibit executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- the federal transparency requirements under the Health Care Reform Law requires manufacturers of drugs, devices, biologics and medical supplies to report to the HHS information related to physician payments and other transfers of value and physician ownership and investment interests;
- Health Insurance Portability and Accountability Act (HIPAA), as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 (HITECH Act), which governs the conduct of certain electronic healthcare transactions and protects the security and privacy of protected health information; and
- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payer, including commercial insurers.

The PPACA, among other things, amends the intent requirement of the Federal Anti-Kickback Statute and criminal healthcare fraud statutes. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. In addition, the PPACA provides that the government may assert that a claim including items or services resulting from a violation of the Federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act.

If our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines and the curtailment or restructuring of our operations. Any penalties, damages, fines, curtailment or restructuring of our operations could adversely affect our ability to operate our business and our financial results. 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. Moreover, achieving and sustaining compliance with applicable federal and state privacy, security and fraud laws may prove costly.

Our relationships with healthcare providers, other customers, and third-party payers are subject to applicable anti-kickback, fraud and abuse, and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, exclusion from government healthcare programs, contractual damages, reputational harm, and diminished profits and future earnings.

Now that we have begun commercializing VYKAT XR, we are subject to additional healthcare statutory and regulatory requirements and enforcement by the federal government and the states and foreign governments in which we conduct our business. Healthcare providers, physicians, and third-party payers play a primary role in the recommendation and prescription of VYKAT XR. Our arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell, and distribute VYKAT XR. Restrictions under applicable federal and state healthcare laws and regulations include, but are not limited to, the Anti-Kickback Statute, the False Claims Act, HIPAA and the HITECH Act.

Efforts to ensure that our business arrangements with third parties comply with applicable healthcare laws and regulations could involve substantial costs and may require us to undertake or implement additional policies or measures. We may face claims and proceedings by private parties, and claims, investigations and other proceedings by governmental authorities, relating to allegations that our business practices do not comply with statutes, regulations or case law involving applicable fraud and abuse, privacy or data protection, or other healthcare laws and regulations, and it is possible that courts or governmental authorities may conclude that we have not complied with them, or that we may find it necessary or appropriate to settle any such claims or other proceedings. In connection with any such claims, proceedings, or settlements, we may be subject to significant civil, criminal, and administrative penalties, damages, fines, other damages, imprisonment, exclusion from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we do business is found not to be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

We are subject to U.S. and foreign laws regarding privacy, data protection, and data security that could entail substantial compliance costs, while the failure to comply could subject us to significant liability.

Privacy, data protection, and data security have become significant issues in the U.S., Europe, and other jurisdictions where we conduct or may in the future conduct our operations. The regulatory framework for the collection, use, safeguarding, sharing, and transfer of health and other personal information is rapidly evolving worldwide and is likely to remain in flux for the foreseeable future. The scope and interpretation of the laws that are or may be applicable to us are often uncertain, subject to differing interpretations, and may be inconsistent among different jurisdictions.

In the U.S., HIPAA, as amended by the HITECH Act, imposes on covered entities certain requirements relating to the privacy, security, and transmission of individually identifiable health information. The legislation also increased the civil and criminal penalties that may be assessed for violations and gave state attorneys general the authority to file civil actions in federal courts to enforce the HIPAA rules. In addition, for clinical trials conducted in the U.S., any personal information that is collected is further regulated by the Federal Policy for the Protection of Human Subjects. Privacy laws are also being enacted or considered at the state level, including significant new legislation in California, the California Consumer Privacy Act, as amended by the California Privacy Rights Act. While there is currently an exception for protected health information subject to HIPAA and clinical trial regulations, these and other state privacy laws may impact our business activities, and there continues to be uncertainty about how these laws will be interpreted and enforced. Other states have passed privacy legislation, including general privacy legislation similar to the CCPA, and legislation such as Washington's My Health, My Data Act, that also may impact our business activities, in the future and additional states are evaluating similar legislation. In the event we enroll subjects in clinical trials in the E.U. or other jurisdictions, or otherwise acquire or process personal data of individuals in those jurisdictions, we may be subject to additional restrictions and obligations relating to the collection, use, storage, transfer, and other processing of this data. Our activities in the European Economic Area (EEA), for example, are governed by the E.U. General Data Protection Regulation (GDPR).

We may need to take additional steps, such as new contractual negotiations or modifications to our policies or practices relating to cross-border transfers of personal data, to comply with these restrictions and obligations. More generally, laws and regulations governing privacy and data protection exist in many other countries around the world, and these laws (which are evolving and expanding) create complicated and potentially inconsistent obligations that may impact our business.

The increasing number, complexity, and potential inconsistency of current and future laws and regulations relating to privacy, data protection, and data security in the U.S. and other countries make our compliance obligations more difficult and costly. If we fail to comply with applicable laws and regulations or experience a breach of security that results in unauthorized disclosure of personal information - or if a third party with whom we share personal information or who processes such information for us fails to comply with applicable requirements or experiences a security breach or incident- or if any of these is reported or perceived to have occurred, it could lead to government investigations, enforcement actions, and other proceedings, as well as civil claims and litigation against us. We could incur substantial costs to defend against any such claims or proceedings and may also be held liable for significant fines, penalties, and monetary judgments. Any of the foregoing could have a material adverse effect on our business, results of operations, reputation, and prospects.

Risks related to ownership of our securities

Our stock price may be volatile, and purchasers of our securities could incur substantial losses.

Our stock price has been and is likely to continue to be volatile. The stock market in general, and the market for biotechnology companies in particular, have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, investors may not be able to sell their common stock at or above the purchase price. The market price for our common stock may be influenced by many factors, including the following:

- our ability to successfully commercialize, and realize significant revenues from sales of VYKAT XR;
- the success of competitive products or technologies;
- the results of other clinical trials of our products or those of our competitors;
- regulatory or legal developments in the U.S. and other countries, especially changes in laws or regulations applicable to our products;
- introductions and announcements of new products by us, our commercialization partners, or our competitors, and the timing of these introductions or announcements;
- actions taken by regulatory agencies with respect to our products, clinical trials, manufacturing process or sales and marketing terms;
- variations in our financial results or those of companies that are perceived to be similar to us;

- the success of our efforts to acquire or in-license additional products or planned products;
- developments concerning our collaborations, including but not limited to those with our sources of manufacturing supply and our commercialization partners;
- developments concerning our ability to bring our manufacturing processes to scale in a cost-effective manner;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures or capital commitments;
- developments or disputes concerning patents or other proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our products;
- our ability or inability to raise additional capital and the terms on which we raise it;
- the recruitment or departure of key personnel;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- actual or anticipated changes in earnings estimates or changes in stock market analyst recommendations regarding our common stock, other comparable companies or our industry generally;
- trading volume of our common stock;
- sales of our common stock by us or our stockholders;
- general economic, industry and market conditions; including those due to inflation; and
- the other risks described in this "Risk Factors" section.

These broad market and industry factors may seriously harm the market price of our common stock, regardless of our operating performance. In the past, following periods of volatility in the market, securities class-action litigation has often been instituted against companies. Such litigation, if instituted against us, could result in substantial costs and diversion of management's attention and resources, which could materially and adversely affect our business, financial condition, results of operations and growth prospects.

Future sales of our common stock, or the perception that future sales may occur, may cause the market price of our common stock to decline, even if our business is doing well.

Sales of substantial amounts of our common stock in the public market, or the perception that these sales may occur, could materially and adversely affect the price of our common stock and could impair our ability to raise capital through the sale of additional equity securities. All of our shares of common stock are freely tradable, without restriction, in the public market, except for any shares held by our affiliates.

In the future, we may issue additional shares of common stock or other equity or debt securities convertible into common stock in connection with a financing, acquisition, litigation settlement, employee arrangement or otherwise. Any such issuance could result in substantial dilution to our existing stockholders and could cause our stock price to decline.

Our ability to use our net operating loss carry forwards and certain other tax attributes will be limited.

Our ability to utilize our federal net operating loss, carryforwards and federal tax credit will be limited under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (the Code). The limitations apply if an "ownership change," as defined by Section 382, occurs. Generally, an ownership change occurs if the percentage of the value of the stock that is owned by one or more direct or indirect "five percent shareholders" increases by more than 50% over their lowest ownership percentage at any time during the applicable testing period (typically three years). The Company has completed a Section 382 analysis from January 1, 2017 through December 31, 2023 and determined that a change in ownership has occurred on March 7, 2017, December 21, 2018, June 30, 2020 and September 26, 2023. As a result, the net operating loss carryforwards and tax credit carryforwards maybe subject to annual limitations before being applied to reduce future income tax liabilities. For years ended after December 31, 2023, the utilization of net operating losses and tax credit carryforwards are subject to further limitation in the event an additional ownership change were to occur for tax purposes. In addition, we raised capital in May 2024 and July 2025 that may further limit our ability to utilize our net operating losses and other tax attributes to offset taxable income. As a result, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards and other tax attributes to offset U.S. federal taxable income will be subject to limitations, which could potentially result in increased future tax liability to us.

If securities or industry analysts do not continue to publish research, or publish inaccurate or unfavorable research, about our business, our stock price and trading volume could decline.

The trading market for our common stock will continue to depend, in part, on the research and reports that securities or industry analysts publish about us or our business. If one or more of the analysts who cover us downgrade our stock or publish inaccurate or unfavorable research about our business, our stock price would likely decline. In addition, if our operating results fail to meet the forecast of analysts, our stock price would likely decline. If one or more of these analysts cease coverage of our company or fail to publish reports on us regularly, demand for our common stock could decrease, which might cause our stock price and trading volume to decline.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of us 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 stockholders might otherwise receive a premium for their shares. These provisions could also 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, 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. Because our Board is responsible for appointing the members of our management team, these provisions could in turn affect any attempt by our stockholders to replace current members of our management team. Among others, these provisions include the following:

- our Board is divided into three classes with staggered three-year terms which may delay or prevent a change of our management or a change in control;
- our Board has the right to elect directors to fill a vacancy created by the expansion of our Board or the resignation, death or removal of a director, which will prevent stockholders from being able to fill vacancies on our Board;
- our stockholders are not able to act by written consent or call special stockholders' meetings; as a result, a holder, or holders, controlling a majority of our capital stock cannot take certain actions other than at annual stockholders' meetings or special stockholders' meetings called by our Board, the chairman of our board, the chief executive officer or the president;
- our certificate of incorporation prohibits cumulative voting in the election of directors, which limits the ability of minority stockholders to elect director candidates;
- amendments of our certificate of incorporation and bylaws require the approval of 66 2/3% of our outstanding voting securities;
- our stockholders are required to provide advance notice and additional disclosures in order to nominate individuals for election to our Board or to propose matters that can be acted upon at a stockholders' meeting, which may discourage or deter a potential acquirer from conducting a solicitation of proxies to elect the acquirer's own slate of directors or otherwise attempting to obtain control of our company; and
- our Board are able to issue, without stockholder approval, shares of undesignated preferred stock, which makes it possible for our Board to issue preferred stock with voting or other rights or preferences that could impede the success of any attempt to acquire us.

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.

Our employment agreements with our executive officers may require us to pay severance benefits to any of those persons who are terminated in connection with a change in control of us, which could harm our financial condition or results.

Certain of our executive officers are parties to employment agreements that contain change in control and severance provisions providing for aggregate cash payments for severance and other benefits and acceleration of equity vesting in the event of a termination of employment in connection with a change in control of us. The accelerated vesting of options could result in dilution to our existing stockholders and harm the market price of our common stock. The payment of these severance benefits could harm our financial condition and results. In addition, these potential severance payments may discourage or prevent third parties from seeking a business combination with us.

We have not paid dividends in the past and do not expect to pay dividends in the future, and, as a result, any return on investment may be limited to the value of our stock.

We have never paid dividends and do not anticipate paying dividends in the foreseeable future. The payment of dividends will depend on our earnings, capital requirements, financial condition, prospects and other factors our Board may deem relevant. If we do

not pay dividends, our stock may be less valuable because a return on your investment will only occur if our stock price appreciates and you sell our common stock thereafter.

General risks

Our information technology systems may fail or experience security breaches and incidents that could adversely impact our business and operations and subject us to liability.

We have experienced significant growth in the complexity of our data and the software tools that we rely upon. We rely significantly upon information technology systems and infrastructure owned and maintained by us or by third party providers to generate, collect, store, and transmit confidential and proprietary information and data (including but not limited to intellectual property, proprietary business information, and personal information) and to operate our business.

We expect to continue to incur significant costs related to technical and procedural controls to reduce the risks to our information technology systems. Despite these measures, our information technology and other internal infrastructure systems face the risk of failures, interruptions, security breaches and incidents, or other harm from various causes or sources, and third parties with whom we share confidential or proprietary information face similar risks and may experience similar events that materially impact us.

The techniques used by cyber criminals change frequently, may not be recognized until launched, and can originate from a wide variety of sources, including outside groups and individuals with a range of motives (including industrial espionage) and expertise, such as organized crime affiliates, terrorist organizations, or hostile foreign governments or agencies. The costs to us to investigate and mitigate actual and suspected cybersecurity breaches and incidents could be significant. We may not be able to anticipate all types of security threats and implement preventive measures effective against all such threats. In addition, an increased amount of work is occurring remotely, including through the use of mobile devices. This could increase our cybersecurity risk, create data accessibility concerns, and make us more susceptible to communication disruptions.

If we do not accurately predict and identify our information technology systems requirements and failures and timely enhance our information technology systems, or if our remediation efforts are not successful, it could result in a material disruption of our business operations, including the loss or unauthorized disclosure of our trade secrets, individuals' personal information, or other proprietary or sensitive data.

Moreover, any security breach or other event that leads to loss of, unauthorized access to, disclosure of, or other processing of personal information could harm our reputation, compel us to comply with federal and/or state notification laws and foreign law equivalents, subject us to mandatory corrective action, and otherwise subject us to liability under laws and regulations that protect the privacy and security of personal information. For more information see our risk factor "*We are subject to U.S. and foreign laws regarding privacy, data protection, and data security that could entail substantial compliance costs, while the failure to comply could subject us to significant liability*".

Unfavorable U.S. or global economic conditions as a result of international conflict, or otherwise, could adversely affect our ability to raise capital and our business, results of operations and financial condition.

While the potential economic impact brought by the hostilities in the Ukraine and the Middle East are difficult to assess or predict, these conditions have resulted in, and may continue to result in, extreme volatility and disruptions in the capital and credit markets, reducing our ability to raise additional capital through equity, equity-linked or debt financings, which could negatively impact our short-term and long-term liquidity and our ability to operate in accordance with our operating plan, or at all. Additionally, our results of operations could be adversely affected by general conditions in the global economy and financial markets. A severe or prolonged economic downturn could result in a variety of risks to our business, including weakened demand for our products and services our ability to raise additional capital when needed on favorable terms, if at all. A weak or declining economy could strain our customers' budgets or cause delays in their payments to us. Additionally, inflation and surging oil and gas prices could increase our costs of production. Any of the foregoing could harm our business, and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our ability to raise capital, business, results of operations and financial condition.

We maintain our cash at financial institutions, often in balances that exceed federally insured limits.

Our cash is held in accounts at U.S. banking institutions that we believe are of high quality. Cash held in non-interest-bearing and interest-bearing operating accounts may exceed the Federal Deposit Insurance Corporation (FDIC) insurance limits. If such banking institutions were to fail, we could lose all or a portion of those amounts held in excess of such insurance limitations. Any material loss that we may experience in the future could have an adverse effect on our ability to pay our operational expenses or make other payments and may require us to move our accounts to other banks, which could cause a temporary delay in making payments to our vendors and employees and cause other operational inconveniences.

Environmental, social, and governance (ESG) matters are subject to increasing scrutiny and evolving expectations from customers, regulators, investors and other stakeholders and may expose us to reputational, cost and other risks.

Companies across all industries are subject to increasing scrutiny and evolving expectations regarding ESG matters. In particular, customers, regulators, investors and other stakeholders are increasingly focusing on environmental issues, including climate change, energy use, industrial waste, and other sustainability concerns. Failure to implement sufficient standards and practices for responsible corporate citizenship, support for local communities, employee diversity and human capital management, health and safety practices, supply chain management, and corporate governance can increase our costs of production, decrease our revenue, and negatively affect our reputation, employee retention, and the general willingness of customers and suppliers to do business with us and investors to invest in us. If we do not adapt to or comply with evolving ESG standards and regulations, the resulting consequences could have a material adverse effect on our reputation, business and financial condition.

If our facilities or our third-party manufacturers' facilities become unavailable or inoperable, our research and development program and commercialization plan could be adversely impacted and manufacturing of our products could be interrupted.

Our Redwood City, California, facilities house our corporate, research and development and quality assurance teams. Our drug product is manufactured and packaged at various locations in the United States. Our facilities in Redwood City and those of our third-party manufacturers are vulnerable to natural disasters, public health crises, climate change and catastrophic events. For example, our Redwood City facilities are located near earthquake fault zones and are vulnerable to damage from earthquakes as well as other types of disasters, including fires, wildfires, floods, power loss, communications failures and similar events. If any disaster, public health crisis or catastrophic event were to occur, our ability to operate our business would be seriously, or potentially completely, impaired. If our facilities or our third-party manufacturer's facilities become unavailable for any reason, we cannot provide assurances that we will be able to secure alternative manufacturing facilities with the necessary capabilities and equipment on acceptable terms, if at all. The inability to manufacture our drug product, combined with our limited inventory of drug product, may result in the loss of future customers or harm our reputation, and we may be unable to re-establish relationships with those customers in the future. If our or our third-party manufacturer's capabilities are impaired, we may not be able to manufacture and ship our products in a timely manner, which would adversely impact our business.

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

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

We have social media posts at X (formerly Twitter) - @SolenoTX and LinkedIn - Soleno Therapeutics, Inc. It is possible that information we post on social media channels could be deemed to be material information. The information on, or that may be accessed through, our website and social media channels is not incorporated by reference into this Quarterly Report on Form 10-Q and should not be considered a part of this Quarterly Report on Form 10-Q.

(c) ***Insider Adoption or Termination of Trading Arrangements***

During the three months ended June 30, 2025, the Company did not adopt, modify or terminate and no directors or officers, as defined in Rule 16a-1(f), adopted, modified or terminated a "Rule 10b5-1 trading arrangement" or a "non-Rule 10b5-1 trading arrangement," each as defined in Regulation S-K Item 408.

Item 6. Exhibits

See the Exhibit Index on the page immediately preceding the exhibits for a list of exhibits filed as part of this Quarterly Report on Form 10-Q, which Exhibit Index is incorporated herein by reference.

EXHIBIT INDEX

Exhibit Number	Description of Document	Incorporated by Reference from			Filed Herewith
		Registrant's Form	Date Filed with the SEC	Exhibit Number	
31.1	Certification of Principal Executive Officer Required Under Rule 13a-14(a) and 15d-14(a) of the Securities and Exchange Act of 1934, as amended				X
31.2	Certification of Principal Financial and Accounting Officer Required Under Rule 13a-14(a) and 15d-14(a) of the Securities and Exchange Act of 1934, as amended				X
32.1†	Certification of Principal Executive Officer Required Under Rule 13a-14(b) of the Securities and Exchange Act of 1934, as amended, and 18 U.S.C. §1350				X
32.2†	Certification of Principal Financial and Accounting Officer Required Under Rule 13a-14(b) of the Securities and Exchange Act of 1934, as amended, and 18 U.S.C. §1350				X
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because XBRL tags are embedded within the Inline XBRL document.				X
101.SCH	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents.				X
104	Cover Page formatted as Inline XBRL and contained in Exhibit 101.				X

† The certifications attached as Exhibit 32.1 and 32.2 that accompany this Quarterly Report on Form 10-Q, are deemed furnished and not filed with the Securities and Exchange Commission and are not to be incorporated by reference into any filing of the Registrant under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Quarterly Report on 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.

Date: August 6, 2025

SOLENO THERAPEUTICS, INC.

By: /s/ James Mackaness
James Mackaness
Chief Financial Officer
(authorized officer and principal financial and accounting officer)

Exhibit 31.1

CERTIFICATION OF THE CHIEF EXECUTIVE OFFICER
PURSUANT TO
SECURITIES EXCHANGE ACT RULES 13A-14(A) AND 15D-14(A)

I, Anish Bhatnagar, M.D., certify that:

1. I have reviewed this quarterly report on Form 10-Q of Soleno 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: August 6, 2025

/s/ Anish Bhatnagar
Anish Bhatnagar
President, Chief Executive Officer
(principal executive officer)

Exhibit 31.2

CERTIFICATION OF THE CHIEF FINANCIAL OFFICER
PURSUANT TO
SECURITIES EXCHANGE ACT RULES 13A-14(A) AND 15D-14(A)

I, James Mackaness, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Soleno 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: August 6, 2025

/s/ James Mackaness
James Mackaness
Chief Financial Officer
(principal financial and accounting officer)

Exhibit 32.1

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

In connection with the Quarterly Report of Soleno Therapeutics, Inc. (the "Company") on Form 10-Q for the fiscal quarter ended June 30, 2025, as filed with the Securities and Exchange Commission (the "Report"), Anish Bhatnagar, President, Chief Executive Officer of the Company does hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

- The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- The information in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: August 6, 2025

/s/ Anish Bhatnagar
Anish Bhatnagar
President, Chief Executive Officer
(principal executive officer)

Exhibit 32.2

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

In connection with the Quarterly Report of Soleno Therapeutics, Inc. (the "Company") on Form 10-Q for the fiscal quarter ended June 30, 2025, as filed with the Securities and Exchange Commission (the "Report"), James Mackaness, Chief Financial Officer of the Company does hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

- The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- The information in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: August 6, 2025

/s/ James Mackaness
James Mackaness
Chief Financial Officer
(principal financial and accounting officer)