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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2025
OR

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

For the transition period from _____ to _____
Commission File Number 001-38981

Mirum Pharmaceuticals, Inc.
(Exact name of registrant as specified in its charter)

Delaware	**83-1281555**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
989 East Hillsdale Boulevard, Suite 300, Foster City, California	**94404**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (650) 667-4085

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common stock, par value $0.0001 per share	MIRM	Nasdaq Global Market

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

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§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, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

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

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

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

The number of shares of registrant's common stock, par value $0.0001 per share, outstanding as of October 31, 2025 was 51,393,574.

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SUMMARY OF RISKS ASSOCIATED WITH OUR BUSINESS

An investment in shares of our common stock involves a high degree of risk. Below is a list of the more significant risks associated with our business. This summary does not address all of the risks that we face. Additional discussion of the risks listed in this summary, as well as other risks that we face, are set forth under Part II, Item 1A, "Risk Factors" in this Quarterly Report on Form 10-Q.

- The success of our business depends, in part, on our ability to market and sell our approved medicines profitably.
- If we are unable to adequately grow, maintain and scale our marketing and sales capabilities or enter into or maintain rights pursuant to agreements with third parties to market and sell our approved medicines, we may not be able to generate viable revenues.
- Our approved medicines or any one of our product candidates, if approved, may fail to achieve the market acceptance among physicians, patients and others in the medical community necessary for commercial success.
- We rely completely on third parties to manufacture and distribute our clinical and commercial drug supplies, including certain sole-source suppliers and manufacturers. These third parties may fail to obtain and maintain regulatory approval for their facilities, fail to provide us with sufficient quantities of drug substance, drug product, or labeled finished product in a timely fashion, or fail to do so at acceptable quality levels or prices.
- Our business depends, in part, on the success of our product candidates, each of which requires significant clinical testing before we can seek regulatory approval and potentially launch commercial sales.
- We have encountered and may continue to encounter delays and difficulties enrolling patients in our clinical trials, and as a result, our clinical development activities could be delayed or otherwise adversely affected.
- Our clinical trials may fail to adequately demonstrate the safety and efficacy of our product candidates, which could prevent or delay regulatory approval and commercialization.
- Clinical drug development involves a lengthy and expensive process with uncertain outcomes, and results of earlier studies and trials may not be predictive of future trial results.
- Any delays in the commencement or completion, or termination or suspension, of our clinical trials could result in increased costs for us, delay or limit our ability to generate revenue and adversely affect our commercial prospects.
- Our product candidates are subject to extensive regulation and compliance, which is costly and time consuming, and such regulation may cause unanticipated delays or prevent the receipt of the required approvals to commercialize our product candidates.
- We face significant competition from other biotechnology and pharmaceutical companies with products that may directly or indirectly compete with ours, and our operating results will suffer if we fail to compete effectively.
- We may fail to realize all of the anticipated benefits of our commercial and product candidate acquisitions or those benefits may take longer to realize than expected.
- We depend on intellectual property licensed from third parties and termination of any of these licenses could result in the loss of significant rights, which would harm our business.
- We may need substantial additional financing to continue our commercialization efforts for our approved medicines, develop our product candidates and implement our operating plans. If we fail to obtain additional financing when needed, we may be forced to delay, reduce or eliminate our product development programs or commercialization efforts.
- We do not currently have patent protection or regulatory exclusivity for certain of our approved medicines or rely on regulatory exclusivity. If we are unable to obtain and maintain sufficient intellectual property protection for our approved medicines and our product candidates, or if the scope of the intellectual property protection is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our approved medicines and our other product candidates, if approved, may be adversely affected.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

Mirum Pharmaceuticals, Inc.
Condensed Consolidated Balance Sheets
(Unaudited)
(In thousands, except share and per share data)

	September 30, 2025	December 31, 2024
		(Note 2)
Assets		
Current assets:		
Cash and cash equivalents	$ 282,021	$ 222,503
Short-term investments	93,527	57,812
Accounts receivable	107,132	78,286
Inventory	24,021	22,403
Prepaid expenses and other current assets	23,874	11,784
Total current assets	530,575	392,788
Restricted cash	480	425
Long-term investments	2,470	12,526
Property and equipment, net	1,277	1,139
Operating lease right-of-use assets	8,500	8,675
Intangible assets, net	231,895	249,819
Other assets	9,918	5,382
Total assets	$ 785,115	$ 670,754
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 12,167	$ 14,618
Accrued expenses and other current liabilities	148,192	111,933
Total current liabilities	160,359	126,551
Operating lease liabilities, noncurrent	7,361	7,972
Convertible notes payable, net, noncurrent	309,368	308,082
Other liabilities	15,981	2,509
Total liabilities	493,069	445,114
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, $0.0001 par value; 10,000,000 shares authorized, and no shares issued and outstanding as of September 30, 2025 and December 31, 2024	—	—
Common stock, $0.0001 par value; 200,000,000 shares authorized; 51,314,176 and 48,338,096 shares issued and outstanding as of September 30, 2025 and December 31, 2024, respectively	5	5
Additional paid-in capital	953,818	870,189
Accumulated deficit	(661,814)	(644,181)
Accumulated other comprehensive income (loss)	37	(373)
Total stockholders' equity	292,046	225,640
Total liabilities and stockholders' equity	$ 785,115	$ 670,754

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

Mirum Pharmaceuticals, Inc.
Condensed Consolidated Statements of Operations
(Unaudited)
(In thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Revenue:				
Product sales, net	$ 133,010	$ 90,302	$ 372,380	$ 236,979
License and other revenue	—	75	—	495
Total revenue	133,010	90,377	372,380	237,474
Operating expenses:				
Cost of sales	25,537	20,806	71,976	58,863
Research and development	42,960	31,710	135,071	96,604
Selling, general and administrative	61,910	50,545	182,902	145,391
Total operating expenses	130,407	103,061	389,949	300,858
Income (loss) from operations	2,603	(12,684)	(17,569)	(63,384)
Other income (expense):				
Interest income	3,251	3,469	9,307	10,588
Interest expense	(3,606)	(3,586)	(10,791)	(10,732)
Other income (expense), net	395	(1,087)	2,589	982
Net income (loss) before provision for income taxes	2,643	(13,888)	(16,464)	(62,546)
(Benefit from) provision for income taxes	(262)	347	1,169	1,606
Net income (loss)	$ 2,905	$ (14,235)	$ (17,633)	$ (64,152)
Net income (loss) per share, basic	$ 0.06	$ (0.30)	$ (0.35)	$ (1.36)
Net income (loss) per share, diluted	$ 0.05	$ (0.30)	$ (0.35)	$ (1.36)
Weighted-average shares of common stock outstanding, basic	50,639,231	47,782,619	49,758,104	47,316,789
Weighted-average shares of common stock outstanding, diluted	56,993,841	47,782,619	49,758,104	47,316,789

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

Mirum Pharmaceuticals, Inc.
Condensed Consolidated Statements of Comprehensive Income (Loss)
(Unaudited)
(In thousands)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Net income (loss)	$ 2,905	$ (14,235)	$ (17,633)	$ (64,152)
Other comprehensive income (loss):				
Unrealized gain on available-for-sale investments	68	291	33	298
Cumulative translation adjustments	(383)	1,106	377	(1,022)
Comprehensive income (loss)	$ 2,590	$ (12,838)	$ (17,223)	$ (64,876)

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

Mirum Pharmaceuticals, Inc.
Condensed Consolidated Statements of Stockholders' Equity
(Unaudited)
(In thousands, except share amounts)

	Common Stock		Additional Paid-In Capital	Accumulated Deficit	Accumulated Other Comprehensive Income (Loss)	Total Stockholders' Equity
	Shares	Amount				
Balance as of December 31, 2024	48,338,096	$ 5	$ 870,189	$ (644,181)	$ (373)	$ 225,640
Issuance of common stock in connection with equity award plans	1,112,939	—	6,359	—	—	6,359
Conversion of convertible notes, net	188	—	6	—	—	6
Stock-based compensation	—	—	15,813	—	—	15,813
Net loss	—	—	—	(14,677)	—	(14,677)
Other comprehensive income	—	—	—	—	115	115
Balance as of March 31, 2025	49,451,223	5	892,367	(658,858)	(258)	233,256
Issuance of common stock in connection with equity award plans	547,323	—	6,575	—	—	6,575
Issuance of common stock in connection with employee stock purchase plan	59,752	—	2,113	—	—	2,113
Conversion of convertible notes, net	94	—	3	—	—	3
Stock-based compensation	—	—	18,462	—	—	18,462
Net loss	—	—	—	(5,861)	—	(5,861)
Other comprehensive income	—	—	—	—	610	610
Balance as of June 30, 2025	50,058,392	5	919,520	(664,719)	352	255,158
Issuance of common stock in connection with equity award plans	1,255,533	—	16,096	—	—	16,096
Conversion of convertible notes, net	251	—	8	—	—	8
Stock-based compensation	—	—	18,194	—	—	18,194
Net income	—	—	—	2,905	—	2,905
Other comprehensive loss	—	—	—	—	(315)	(315)
Balance as of September 30, 2025	51,314,176	$ 5	$ 953,818	$ (661,814)	$ 37	$ 292,046

	Common Stock		Additional Paid-In Capital	Accumulated Deficit	Accumulated Other Comprehensive Income (Loss)	Total Stockholders' Equity
	Shares	Amount				
Balance as of December 31, 2023	46,723,143	$ 5	$ 803,260	$ (556,239)	$ 1,644	$ 248,670
Issuance of common stock in connection with equity award plans	337,963	—	1,205	—	—	1,205
Stock-based compensation	—	—	11,664	—	—	11,664
Net loss	—	—	—	(25,279)	—	(25,279)
Other comprehensive loss	—	—	—	—	(1,657)	(1,657)
Balance as of March 31, 2024	47,061,106	5	816,129	(581,518)	(13)	234,603
Issuance of common stock in connection with equity award plans	442,652	—	5,858	—	—	5,858
Issuance of common stock in connection with employee stock purchase plan	68,089	—	1,433	—	—	1,433
Stock-based compensation	—	—	12,196	—	—	12,196
Net loss	—	—	—	(24,638)	—	(24,638)
Other comprehensive loss	—	—	—	—	(464)	(464)
Balance as of June 30, 2024	47,571,847	5	835,616	(606,156)	(477)	228,988
Issuance of common stock in connection with equity award plans	396,274	—	3,863	—	—	3,863
Stock-based compensation	—	—	12,011	—	—	12,011
Net loss	—	—	—	(14,235)	—	(14,235)
Other comprehensive income	—	—	—	—	1,397	1,397
Balance as of September 30, 2024	47,968,121	$ 5	$ 851,490	$ (620,391)	$ 920	$ 232,024

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

Mirum Pharmaceuticals, Inc.
Condensed Consolidated Statements of Cash Flows
(Unaudited)
(In thousands)

	Nine Months Ended September 30,	
	2025	**2024**
Operating activities		
Net loss	$ (17,633)	$ (64,152)
Reconciliation of net loss to net cash provided by operating activities:		
Stock-based compensation	52,398	35,193
Depreciation and amortization	18,170	17,573
Inventory reserves and firm commitment losses	2,889	1,783
Amortization of debt discount and offering costs	1,303	1,245
Unrealized foreign exchange gain	(1,913)	(1,082)
Non-cash lease expense	1,273	985
Other	(1,019)	(425)
Change in operating assets and liabilities:		
Accounts receivable	(29,047)	(562)
Prepaid and other current assets	(11,746)	(1,122)
Inventory	(6,166)	(2,952)
Other assets	(4,026)	(1,496)
Accounts payable, accrued expenses and other liabilities	46,660	30,973
Operating lease liabilities	(1,383)	(568)
Net cash provided by operating activities	49,760	15,393
Investing activities		
Purchase of investments	(85,923)	(77,543)
Proceeds from maturities of investments	61,317	7,750
Purchase of property and equipment	(354)	(973)
Payments made for additions to intangible assets	—	(20,000)
Net cash used in investing activities	(24,960)	(90,766)
Financing activities		
Proceeds from issuance of common stock pursuant to equity plans	31,143	12,359
Net cash provided by financing activities	31,143	12,359
Effect of exchange rate on cash, cash equivalents and restricted cash	3,630	82
Net increase (decrease) in cash, cash equivalents and restricted cash	59,573	(62,932)
Cash, cash equivalents and restricted cash at beginning of period	222,928	286,326
Cash, cash equivalents and restricted cash at end of period	$ 282,501	$ 223,394
Supplemental disclosure of cash flow information:		
Operating cash flows paid for operating lease	$ 1,904	$ 881
Cash paid for interest	$ 6,325	$ 6,325
Cash paid for income taxes	$ 2,151	$ 771
Non-cash operating, investing and financing activities:		
Right-of-use assets obtained in exchange for lease liabilities	$ 1,673	$ 9,633
Decrease in ROU assets and lease liabilities due to lease modification	$ 649	$ 723
Stock-based compensation capitalized to inventory	$ 543	$ 909
Conversion of convertible notes, net into shares of common stock	$ 17	$ —

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

Mirum Pharmaceuticals, Inc.
Notes to Unaudited Condensed Consolidated Financial Statements
(Unaudited)

1. Organization and Description of Business

Mirum Pharmaceuticals, Inc. (the "Company") was incorporated in the State of Delaware on May 2, 2018, and is headquartered in Foster City, California. The Company is a biopharmaceutical company dedicated to transforming the treatment of rare diseases.

The Company has three approved medicines: LIVMARLI® (maralixibat) ("Livmarli"), Cholbam® (cholic acid) capsules ("Cholbam"), and Chenodal® or CTEXLI® (chenodiol) tablets ("Chenodal" or "Ctexli").

Livmarli is approved for the treatment of cholestatic pruritus in patients with Alagille syndrome ("ALGS") in the United States ("U.S."), the European Union ("EU") and various other countries around the world and for cholestatic pruritus in patients with primary familial intrahepatic cholestasis ("PFIC") in the U.S. and for the treatment of PFIC in the EU.

Cholbam is FDA-approved for the treatment of bile acid synthesis disorders due to single enzyme deficiencies and adjunctive treatment of peroxisomal disorders in patients who show signs or symptoms of liver disease. In February 2025, the Company received FDA approval for chenodiol tablets for the treatment of CTX in adults, which is commercialized under the brand name Ctexli. Prior to the approval of Ctexli, chenodiol tablets were commercialized under the brand name Chenodal under a medical necessity recognition from the FDA. Cholbam, Ctexli and Chenodal, collectively, are referred to as the "Bile Acid Medicines".

The Company's development pipeline consists of the clinical-stage product candidate volixibat, MRM-3379 and indication expansion opportunities for Livmarli.

The Company commenced significant operations in November 2018. The Company views its operations and manages its business as one operating segment and has determined its operating segment on the same basis that it uses to evaluate its performance internally.

Liquidity

The Company has a limited operating history, has incurred significant operating losses since its inception, and the revenue and income potential of the Company's business and market are unproven. As of September 30, 2025, the Company had an accumulated deficit of $661.8 million and unrestricted cash, cash equivalents and investments of $378.0 million. The Company's convertible notes are convertible at the option of the holders during the fourth quarter of 2025. If holders of the convertible notes elect to convert, the Company may elect to settle such conversions in cash, common stock or a combination of the two. The Company believes that its unrestricted cash, cash equivalents and investments of $378.0 million as of September 30, 2025, provide sufficient capital resources to continue its operations for at least twelve months from the issuance date of the accompanying unaudited condensed consolidated financial statements.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles ("GAAP") for interim financial information and pursuant to Form 10-Q and Article 10 of Regulation S-X of the Securities and Exchange Commission ("SEC"). Accordingly, the accompanying unaudited condensed consolidated financial statements do not include all of the information and notes required by GAAP for complete financial statements. The unaudited interim financial statements reflect all adjustments which, in the opinion of management, are necessary for a fair statement of the results for the periods presented. All such adjustments are of a normal and recurring nature. The unaudited condensed consolidated balance sheet as of December 31, 2024 has been derived from the audited consolidated financial statements at that date but does not include all information and footnotes required by GAAP for complete financial statements. The operating results presented in these unaudited condensed consolidated financial statements are not necessarily indicative of the results that may be expected for any future periods. The accompanying unaudited condensed consolidated financial statements include the accounts of the Company and its wholly owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation.

These unaudited condensed consolidated financial statements should be read in conjunction with the audited consolidated financial statements and the notes thereto in the Company's Annual Report on Form 10-K ("Annual Report") for the fiscal year ended December 31, 2024, as filed with the SEC on February 26, 2025.

Use of Estimates

The preparation of consolidated financial statements in accordance with GAAP requires management to make estimates and assumptions that impact the reported amounts. These estimates and assumptions are based upon historical experience, knowledge of current events and various other factors believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities and the recording of revenue and expenses that are not readily apparent from other sources. Actual results could differ materially from those estimates.

The Company's unaudited condensed consolidated financial statements as of and for the three and nine months ended September 30, 2025 reflect the Company's estimates of the impact of the geopolitical and macroeconomic environment, including the impact of inflation, tariffs and trade tensions, high interest rates and foreign exchange rate fluctuations. The duration and the scope of these conditions cannot be predicted; therefore, the extent to which these conditions will directly or indirectly impact the Company's business, results of operations and financial condition, is uncertain. The Company is not aware of any specific event or circumstance that would require an update to its estimates, judgments and assumptions or a revision of the carrying value of the Company's assets or liabilities as of the date of this filing.

Significant Accounting Policies

There have been no significant changes to the Company's accounting policies during the nine months ended September 30, 2025, as compared to the significant accounting policies described in Note 2 of the "Notes to Consolidated Financial Statements" in the Company's audited consolidated financial statements included in the Annual Report.

Cash, Cash Equivalents and Restricted Cash

The Company considers all highly liquid investments that are readily convertible into cash without penalty and with original maturities of three months or less at the date of purchase to be cash equivalents. The carrying amounts reported in the unaudited condensed consolidated balance sheets for cash and cash equivalents are valued at cost, which approximate their fair value.

The following table provides a reconciliation of cash, cash equivalents and restricted cash reported within the unaudited condensed consolidated balance sheets that together reflect the same amounts shown in the unaudited condensed consolidated statements of cash flows (in thousands):

	As of September 30,	
	2025	2024
Cash and cash equivalents	$ 282,021	$ 222,969
Restricted cash	480	425
Total cash, cash equivalents, and restricted cash	$ 282,501	$ 223,394

Investments

The Company classifies all investments in securities as available-for-sale. Management determines the appropriate classification of its investments in securities at the time of purchase. Investments with original maturities beyond three months at the date of purchase and which mature at, or less than twelve months from the balance sheet date, are classified as a current asset.

Investments are recorded at fair value, with unrealized gains and losses reported as accumulated other comprehensive income (loss) until realized, with the exception of any declines in fair value below the cost basis that are a result of a credit loss, which, if any, are reported in other income (expense), net in the current period through an allowance for credit losses. Each reporting period, the Company evaluates whether declines in fair values of its available-for-sale securities below their cost basis are a result of credit loss or other factors and whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. This evaluation consists of several qualitative and quantitative factors regarding the severity and duration of the unrealized loss, the creditworthiness of the security issuers, as well as the Company's ability and intent to hold the available-for-sale security until a forecasted recovery occurs. Additionally, the Company assesses whether it has plans to sell the security or it is more likely than not it will be required to sell any available-for-sale securities before recovery of its amortized cost basis. The cost of debt securities is adjusted for amortization of premiums and accretion of discounts to maturity. Such amortization and accretion, as well as interest and dividends, are included in interest income. Realized gains and losses from the sale of available-for-sale securities, if any, are determined on a specific identification basis and are also included in Other income (expense). To date, the Company has not identified any declines in fair value of its investments related to credit loss.

Concentrations of Credit Risk and Off-Balance Sheet Risk

Financial instruments that potentially subject the Company to a concentration of credit risk consist of cash and cash equivalents, accounts receivable and investments. The Company limits the amount of credit exposure by investing cash that is not required for immediate operating needs in money market funds, government obligations, corporate debt securities and/or commercial paper. Additionally, the Company has established guidelines regarding diversification of its investments and their maturities, which are designed to maintain principal and maximize liquidity. To date, the Company has not experienced any losses associated with these credit risks and continues to believe that this exposure is not significant.

The Company relies on a single third-party logistics provider ("3PL") and a single specialty pharmacy for all of the Company's sales of its approved medicines in each of the U.S. and Canada as well as a single 3PL outside of North America.

The Company sources materials and services through several vendors. Certain materials are sourced from a single vendor. The loss of certain vendors could result in a temporary disruption of the Company's commercialization efforts.

As of September 30, 2025 and December 31, 2024, the Company did not have any customer who individually accounted for 10% or more of accounts receivable. For the three and nine months ended September 30, 2025 and 2024, the Company did not have revenue attributable to any one customer in excess of 10% of sales.

Accounts Receivable

The Company has accounts receivable amounts due from product sales. The Company may also have accounts receivable amounts due from license agreements for milestones achieved, but not yet paid. Amounts payable to the Company are recorded as accounts receivable when the Company's right to consideration is unconditional. The Company estimates the allowance for credit losses using the current expected credit loss model. Under this model, the allowance for credit losses reflects the Company's estimate of lifetime expected credit losses. The Company evaluates the collectability of the cash flows based on the risk of loss over the contractual life, even when that risk is remote, based on judgments about the creditworthiness of its customers, historical experience and other relevant information that is available to the Company. There was no allowance for credit losses as of September 30, 2025. There was no bad debt expense for the three and nine months ended September 30, 2025 and 2024.

Product Sales, Net

Revenues from product sales are recorded at the net sales price, or the transaction price, which may include fixed or variable consideration for discounts, government rebates, co-pay assistance, returns and other allowances that are offered within contracts with a customer relating to the sale of the Company's approved medicines. Estimates of variable consideration are calculated based on the actual product sales each reporting period and the nature of the variable consideration related to those sales. Overall, these estimates reflect the Company's best estimate of the amount of consideration to which the Company expects to be 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 product sales, net only to the extent that it is considered probable that a significant reversal in the amount of the cumulative revenue recognized will not occur when the uncertainty associated with the variable consideration is subsequently resolved. Estimates are reviewed and updated quarterly as additional information becomes known. Actual amounts of consideration ultimately received may differ materially from estimates. If actual results vary from estimates, the Company will adjust these estimates, which would affect product sales, net and earnings in the period such variances are adjusted. Significant categories of sales deductions are as follows:

Government Rebates: The Company records rebates payable under Medicaid and other government programs as a reduction of revenue at the time product revenues are generated. The Company's rebate calculations may require estimates, including estimates of customer and payor mix and revenue projections, to determine which sales will be subject to rebates and the amount of such rebates. The Company updates its estimates and assumptions on a quarterly basis and records any necessary adjustments to revenue in the period identified. Government rebate payments vary in timing depending on the nature, and are generally quarterly or annually.

Other Incentives: Other incentives include a branded co-pay assistance program for eligible patients with commercial insurance in the United States. The branded co-pay assistance program assists commercially insured patients who have coverage for the Company's approved medicines and is intended to reduce each participating patient's portion of the financial responsibility of the purchase price up to a specified dollar amount of assistance. The calculation of the accrual for co-pay assistance is based upon an identification of claims and the cost per claims associated with product that

has been recognized as revenue. The Company records amounts paid under the brand specific co-pay assistance program for each patient as a reduction of revenue from product sales.

Product Returns: The Company records revenue for product sales, net of estimated product returns. Customers have limited return rights related only to the product's damage or defect identified upon delivery of the product. The Company estimates the amount of product sales that may be returned and records the estimate as a reduction of revenue and a liability in the period the related product revenue is recognized.

The Company relies on a specialty pharmacy for all of its sales of its approved medicines in each of the U.S. and Canada. Outside of North America, the Company's approved medicines are sold to its authorized distributors, licensed partners or directly to government purchasers or hospitals, which act as end users. For revenues from distributors and the Company's licensed partner in Japan, Takeda, the Company records net product sales at the time control of the product is transferred, based on the estimated variable consideration. The transaction price, which may include fixed or variable consideration may be subject to constraint and is included in the product sales price only to the extent that it is probable that a significant reversal of the amount of the cumulative revenue recognized will not occur in a future period. The Company recognizes its best estimate of the consideration expected to be received when control of the inventory is transferred. Such estimates may be complex and include estimates as to if and when the distributors and licensed partner's sales in the market will occur. Estimates are reviewed and updated as additional information, including in-market sales information of our authorized distributors and licensed partners, becomes known. Actual amounts may ultimately differ from the Company's estimates. If actual results vary, the Company adjusts these estimates, which could have an effect on earnings in the period of adjustment. The Company is entitled to payment in connection with the supply of product under standard industry payment terms. Actual consideration amounts are determined and settled generally quarterly or annually. If the consideration received or receivable exceeds the Company's estimates of product sales, the Company recognizes a liability. If the estimated product sales exceed the payment received or receivable, the Company records a receivable or contract asset related to the amounts not yet collected, depending on the circumstances. As of September 30, 2025, the Company did not have any contract assets and recorded a liability of $3.6 million related to consideration received in excess of the Company's estimated net product sales.

Liabilities associated with sales deductions are included in accrued expenses and other current liabilities or in other liabilities on the accompanying unaudited condensed consolidated balance sheets depending on contractual settlement timelines.

The following table represents total revenues and disaggregates Product sales, net by approved medicine (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Product sales, net:				
Livmarli	$ 92,235	$ 59,126	$ 253,619	$ 149,202
Bile Acid Medicines	40,775	31,176	118,761	87,777
Total product sales, net	133,010	90,302	372,380	236,979
License and other revenue	—	75	—	495
Total revenues	$ 133,010	$ 90,377	$ 372,380	$ 237,474

The following table sets forth Product sales, net by geographic area based on the ship-to location (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
United States	$ 103,713	$ 73,905	$ 286,167	$ 195,096
Rest of the world	29,297	16,397	86,213	41,883
Total product sales, net	$ 133,010	$ 90,302	$ 372,380	$ 236,979

Foreign Currency

The unaudited condensed consolidated financial statements are presented in U.S. dollars. The functional currency for most of the Company's foreign subsidiaries is their local currency. Balance sheet accounts of international subsidiaries are translated at the current exchange rates as of the end of each accounting period. Income statement items are

translated at average exchange rates for the period. The resulting translation adjustments are recorded as a separate component of stockholders' equity.

Foreign currency transaction gains and losses are included in other income (expense), net in the unaudited condensed consolidated statements of operations. Transaction gains and losses result primarily from fluctuations in exchange rates when intercompany receivables and payables are denominated in currencies other than the functional currency of our subsidiary that recorded the transaction. Unrealized foreign exchange gains amounted to $0.6 million for the three months ended September 30, 2025 and unrealized foreign exchange losses amounted to $1.0 million for the three months ended September 30, 2024. Unrealized foreign exchange gains amounted to $1.9 million and $1.1 million for the nine months ended September 30, 2025 and 2024, respectively. Realized foreign exchange losses amounted to $0.2 million and $1.0 million for the three and nine months ended September 30, 2025, and were not material for the three and nine months ended September 30, 2024.

Net Income (Loss) Per Share

Basic net income (loss) per share is computed by dividing net income (loss) by the weighted-average shares of common stock outstanding for the period, without consideration for potentially dilutive securities. Diluted net income (loss) per share is computed by dividing the net income (loss) by the weighted-average shares of common stock and potentially dilutive securities outstanding for the period determined using the treasury-stock and if-converted methods. Diluted net income (loss) per share excludes the potential impact of the Company's common stock subject to repurchase, common stock options, restricted stock units, contingently issuable employee stock purchase plan shares and common stock issuable upon conversion of convertible notes to the extent their effect would be anti-dilutive. Basic and diluted net income (loss) per share were the same for the nine months ended September 30, 2025 and 2024 and for the three months ended September 30, 2024.

The following table sets forth the computation of basic and diluted earnings per share for the three and nine months ended September 30, 2025 and 2024 (in thousands, except share and per share data):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Numerator:				
Net income (loss), basic and diluted	$ 2,905	$ (14,235)	$ (17,633)	$ (64,152)
Denominator:				
Weighted-average shares of common stock outstanding, basic	50,639,231	47,782,619	49,758,104	47,316,789
Effect of dilutive securities:				
Options to purchase common stock and restricted stock units	6,339,795	—	—	—
Employee stock purchase plan contingently issuable	14,815	—	—	—
Weighted-average shares of common stock outstanding, diluted	56,993,841	47,782,619	49,758,104	47,316,789
Net income (loss) per share, basic	$ 0.06	$ (0.30)	$ (0.35)	$ (1.36)
Net income (loss) per share, diluted	$ 0.05	$ (0.30)	$ (0.35)	$ (1.36)

The following outstanding potential dilutive shares have been excluded from the calculation of diluted net income (loss) per share for the periods presented due to their anti-dilutive effect:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Options to purchase common stock and restricted stock units	1,881,060	11,853,261	11,183,252	11,853,261
Common stock issuable upon conversion of convertible notes	9,963,711	9,964,247	9,963,711	9,964,247
Employee stock purchase plan contingently issuable	—	69,109	50,124	69,109
Total	11,844,771	21,886,617	21,197,087	21,886,617

Recent Accounting Pronouncements Not Yet Adopted

From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board ("FASB") or other standard setting bodies and adopted by the Company as of the specified effective date. Unless otherwise discussed, the Company believes that the impact of recently issued standards that are not yet effective will not have a material impact on the accompanying unaudited condensed consolidated financial statements and disclosures.

In December 2023, the FASB issued ASU No. 2023-09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures* ("ASU 2023-09"). This new guidance is designed to enhance the transparency and decision usefulness of income tax disclosures. The amendments of this update are related to the rate reconciliation and income taxes paid. ASU 2023-09 is effective for fiscal years beginning after December 15, 2024. The Company does not expect that adoption of this new accounting standard will have a material impact on its consolidated financial statements.

In November 2024, the FASB issued ASU 2024-03, *Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses* ("ASU 2024-03"), which requires disclosure of disaggregated information about specific categories underlying certain income statement expense line items in the footnotes to the financial statements for both annual and interim periods. Subsequently in January 2025, the FASB issued ASU 2025-01, *Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Clarifying the Effective Date* ("ASU 2025-01") to clarify the effective date of ASU 2024-03. ASU 2024-03 is effective for fiscal years beginning after December 15, 2026, and interim periods within annual reporting periods beginning after December 15, 2027. Early adoption is permitted. The Company is currently assessing the impact that adopting this new accounting standard will have on its consolidated financial statements.

In July 2025, the FASB issued ASU 2025-05, *Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses for Accounts Receivable and Contract Assets* ("ASU 2025-05"). The new guidance provides a practical expedient related to the estimation of expected credit losses for current accounts receivable and current contract assets that arise from transactions accounted for under FASB Accounting Standards Codification 606. ASU 2025-05 is effective for annual reporting periods beginning after December 15, 2025, and interim reporting periods within those annual reporting periods. Early adoption is permitted. The Company does not expect that adoption of this new accounting standard will have a material impact on its consolidated financial statements.

In September 2025, the FASB issued ASU 2025-06, *Intangibles - Goodwill and Other - Internal-Use Software (Subtopic 350-40): Targeted Improvements to the Accounting for Internal-Use Software* ("ASU 2025-06"), to modernize the accounting guidance for the costs to develop software for internal use by aligning it with current development practices, especially agile and iterative methods. The guidance removes references to development stages and clarifies when capitalization of software costs should occur. The new guidance also clarifies disclosure requirements for all capitalized internal-use software costs, ASU 2025-06 is effective for annual reporting periods beginning after December 15, 2027, and interim reporting periods within those annual reporting periods. Early adoption is permitted as of the beginning of an annual reporting period. The Company is currently assessing the impact that adopting this new accounting standard will have on its consolidated financial statements.

3. Fair Value Measurements

Financial assets and liabilities subject to fair value measurements on a recurring basis and the level of inputs used in such measurements by major security type are presented in the following table (in thousands):

	September 30, 2025			
	Level 1	Level 2	Level 3	Total
Financial assets:				
Money market funds	$ 229,140	$ —	$ —	$ 229,140
Corporate debt securities	—	71,571	—	71,571
U.S. government bonds	—	20,003	—	20,003
Commercial paper	—	5,422	—	5,422
Total financial assets	$ 229,140	$ 96,996	$ —	$ 326,136

	December 31, 2024			
	Level 1	Level 2	Level 3	Total
Financial assets:				
Money market funds	$ 202,965	$ —	$ —	$ 202,965
U.S. treasury bills	$ 2,964	$ —	$ —	$ 2,964
Corporate debt securities	$ —	$ 37,978	$ —	$ 37,978
U.S. government bonds	$ —	$ 26,904	$ —	$ 26,904
Agency bonds	$ —	$ 2,492	$ —	$ 2,492
Total financial assets	$ 205,929	$ 67,374	$ —	$ 273,303

The carrying amounts of certain financial instruments such as cash and cash equivalents, accounts receivable, prepaid expenses, other current assets, accounts payable and accrued expenses as of September 30, 2025 and December 31, 2024 approximate their related fair values due to their short-term nature.

Money market funds and U.S. treasury bills are highly liquid investments and are actively traded. The pricing information on these investment instruments is readily available and can be independently validated as of the measurement date. This approach results in the classification of these securities as Level 1 of the fair value hierarchy.

Certain financial instruments classified within Level 2 of the fair value hierarchy include the types of instruments that trade in markets that are not considered to be active, but are valued based on quoted market prices, broker or dealer quotations, or alternative pricing sources with reasonable levels of price transparency. The Company reviews trading activity and pricing for these investments as of each measurement date.

4. Financial Instruments

The fair value and amortized cost of cash equivalents and available-for-sale investments by major security type are presented in the following table (in thousands):

	September 30, 2025			
	Amortized Cost	Unrealized Gain	Unrealized Loss	Estimated Fair Value
Cash equivalents and investments:				
Money market funds	$ 229,140	$ —	$ —	$ 229,140
Corporate debt securities	71,446	126	(1)	71,571
U.S. government bonds	19,966	37	—	20,003
Commercial paper	5,420	2	—	5,422
Total cash equivalents and investments	$ 325,972	$ 165	$ (1)	$ 326,136
Classified as:				
Cash equivalents				$ 230,139
Short-term investments				93,527
Long-term investments				2,470
Total cash equivalents and investments				$ 326,136

	December 31, 2024			
	Amortized Cost	Unrealized Gain	Unrealized Loss	Estimated Fair Value
Cash equivalents:				
Money market funds	$ 202,965	$ —	$ —	$ 202,965
U.S. treasury bills	$ 2,957	$ 7	$ —	$ 2,964
Corporate debt securities	$ 37,942	$ 50	$ (14)	$ 37,978
U.S. government bonds	$ 26,819	$ 90	$ (5)	$ 26,904
Agency bonds	$ 2,490	$ 2	$ —	$ 2,492
Total cash equivalents and investments	$ 273,173	$ 149	$ (19)	$ 273,303
Classified as:				
Cash equivalents				$ 202,965
Short-term investments				57,812
Long-term investments				12,526
Total cash equivalents and investments				$ 273,303

As of September 30, 2025, the remaining contractual maturities of available-for-sale debt securities were as follows (in thousands):

	Estimated Fair Value
Due within one year	$ 94,526
One to two years	2,470
Total	$ 96,996

During the three and nine months ended September 30, 2025 and 2024, there have been no significant realized gains or losses on available-for-sale investments, no investments have been in a continuous unrealized loss position for more than 12 months, and the Company did not recognize any material unrealized gains or losses on these securities.

5. Balance Sheet Components

Inventory

Inventory consists of the following (in thousands):

	September 30, 2025	December 31, 2024
Raw materials	$ 2,869	$ 3,030
Work in progress	18,305	16,089
Finished goods	2,847	3,284
Total inventory	$ 24,021	$ 22,403

Intangible Assets, Net

The components of the Company's intangible assets were as follows (in thousands, except for weighted-average remaining amortization period):

	September 30, 2025			
	Gross Carrying Value	Accumulated Amortization	Net Carrying Amount	Weighted-Average Remaining Amortization Period (Years)
Commercial milestones	$ 59,000	$ (10,518)	$ 48,482	11.9
Developed technology	226,620	(43,503)	183,117	10.0
Assembled workforce	970	(674)	296	0.9
Total intangible assets	$ 286,590	$ (54,695)	$ 231,895	10.4

	December 31, 2024			
	Gross Carrying Value	Accumulated Amortization	Net Carrying Amount	Weighted-Average Remaining Amortization Period (Years)
Commercial milestones	$ 59,000	$ (7,092)	$ 51,908	12.6
Developed technology	226,620	(29,248)	197,372	10.7
Assembled workforce	970	(431)	539	1.7
Total intangible assets	$ 286,590	$ (36,771)	$ 249,819	11.1

Amortization expense was $6.0 million and $17.9 million for the three and nine months ended September 30, 2025, respectively, and $5.9 million and $17.1 million for the three and nine months ended September 30, 2024, respectively. Amortization expense was included in cost of sales in the accompanying unaudited condensed consolidated statements of operations. The following table summarizes the estimated future amortization expense associated with the Company's intangible assets as of September 30, 2025 (in thousands):

	Amount
2025 (remaining three months)	5,974
2026	23,791
2027	23,575
2028	23,575
2029	23,575
Thereafter	131,405
	$ 231,895

Accrued Expenses

Accrued expenses consist of the following (in thousands):

	September 30, 2025	December 31, 2024
Accrued sales deductions	$ 55,916	$ 37,361
Accrued compensation and related benefits	36,981	32,248
Accrued royalties payable	12,572	9,361
Accrued professional service fees	12,889	12,545
Accrued clinical trials	11,190	7,148
Accrued contract manufacturing and non-clinical costs	8,990	7,555
Accrued interest	5,271	2,108
Accrued loss on firm purchase commitments	2,348	1,898
Operating lease liabilities, current	2,035	1,709
Total accrued expenses and other current liabilities	$ 148,192	$ 111,933

Other liabilities

Other liabilities consist of the following (in thousands):

	September 30, 2025	December 31, 2024
Noncurrent accrued sales deductions and other	$ 12,449	$ —
Noncurrent accrued loss on firm purchase commitments	3,532	2,509
Total other liabilities	$ 15,981	$ 2,509

6. Asset Acquisitions

License Agreement with Enthorin Therapeutics, LLC

In October 2024, the Company entered into a license agreement with Enthorin Therapeutics, LLC and Dart Neuroscience LLC ("Enthorin"), under which the Company acquired the worldwide rights to develop, manufacture and commercialize a compound designated as ENT-3379, renamed MRM-3379, in exchange for an upfront payment of $7.5 million and up to an additional $217.5 million upon the achievement of regulatory and sales-based milestones as well as mid-single digit percent royalties on any future sales of MRM-3379. Through September 30, 2025, no milestones have been achieved.

Asset Purchase Agreement with Travere Therapeutics, Inc.

On August 31, 2023, the Company completed the acquisition of assets of Travere Therapeutics, Inc. ("Travere"). In accordance with the terms and conditions of the Asset Purchase Agreement entered into with Travere, the Company purchased from Travere substantially all of the assets related to its business of development, manufacturing (including synthesis, formulation, finishing or packaging) and commercialization of the Bile Acid Medicines. The Company paid $210.4 million upon closing of the transaction, and up to an additional $235.0 million is payable upon the achievement of certain milestones based on specified amounts of annual net sales of the Bile Acid Medicines. Through September 30, 2025, no milestones have been achieved.

The Company is obligated to pay tiered royalties, based on licensing agreements acquired with the Bile Acid Medicines, with rates ranging from high single digit to mid-teens based on net sales of the Bile Acid Medicines.

Assignment and License Agreement with Shire International GmbH (Takeda)

In November 2018, the Company entered into an Assignment and License Agreement (the "Shire Agreement") with Shire International GmbH ("Shire"), which was subsequently acquired by Takeda Pharmaceutical Company Limited ("Takeda"). Under the terms of the Shire Agreement, Shire granted the Company an exclusive, royalty bearing worldwide license to develop and commercialize its two product candidates, Livmarli and volixibat. As part of the Shire Agreement, the Company was assigned license agreements held by Shire with Satiogen Pharmaceuticals, Inc. ("Satiogen"), Pfizer Inc. ("Pfizer") and Sanofi-Aventis Deutschland GmbH ("Sanofi"). The Company has the right to sublicense under the Shire

Agreement and additionally has the right to sublicense under the Satiogen, Pfizer and Sanofi licenses subject to the terms of those license agreements.

The Company is obligated to pay Shire up to an aggregate of $109.5 million upon the achievement of certain clinical development and regulatory milestones for Livmarli in certain indications and an additional $25.0 million upon regulatory approval of Livmarli for each and every other indication. In addition, the Company is required to pay up to an aggregate of $30.0 million upon the achievement of certain clinical development and regulatory milestones for volixibat solely for the first indication sought. Upon commercialization, the Company is obligated to pay Shire product sales milestones on total licensed products up to an aggregate of $30.0 million. Through September 30, 2025 under this agreement, the Company paid or accrued $91.5 million for the achievement of various clinical development, regulatory and commercial milestones.

The Company is also obligated to pay tiered royalties with rates ranging from low double-digits to mid-teens based upon annual worldwide net sales for all licensed products; however, these royalties are reduced in part by royalties due under the Satiogen and Sanofi licenses, as discussed below, related to Livmarli and volixibat, as applicable. The Company's royalty obligations will continue on a licensed product-by-licensed product and country-by-country basis until the later to occur of the expiration of the last valid claim in a licensed patent covering the applicable licensed product in such country, expiration of any regulatory exclusivity for the licensed product in a country and ten years after the first commercial sale of a licensed product in such country. During the nine months ended September 30, 2024, the Company achieved the $10.0 million regulatory milestone associated with the approval of Livmarli for the treatment of cholestatic pruritus in patients with PFIC five years of age and older (now twelve months of age and older) by the FDA and the $10.0 million associated with the approval of Livmarli for the treatment of cholestatic pruritus in patients with PFIC three months and older by the European Medicines Agency, both of which were capitalized as intangible assets. During the nine months ended September 30, 2025, the Company achieved a $5.0 million development milestone related to maralixibat, which was recognized in research and development expense in the accompanying unaudited condensed consolidated statements of operations. There were no volixibat development and regulatory milestones achieved during the three and nine months ended September 30, 2025 and 2024.

Satiogen License

Through the Shire Agreement, the Company was assigned a license agreement with Satiogen pursuant to which the Company obtained an exclusive, worldwide license to certain patents and know-how, with the right to sublicense to a third party subject to certain financial considerations. Pursuant to the terms of the license agreement, the Company was obligated to pay to Satiogen up to an aggregate of $10.5 million upon the achievement of certain milestones, of which $0.5 million was for initiation of certain development activities, $5.0 million for the completion of regulatory approvals and $5.0 million for commercialization activities. Additionally, the Company was required to pay a low single-digit royalty on net sales. The Company's royalty obligations continued on a licensed product-by-licensed product and country-by-country basis until the expiration of the last valid claim in a licensed patent covering the applicable licensed product in such country. Royalty obligations under the Satiogen license were creditable against the royalty obligations to Shire under the Shire Agreement.

In May 2022, the Company completed the merger and acquisition of Satiogen. Through the transaction, the Company obtained all Satiogen licensing payments and Satiogen-owned intellectual property relating to Livmarli and volixibat. The transaction resulted in a reduction of total licensing royalty obligations for Livmarli and volixibat.

Pfizer License

Through the Shire Agreement, the Company was assigned a license agreement with Pfizer pursuant to which the Company obtained an exclusive, worldwide license to certain Pfizer know-how with a right to sublicense. Upon commercialization of any product utilizing the licensed product, the Company pays to Pfizer a low single-digit royalty on net sales of product sold by the Company, its affiliates or sublicensees. The Company's royalty obligations continue on a licensed product-by-licensed product basis until the eighth anniversary of the first commercial sale of such licensed product anywhere in the world.

Sanofi License

Through the Shire Agreement, the Company was assigned a license agreement with Sanofi pursuant to which the Company obtained an exclusive, worldwide license to certain patents and know-how with the right to sublicense to a third party subject to certain financial considerations. The Company is obligated to pay up to an aggregate of $36.0 million upon the achievement of certain regulatory, commercialization and product sales milestones. Additionally, upon commercialization, the Company is required to pay tiered royalties in the mid to high single-digit range based upon net sales of licensed products sold by the Company and sublicensees in a calendar year, subject to adjustments in certain circumstances. The Company's royalty obligations continue on a licensed product-by-licensed product and country-by-

country basis until the later to occur of the expiration of the last valid claim in a licensed patent covering the applicable licensed product in such country and ten years after the first commercial sale of a licensed product in such country. Royalty obligations under the Sanofi license are creditable against the royalty obligations to Shire under the Shire Agreement. The Company has not paid milestone payments pursuant to this agreement for the periods presented. Through September 30, 2025, no milestones have been achieved.

7. License Agreements

Licensing Agreement with Takeda

In September 2021, the Company entered into an exclusive licensing agreement with Takeda for the development and commercialization of Livmarli in Japan for ALGS, PFIC, and BA. Further, in October 2024, the parties entered into a commercial supply agreement. Under the terms of the agreements, Takeda is responsible for development and commercialization of Livmarli for licensed indications in Japan, while the Company is responsible for commercial supply to Takeda. In accordance with the agreements, in exchange for commercial inventory supply, the Company is eligible to receive a percentage of Takeda's net sales, which range from high double digits declining to mid double digits over the first four years from commercial launch and thereafter remains at mid double digits. The Company records net product sales at the time control of the product is transferred, based on the estimated variable consideration.

8. Leases

The Company has operating leases for office spaces in various locations, including its headquarters in Foster City, the lease for which was entered into in January 2024 and has a lease term of approximately five years. In March 2025, the Company entered into an operating lease agreement for office space at an international location. The lease commenced in the second quarter of 2025 and has a lease term of approximately five years.

The following tables contain a summary of other information and the undiscounted future minimum payments pertaining to the Company's operating leases that had commenced as of the end of the periods presented:

	September 30, 2025
Weighted-average incremental borrowing rate	7.4%
Weighted-average remaining lease term (in years)	4.0 years

	Undiscounted Rent Payments as of September 30, 2025 (in thousands)
2025 (remaining three months)	$ 658
2026	2,668
2027	2,733
2028	2,734
2029	1,948
Thereafter	99
Total undiscounted lease payments	10,840
Less: imputed interest	(1,444)
Total lease liability	$ 9,396

Rent expense was $0.6 million and $1.8 million for the three and nine months ended September 30, 2025, respectively, and $0.6 million and $1.3 million for the three and nine months ended September 30, 2024, respectively. Variable lease payments for operating expenses for the three and nine months ended September 30, 2025 and 2024 were not material.

9. Convertible Notes

Except as described below, the Company's convertible notes are described in Note 10 of the "Notes to Consolidated Financial Statements" in the Annual Report.

The convertible notes consisted of the following (in thousands):

	September 30, 2025	December 31, 2024
Principal amount	$ 316,233	$ 316,250
Unamortized debt discount and issuance costs	(6,865)	(8,168)
Net carrying amount	$ 309,368	$ 308,082

For each of the fourth quarter of 2024 and the first three quarters of 2025, the last reported sale price of the Company's common stock exceeded 130% of the conversion price of the 4.00% Convertible Senior Notes due 2029 (the "Notes") for more than 20 trading days during the 30 consecutive trading days ended on each respective quarter end. As a result, for the quarterly periods ended March 31, 2025, June 30, 2025 and September 30, 2025, respectively, the Notes were, and for the quarterly period ending December 31, 2025, the Notes are, convertible at the option of the holders of the Notes. During the three and nine months ended September 30, 2025 and through the date of this filing, the amount of the principal balance of the Notes that has been converted was not material.

The Company incurred $10.9 million of transaction costs related to the issuance of the Notes, which are being amortized to interest expense over the term of the Notes using the effective interest method. As of September 30, 2025, the remaining amortization period of the debt discount was approximately 3.6 years and the effective interest on the Notes was 4.6%. The following table sets forth interest expense recognized related to the Notes (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Coupon interest expense	$ 3,163	$ 3,162	$ 9,488	$ 9,487
Amortization of debt discount and issuance costs	443	424	1,303	1,245
Total interest expense on convertible notes	$ 3,606	$ 3,586	$ 10,791	$ 10,732

As of September 30, 2025 and December 31, 2024, the estimated fair value of the Notes was $760.9 million and $481.9 million, respectively. The fair values were determined based on the quoted price of the convertible notes in an inactive market on the last trading day of the reporting period and have been classified as Level 2 in the fair value hierarchy.

10. Stockholders' Equity

Common Stock

On August 12, 2025, the Company filed an automatic shelf registration statement on Form S-3 with the SEC (the "2025 Shelf Registration"), which became effective upon filing, pursuant to which the Company registered for sale from time to time in one or more offerings an unlimited amount of any combination of the Company's common stock, preferred stock, debt securities and warrants, so long as the Company continues to satisfy the requirements of a "well-known seasoned issuer" under SEC rules. This automatic shelf registration statement will remain in effect for up to three years from the date it became effective. As of September 30, 2025, the Company had not issued any securities pursuant to the automatic shelf registration statement.

On November 2, 2023, the Company entered into a Sales Agreement (the "2023 Sales Agreement") with Leerink and Cantor Fitzgerald & Co. (the "Sales Agents"), pursuant to which the Company may, from time to time, sell up to an aggregate amount of $200.0 million of its common stock through the Sales Agents in an "at-the-market" offering (the "ATM Offering"). The Company is not required to sell shares under the 2023 Sales Agreement. Sales of the Company's common stock, if any, under the 2023 Sales Agreement may be made in any transactions that are deemed to be "at the market offerings" as defined in Rule 415 under the Securities Act. The Company will pay a given designated Sales Agent a commission of up to 3.0% of the aggregate gross proceeds of any shares of common stock sold through such Sales Agent pursuant to the 2023 Sales Agreement. As of September 30, 2025, the Company had not issued any securities pursuant to the 2023 Sales Agreement.

Common Stock Reserved for Issuance

Common stock reserved for issuance is as follows:

	As of September 30, 2025
Stock options, restricted stock units and performance stock units issued and outstanding	11,183,252
Reserved for future stock awards or option grants	3,804,599
Reserved for employee stock purchase plan	1,806,244
Common stock issuable upon conversion of convertible notes	9,963,711
	26,757,806

11. Stock-Based Compensation

Stock Options

The following table summarizes stock option activity during the nine months ended September 30, 2025 (in thousands, except share and per share data):

	Number of Awards	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life (in Years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2024	10,031,486	$ 18.12	6.5	$ 233,161
Granted	1,740,525	$ 47.83		
Exercised	(2,151,412)	$ 13.50		
Canceled and forfeited	(206,252)	$ 32.99		
Outstanding as of September 30, 2025	9,414,347	$ 24.35	6.6	$ 460,953
Vested and exercisable as of September 30, 2025	5,934,048	$ 16.91	5.5	$ 334,682

The weighted-average grant date fair value per share of stock options granted during the nine months ended September 30, 2025 and 2024 was $31.86 and $19.38 per share, respectively. The total intrinsic value of options exercised during the nine months ended September 30, 2025 and 2024 was $94.1 million and $14.3 million, respectively. Intrinsic value is calculated as the difference between the exercise price of the underlying options and the fair value of the common stock for the options that had exercise prices that were lower than the per share fair value of the common stock on the date of exercise. As of September 30, 2025, the total unrecognized stock-based compensation related to unvested stock option awards granted was $76.5 million, which the Company expects to recognize over a weighted-average period of approximately 2.8 years.

The fair value of each employee and non-employee stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. Due to the Company's limited operating history and a lack of company specific historical and implied volatility data, the expected stock price volatility was based upon the weighting of the Company's historical volatility and the historical volatility of a peer group of publicly traded companies. The historical volatility data was computed using the daily closing prices for the Company's and its peer companies' shares during the equivalent period of the calculated expected term of the stock-based awards. Due to the lack of historical exercise history, the expected term of the Company's stock options for employees has been determined utilizing the "simplified" method for awards. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is zero based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

The following assumptions were used to estimate the fair value of stock option awards granted during the following periods:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Expected term (in years)	6.08-6.08	6.08-6.08	5.50-6.08	5.50-6.08
Expected volatility	68.98%-69.30%	73.10%-76.30%	68.98%-71.31%	72.34%-80.36%
Risk-free interest rate	3.69%-4.03%	3.48%-4.24%	3.69%-4.65%	3.48%-4.62%
Expected dividend yield	—	—	—	—

Restricted Stock Units

The following table summarizes the activity under the Company's restricted stock units for the nine months ended September 30, 2025:

	Number of Awards	Weighted-Average Grant Date Fair Value per Award
Unvested and outstanding as of December 31, 2024	1,392,562	$ 26.63
Granted	727,086	$ 47.76
Vested	(616,708)	$ 24.82
Cancelled/Forfeited	(87,778)	$ 35.22
Unvested and outstanding as of September 30, 2025	1,415,162	$ 37.74

The fair value of restricted stock unit awards granted to employees and nonemployees is equal to the closing market price of the Company's common stock on the grant date.

As of September 30, 2025, the total unrecognized stock-based compensation related to restricted stock unit awards granted was $39.0 million, which the Company expects to recognize over a weighted-average period of approximately 2.0 years.

Performance Stock Units

The fair value of performance stock units ("PSUs") granted to employees is equal to the closing market price of the Company's common stock on the grant date. PSUs are subject to vest only if certain specified sales-based criteria are achieved and the employees' continued service with the Company. As of September 30, 2025, certain specified sales-based criteria were deemed probable of achievement or already achieved. Stock-based compensation for PSUs is recognized over the service period beginning in the period the Company determines it is probable that the performance criteria will be achieved. PSUs generally vest over a three-year service period. The number of shares earned is adjusted based on the specified sales-based criteria achievement.

The following table summarizes the activity under the Company's performance stock units for the nine months ended September 30, 2025:

	Number of Awards	Weighted-Average Grant Date Fair Value per Award
Unvested and outstanding as of December 31, 2024	284,955	$ 25.15
Granted	217,547	$ 40.18
Vested	(147,675)	$ 23.92
Cancelled/Forfeited	(1,084)	$ 25.39
Unvested and outstanding as of September 30, 2025	353,743	$ 34.91

As of September 30, 2025, the total unrecognized stock compensation related to performance stock units granted was $11.9 million, which the Company expects to recognize over a weighted-average period of approximately 1.6 years.

Compensation Expense

Total stock-based compensation is reflected in the accompanying unaudited condensed consolidated statements of operations as follows (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Selling, general and administrative	$ 12,122	$ 8,018	$ 32,971	$ 23,578
Research and development	5,676	3,571	18,529	10,978
Cost of sales	329	319	898	637
Total	$ 18,127	$ 11,908	$ 52,398	$ 35,193

Stock-based compensation capitalized into inventory was $0.2 million for each of the three months ended September 30, 2025 and 2024, and $0.5 million and $0.9 million for the nine months ended September 30, 2025 and 2024, respectively.

12. Segment Reporting

The Company's chief operating decision maker ("CODM"), the Chief Executive Officer, manages the Company's operations and business as one operating segment and allocates resources to operations of the Company on an entity-wide basis. The CODM assesses performance of the Company and determines resource allocation primarily based on net product sales and income (loss) from operations on a consolidated basis. The CODM uses income (loss) from operations to monitor budget versus actual results and considers any adjustments and actions required for good fiscal management.

The Company's CODM is regularly provided with entity-wide expense categories similar to those found in the consolidated statements of operations, as well as the following (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Revenue:				
Product sales, net	$ 133,010	$ 90,302	$ 372,380	$ 236,979
License and other revenue	—	75	—	495
Total revenue	133,010	90,377	372,380	237,474
Less:				
Cost of sales (excluding intangible amortization and other non-cash expenses)	19,643	13,118	54,294	35,614
General and administrative expenses (excluding stock-based compensation)	13,189	10,796	37,960	32,818
Commercialization and Medical Affairs expenses (excluding stock-based compensation)	36,599	31,731	111,971	88,995
Research and development expenses (excluding stock-based compensation)	37,284	28,139	116,542	85,626
Stock-based compensation	17,798	11,589	51,500	34,556
Intangible amortization and other non-cash expenses	5,894	7,688	17,682	23,249
Income (loss) from operations	$ 2,603	$ (12,684)	$ (17,569)	$ (63,384)

13. Commitments and Contingencies

Certain of the Company's contractual arrangements with contract manufacturing organizations require binding forecasts or commitments to purchase minimum amounts for the manufacture of drug product supply, which may be material to the Company's unaudited condensed consolidated financial statements.

The Company is subject to potential liabilities under government regulations and various claims and legal actions that are pending or may be asserted from time-to-time. These matters arise in the ordinary course and conduct of the Company's business and may include, for example, commercial, intellectual property, and employment matters. The

Company intends to defend itself vigorously in such matters and when warranted, take legal action against others. Furthermore, the Company regularly assesses contingencies to determine the degree of probability and range of possible loss for potential accrual in its unaudited condensed consolidated financial statements.

An estimated loss contingency is accrued in the Company's unaudited condensed consolidated financial statements if it is probable that a liability has been incurred and the amount of the loss can be reasonably estimated. The Company does not accrue amounts for liabilities that it does not believe are probable. Litigation is inherently unpredictable, and unfavorable resolutions could occur. As a result, assessing contingencies is highly subjective and requires judgment about future events. During the periods presented, the Company has not recorded any accrual for loss contingencies associated with government regulations, claims or legal actions, determined that an unfavorable outcome is probable or reasonably possible, or determined that the amount or range of any possible loss is reasonably estimable.

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

You should read the following discussion and analysis of our financial condition and results of operations in conjunction with our unaudited condensed consolidated financial statements and the related notes and other financial information included elsewhere in this Quarterly Report on Form 10-Q and our audited consolidated financial statements and notes thereto and the related Management's Discussion and Analysis of Financial Condition and Results of Operations included in our Annual Report on Form 10-K ("Annual Report") for the year ended December 31, 2024, which was filed with the Securities and Exchange Commission ("SEC") on February 26, 2025. Unless the context requires otherwise, references in this Quarterly Report on Form 10-Q to the "Company," "Mirum," "we," "us" and "our" refer to Mirum Pharmaceuticals, Inc. and its consolidated subsidiaries.

Forward-Looking Statements

In addition to historical financial information, this discussion and analysis contains forward-looking statements based upon current expectations that involve risks and uncertainties. Our actual results could differ materially from those anticipated in these forward-looking statements as a result of various factors, including those set forth in the section titled "Risk Factors" under Part II, Item 1A below. In some cases, you can identify forward-looking statements by terminology such as "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potentially," "predict," "should," "will" or the negative of these terms or other similar expressions.

In addition, statements that "we believe" and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this Quarterly Report on Form 10-Q, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain and investors are cautioned not to unduly rely upon these statements.

Overview

We are a biopharmaceutical company dedicated to transforming the treatment of rare diseases. We have three approved medicines: LIVMARLI® (maralixibat) ("Livmarli"), CHOLBAM® (cholic acid) capsules ("Cholbam"), and CHENODAL® or CTEXLI® (chenodiol) tablets ("Chenodal" or "Ctexli").

Livmarli is a novel, orally administered, minimally-absorbed ileal bile acid transporter ("IBAT") inhibitor ("IBATi") that is approved for the treatment of cholestatic pruritus in patients with Alagille syndrome ("ALGS") in the United States ("U.S."), the European Union ("EU") and various other countries around the world and for cholestatic pruritus in patients with progressive familial intrahepatic cholestasis ("PFIC") in the U.S. and for the treatment of PFIC in the EU. We market and commercialize Livmarli in the U.S., Canada and certain countries in Europe through our specialized and focused commercial team. We have also entered into license and distribution agreements with several rare disease companies for the commercialization of Livmarli in additional countries. In March 2025, our partner Takeda received approval by the Japanese Ministry of Health, Labour, and Welfare for Livmarli for the treatment of cholestatic pruritus in patients with ALGS and PFIC.

On October 22, 2024, we completed a license agreement with Enthorin Therapeutics, LLC and Dart Neuroscience LLC granting us the worldwide right to develop and commercialize MRM-3379, an allosteric inhibitor of Phosphodiesterase 4D ("PDE4D"). We intend to develop MRM-3379 for the treatment of Fragile-X Syndrome ("FXS").

On August 31, 2023, we completed the acquisition of assets of Travere Therapeutics, Inc. ("Travere") that are primarily related to the development, manufacture (including synthesis, formulation, finishing or packaging) and commercialization of chenodiol and Cholbam (also known as Kolbam) (and together with chenodiol, the "Bile Acid Medicines") pursuant to an asset purchase agreement dated July 16, 2023 (such acquisition, the "Bile Acid Portfolio Acquisition").

The FDA approved Cholbam in March 2015, as the first FDA-approved treatment for pediatric and adult patients with bile acid synthesis disorders due to single enzyme defects, and for adjunctive treatment of patients with peroxisomal disorders, including peroxisome biogenesis disorder-Zellweger spectrum disorder ("PBD-ZSD"). Chenodiol is standard of care for the treatment of cerebrotendinous xanthomatosis ("CTX") in the United States with a medical necessity recognition by the FDA and was commercialized under the brand name Chenodal. We submitted a new drug application ("NDA") for chenodiol for the treatment of CTX in 2024 and received FDA approval for the treatment of adults with CTX in February 2025, which is commercialized under the brand name Ctexli. We currently commercialize Cholbam and Ctexli in the U.S. through our specialized and focused commercial team. We have also assumed license and distribution

agreements with several rare disease companies for the commercialization of Cholbam and chenodiol in additional countries.

We are developing Livmarli for certain rare cholestatic conditions through the Phase 3 EXPAND study, which we initiated in the fourth quarter of 2024. We expect to complete enrollment of the EXPAND study in 2026 with topline data expected in the first half of 2027. In addition, we are advancing our product candidate, volixibat, a novel, oral, minimally-absorbed agent designed to inhibit IBAT, for the treatment of adult patients with cholestatic liver diseases. We are developing volixibat in the setting of primary sclerosing cholangitis ("PSC") and primary biliary cholangitis ("PBC"), and in October 2024, we announced that the FDA granted Breakthrough Therapy Designation for volixibat as a potential treatment for cholestatic pruritus in patients with PBC. We conducted an interim analysis of our VISTAS Phase 2b clinical trial in PSC and reported interim data from our VANTAGE Phase 2b clinical trial in PBC in June 2024. The VISTAS Phase 2b clinical trial in PSC completed enrollment in the third quarter of 2025 and topline data is expected in the second quarter of 2026. We expect the VANTAGE Phase 2b clinical trial in PBC to complete enrollment in 2026 with topline data expected in the first half of 2027. We are also developing MRM-3379, a novel PDE4D inhibitor, for the treatment of FXS. A Phase 2 multi-dose safety and efficacy trial has begun enrolling patients.

To date, we have focused primarily on acquiring and in-licensing our product candidates, organizing and staffing our company, business planning, raising capital, advancing our product candidates through clinical development, preparing for commercialization of our product candidates, commercializing our approved medicines, and conducting business development activities relating to, among other things, portfolio expansion through collaborations and acquisitions.

Financial Overview

Our net income was $2.9 million for the three months ended September 30, 2025, our net loss was $14.2 million for the three months ended September 30, 2024, and our net loss was $17.6 million and $64.2 million for the nine months ended September 30, 2025 and 2024, respectively. As of September 30, 2025, we had an accumulated deficit of $661.8 million, compared to $644.2 million as of December 31, 2024. As of September 30, 2025, we had unrestricted cash, cash equivalents and investments of $378.0 million, compared to unrestricted cash, cash equivalents and investments of $292.8 million as of December 31, 2024.

While we generated net income in the third quarter of 2025, we anticipate we will continue to generate net losses for the foreseeable future as we continue commercial activities for our approved medicines, conduct our ongoing and planned clinical trials, seek regulatory approvals for our product candidates and make potential milestone payments to the licensors and other third parties from whom we have in-licensed or acquired our product candidates. We expect that total product sales of our approved medicines will continue to increase on an annual basis; however, due to large periodic orders from Takeda and our distributors, our product revenue may experience fluctuations. Additionally, our product revenues from Takeda are based upon variable consideration estimates. If actual results vary from our estimates, we will make adjustments in the period when such variances become known. As a result, our net losses may fluctuate significantly from quarter-to-quarter and year-to-year.

We expect to satisfy future cash needs through existing capital balances, revenue from our approved medicines and through a combination of equity offerings, debt financings or other capital sources, collaborations, licenses and other similar arrangements. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Our failure to raise capital or enter into such other arrangements when needed could have a negative impact on our financial condition and on our ability to pursue our business plans and strategies. If we are unable to raise additional capital when needed, we could be forced to delay, limit, reduce or terminate the development of one or more of our product candidates or future commercialization efforts or grant rights to develop and market our product candidates even if we would otherwise prefer to develop and market such product candidates ourselves.

Components of Results of Operations

Revenue

Product Sales, Net

We have three approved medicines: Livmarli, Cholbam and Chenodal, or Ctexli. We expect total product sales of our approved medicines will continue to increase on an annual basis.

Our U.S. revenue from product sales, net further depends on our prescription mix of commercial payors, Medicaid and amounts of free medicines provided under our patient assistance program. We expect our prescription mix and resulting gross to net adjustment in the U.S. to remain materially consistent. Our revenue from product sales is recognized when the control of the product is transferred. Under our license agreement with Takeda as well as agreements with distributors, we may receive large periodic orders for our products. The timing of these orders can be inconsistent and can create significant quarter-to-quarter variation in product sales. In addition, we recognize our best estimate of the

consideration that we expect to receive when control of the inventory is transferred to our licensed partners and distributors. Such estimates may be complex and include estimates as to if and when our distributors and licensed partner's sales in the market will occur. Estimates are reviewed and updated quarterly as additional information, including in-market pricing and sales information of our authorized distributors and licensed partners, becomes known which may cause variability of quarterly revenue particularly during periods of product launch.

Although we expect product revenues to increase as we continue commercial activities for our approved medicines, we may not achieve commercial success. Certain of our approved medicines, including the Bile Acid Medicines, are subject to immediate competition from compounded and generic entrants, as the abbreviated new drug application ("ANDA") and NDA for these drug products have no remaining or current patent exclusivity. Chenodiol is standard of care for the treatment of CTX in the U.S. and was commercialized with a medical necessity recognition by the FDA until February 2025. We submitted an NDA for chenodiol for the treatment of CTX in 2024 and received FDA approval for the treatment of adults with CTX in February 2025, which is now commercialized under the brand name Ctexli. The FDA has granted orphan exclusivity for chenodiol for the treatment of CTX.

Operating Expenses

Cost of Sales

Cost of sales consist of raw materials, third-party manufacturing costs, personnel, facility and other costs of manufacturing commercial products, transportation and freight, amortization of finite-lived intangible assets and royalty payments payable on net sales of our approved medicines under licensing agreements. Cost of sales may also include period costs related to certain manufacturing services and charges for inventory valuation reserves. In addition, we have firm commitments for the purchase of minimum order quantities for active pharmaceutical ingredients ("APIs"). We periodically evaluate these firm commitments to determine if these commitments are in excess of our needs. If any net loss is determined, we record a charge to cost of sales in the period identified. As of the date of our acquisition of the Bile Acid Medicines from Travere, inventory acquired was valued at its fair value. As a result, our cost of sales exceeded cost to manufacture the inventory and had a negative impact on our gross margin.

We expect cost of sales to increase in the future mainly due to variable costs associated with increased product sales such as royalties payable and inventory costs, partially offset by lower unit cost of sales for the Bile Acid Medicines, as we sold the acquired inventory valued at fair value in prior periods. We expect cost of sales to remain approximately unchanged as a percent of product sales in the future.

Research and Development Expenses

Research and development expenses primarily relate to clinical development and manufacturing activities of our product candidates. Our research and development expenses include, among other things:

- salaries and related expenses for employee personnel, including benefits, travel and expenses related to stock-based compensation granted to personnel in development functions;
- external expenses paid to clinical trial sites, contract research organizations ("CROs") and consultants that conduct our clinical trials;
- expenses related to drug formulation development and the production of clinical trial supplies, including fees paid to contract manufacturers;
- licensing milestone payments related to development or regulatory events;
- payments made for the acquisition or licensing of in-process research and development assets with no alternative future use;
- expenses related to non-clinical studies;
- expenses related to compliance with drug development regulatory requirements; and
- other allocated expenses, which include direct and allocated expenses for rent and maintenance of facilities, depreciation of equipment, and other supplies.

We expense research and development costs as incurred. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the related goods are delivered or the services are performed. Upfront payments, research and development funding and milestone payments made to third parties in connection with licenses and research and development collaborations are expensed as incurred.

We expect our research and development expense may increase in the future as we continue to develop our volixibat product candidates, execute the EXPAND label expansion study for Livmarli and initiate development of MRM-3379.

Selling, General and Administrative Expense

Sales and marketing expense, which is a component of selling, general and administrative expense, primarily consisted of employee-related expenses for our sales group, brand marketing, patient support groups and pre-commercialization expenses related to our product candidates. General and administrative expense, which is a component of selling, general and administrative expense, primarily consists of corporate support and other administrative expenses, including employee-related expenses.

We anticipate that our selling, general and administrative expenses will increase in the future to support our continued commercialization efforts of our approved medicines in the U.S. and internationally as well as increased costs of operating as a global commercial stage biopharmaceutical public company. These increases will likely include increased costs related to hiring of additional personnel and fees to outside consultants to support further marketing, legal, tax, planning and accounting activities.

Interest Income

Interest income consists of interest earned on our cash equivalents and investments.

Interest Expense

We incur interest expense on our convertible notes. Interest on our convertible notes consists of a 4.00% per annum fixed rate of interest and amortization of debt discount and amortization costs.

Other Income, Net

Other income, net consists of miscellaneous other income and expense and unrealized and realized currency gains and losses on net assets and liabilities denominated in foreign currency.

Critical Accounting Estimates

The preparation of financial statements and related disclosures in conformity with U.S. generally accepted accounting principles ("GAAP") and our discussion and analysis of our financial condition and operating results require our management to make judgments, assumptions and estimates that affect the amounts reported. Management bases its estimates on historical experience, known trends and events, and on various other assumptions it believes to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities. Actual results may differ materially from these estimates under different assumptions or conditions.

There have been no significant changes during the three and nine months ended September 30, 2025, except as described below, in our critical accounting policies and estimates as compared to the critical accounting policies and estimates disclosed in the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations" included in our Annual Report.

Product Sales, Net

Revenues from direct product sales are recorded net of reserves established for applicable discounts and allowances that are offered within contracts with our customers, including amounts from payors and other third parties on behalf of our customers. For revenues from distributors and our licensed partner, Takeda, we record net product sales at the time control of the product is transferred, based on the estimated variable consideration to be received. The transaction price, which may include fixed or variable consideration may be subject to constraint and is included in the product sales price only to the extent that it is probable that a significant reversal of the amount of the cumulative revenue recognized will not occur in a future period. We recognize our best estimate of the consideration that we expect to receive when control of the inventory is transferred to our customer and revenue is recognized. For our distributor and license partner sales, such estimates may be more complex and include estimates as to if and when our distributors and licensed partner's sales in the market will occur. These estimates are reviewed and updated quarterly as additional information, including in-market sales information of our authorized distributors and licensed partners, becomes known. Actual amounts may ultimately differ from our estimates. If actual results vary, we adjust these estimates, which could have an effect on earnings in the period of adjustment.

We are obligated to pay rebates for mandated discounts under the Medicaid Drug Rebate Program and other foreign government programs. Our rebate calculations may require estimates based upon our actual historical experience, customer and payor mix and revenue projections. We update estimates and assumptions on a quarterly basis and record any

necessary adjustments to revenue in the period identified. Estimated rebates are recorded as a reduction of revenue in the period the related sale is recognized. To date, actual government rebates have not differed materially from our estimates.

Recent Accounting Pronouncements

A description of recent accounting pronouncements that may potentially impact our financial position, results of operations or cash flows is disclosed in Note 2 to our unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q.

Results of Operations for the Three Months Ended September 30, 2025 and 2024

The following table summarizes our results of operations for the three months ended September 30, 2025 and 2024 (in thousands):

	Three Months Ended September 30,		
	2025	2024	Change
Revenue:			
Product sales, net	$ 133,010	$ 90,302	$ 42,708
License and other revenue	—	75	(75)
Total revenue	133,010	90,377	42,633
Operating expenses:			
Cost of sales	25,537	20,806	4,731
Research and development	42,960	31,710	11,250
Selling, general and administrative	61,910	50,545	11,365
Total operating expenses	130,407	103,061	27,346
Income (loss) from operations	2,603	(12,684)	15,287
Other income (expense):			
Interest income	3,251	3,469	(218)
Interest expense	(3,606)	(3,586)	(20)
Other income (expense), net	395	(1,087)	1,482
Net income (loss) before provision for income taxes	2,643	(13,888)	16,531
(Benefit from) provision for income taxes	(262)	347	(609)
Net income (loss)	$ 2,905	$ (14,235)	$ 17,140

Product Sales, Net

Product sales, net was $133.0 million for the three months ended September 30, 2025, compared to $90.3 million for the three months ended September 30, 2024. The increase in product sales, net was a result of our continued commercialization of Livmarli in the U.S. for the treatment of ALGS and PFIC, and in certain international markets directly or through distributor and partner orders and from our sales of the Bile Acid Medicines.

The following table disaggregates total Product sales, net (in thousands):

	Three Months Ended September 30,		
	2025	2024	Change
Product sales, net:			
Livmarli	$ 92,235	$ 59,126	$ 33,109
Bile Acid Medicines	40,775	31,176	9,599
Total product sales, net	$ 133,010	$ 90,302	$ 42,708

Cost of Sales

For the three months ended September 30, 2025, cost of sales was $25.5 million, compared to $20.8 million for the three months ended September 30, 2024. The increase in cost of sales was primarily a result of increases in royalty expense of $3.9 million on net sales of Livmarli and the Bile Acid Medicines under licensing agreements, $1.0 million PDUFA fees primarily associated with the approval of our solid dose formulation in Livmarli and $0.7 million in general supply chain support costs. These increases were partially offset by lower product cost of sales of $1.6 million primarily

related to the Bile Acid Medicines as we substantially completed the sale of acquired inventory in prior periods which had been recorded at fair value.

Research and Development Expenses

The following table summarizes the period-over-period changes in research and development expenses relating to our product candidates in development for the periods indicated (in thousands):

	Three Months Ended September 30,		
	2025	2024	Change
Product-specific costs:			
Livmarli	$ 3,033	$ 6,060	$ (3,027)
Volixibat	12,725	8,270	4,455
MRM-3379	3,510	—	3,510
Non product-specific costs:			
Stock-based compensation	5,676	3,571	2,105
Personnel	13,022	8,760	4,262
Other	4,994	5,049	(55)
Total research and development expenses	$ 42,960	$ 31,710	$ 11,250

Research and development expenses were $43.0 million for the three months ended September 30, 2025, an increase of $11.3 million compared to the three months ended September 30, 2024. The increase was primarily due to:

- for volixibat programs, an increase of $4.5 million, primarily due to increased expenses associated with conduct of the PSC and PBC trials as well as manufacturing development expenses;
- for MRM-3379, an increase of $3.5 million, primarily due to planning for our Phase 2 study in FXS and clinical manufacturing expenses; and
- for personnel related and stock-based compensation expenses, an increase of $6.4 million related primarily to increased employee headcount and related equity award grants to support our development pipeline, partially offset by
- for Livmarli, a decrease of $3.0 million, primarily due to lower costs from the PFIC rollover study and other clinical costs partially offset by increased expenses associated with the Livmarli Phase 3 EXPAND label expansion study.

Selling, General and Administrative Expenses

Selling, general and administrative expenses were $61.9 million for the three months ended September 30, 2025, an increase of $11.4 million compared to the three months ended September 30, 2024. The increase was primarily due to increases of $7.7 million in personnel and other compensation-related expenses, including an increase of $4.1 million in stock-based compensation, reflecting an increase in the number of our selling, marketing and administrative employees to support commercial activities for our approved medicines, $1.7 million of legal, accounting and other outside services, $1.0 million in general administrative expenses and $1.0 million of expenses associated with post marketing approval studies.

Interest Income

Interest income was $3.3 million for the three months ended September 30, 2025, a decrease of $0.2 million compared to the three months ended September 30, 2024 largely due to lower yields on investments.

Interest Expense

Interest expense was $3.6 million for the three months ended September 30, 2025 and September 30, 2024, and related to interest expense incurred on our convertible notes.

(Benefit from) provision for income taxes

Benefit from income taxes was $0.3 million benefit for the three months ended September 30, 2025 compared to $0.3 million provision for the three months ended September 30, 2024. The $0.3 million benefit for the three months ended September 30, 2025 was a result of the impact of the One Big Beautiful Bill Act (the “OBBBA”) as the Company

will no longer be required to capitalize its domestic research and experiment costs under Section 174 of the Internal Revenue Code.

Results of Operations for the Nine Months Ended September 30, 2025 and 2024

The following table summarizes our results of operations for the nine months ended September 30, 2025 and 2024 (in thousands):

	Nine Months Ended September 30,		
	2025	2024	Change
Revenue:			
Product sales, net	$ 372,380	$ 236,979	$ 135,401
License and other revenue	—	495	(495)
Total revenue	372,380	237,474	134,906
Operating expenses:			
Cost of sales	71,976	58,863	13,113
Research and development	135,071	96,604	38,467
Selling, general and administrative	182,902	145,391	37,511
Total operating expenses	389,949	300,858	89,091
Loss from operations	(17,569)	(63,384)	45,815
Other income (expense):			
Interest income	9,307	10,588	(1,281)
Interest expense	(10,791)	(10,732)	(59)
Other income, net	2,589	982	1,607
Net loss before provision for income taxes	(16,464)	(62,546)	46,082
Provision for income taxes	1,169	1,606	(437)
Net loss	$ (17,633)	$ (64,152)	$ 46,519

Product Sales, Net

Product sales, net was $372.4 million for the nine months ended September 30, 2025, compared to $237.0 million for the nine months ended September 30, 2024. The increase in product sales, net was a result of our continued commercialization of Livmarli in the U.S. for the treatment of ALGS and PFIC, and in certain international markets directly or through distributor and partner orders and from our sales of the Bile Acid Medicines.

The following table disaggregates total Product sales, net (in thousands):

	Nine Months Ended September 30,		
	2025	2024	Change
Product sales, net:			
Livmarli	$ 253,619	$ 149,202	$ 104,417
Bile Acid Medicines	118,761	87,777	30,984
Total product sales, net	$ 372,380	$ 236,979	$ 135,401

Cost of Sales

For the nine months ended September 30, 2025, cost of sales was $72.0 million, compared to $58.9 million for the nine months ended September 30, 2024. The increase in cost of sales was primarily a result of increases in royalty expense of $11.2 million on net sales of Livmarli and the Bile Acid Medicines under licensing agreements, a $2.3 million increase primarily associated with increased PDUFA fees associated with the approval of our solid dose formulation in Livmarli and higher commercial supply chain costs of $2.8 million. These increases were partially offset by lower product cost of sales of $5.5 million primarily related to the Bile Acid Medicines, as we substantially completed the sale of acquired inventory in prior periods which had been recorded at fair value.

Research and Development Expenses

The following table summarizes the period-over-period changes in research and development expenses relating to our product candidates in development for the periods indicated (in thousands):

	Nine Months Ended September 30,		
	2025	2024	Change
Product-specific costs:			
Livmarli	$ 11,596	$ 20,187	$ (8,591)
Volixibat	37,382	23,140	14,242
MRM-3379	6,957	—	6,957
Non product-specific costs:			
Stock-based compensation	18,529	10,978	7,551
Personnel	39,015	26,374	12,641
License fees (milestone payments)	5,000	—	5,000
Other	16,592	15,925	667
Total research and development expenses	$ 135,071	$ 96,604	$ 38,467

Research and development expenses were $135.1 million for the nine months ended September 30, 2025, an increase of $38.5 million compared to the nine months ended September 30, 2024. The increase was primarily due to:

- for volixibat programs, an increase of $14.2 million, primarily due to increased expenses associated with conduct of the PSC and PBC trials as well as manufacturing development expenses;
- for MRM-3379, an increase of $7.0 million, primarily due to planning for our Phase 2 study in FXS and clinical manufacturing expenses;
- for personnel related and stock-based compensation expenses, an increase of $20.2 million related primarily to increased employee headcount and related equity award grants to support our development pipeline; and
- for license fees, an increase of $5.0 million due to a development milestone payment associated with our Livmarli Phase 3 EXPAND label expansion study, partially offset by
- for Livmarli, a decrease of $8.6 million primarily due to completion of clinical trials including the biliary atresia, PFIC rollover study and a safety study, lower general clinical support costs partially offset by increased expenses associated with the Livmarli Phase 3 EXPAND label expansion study.

Selling, General and Administrative Expenses

Selling, general and administrative expenses were $182.9 million for the nine months ended September 30, 2025, an increase of $37.5 million compared to the nine months ended September 30, 2024. The increase was primarily due to increases of $24.5 million in personnel and other compensation-related expenses, including an increase of $9.4 million in stock-based compensation, reflecting an increase in the number of our selling, marketing and administrative employees to support commercial activities for our approved medicines, $4.0 million in advertising, promotion and medical affairs expenses associated with commercial activities, $4.0 million in other general administrative expenses, $2.6 million of expenses associated with post marketing studies and $2.4 million associated with legal, accounting and other outside services.

Interest Income

Interest income was $9.3 million for the nine months ended September 30, 2025, a decrease of $1.3 million compared to the nine months ended September 30, 2024 largely due to lower yields on investments.

Interest Expense

Interest expense for the nine months ended September 30, 2025 was unchanged in comparison to the nine months ended September 30, 2024, and related to interest expense incurred on our convertible notes.

Liquidity and Capital Resources

Overview

Since inception, we have funded our operations primarily through debt, equity, revenue interest financings and, to a lesser extent, cash from our product sales and license and collaboration revenue. We had $378.0 million of unrestricted cash, cash equivalents and investments as of September 30, 2025, compared to unrestricted cash, cash equivalents and investments of $292.8 million as of December 31, 2024. We have incurred significant operating losses since our inception. As of September 30, 2025, we had an accumulated deficit of $661.8 million, compared to $644.2 million as of December 31, 2024.

In August 2025, we filed an automatic shelf registration statement on Form S-3 with the SEC (the "2025 Shelf Registration"), which became effective upon filing, pursuant to which we may register for sale from time to time in one or more offerings an unlimited amount of any combination of our common stock, preferred stock, debt securities and warrants, so long as we continue to satisfy the requirements of a "well-known seasoned issuer" under SEC rules. This automatic shelf registration statement will remain in effect for up to three years from the date it became effective. As of September 30, 2025, we have not issued any securities pursuant to the 2025 Shelf Registration.

In November 2023, we entered into a Sales Agreement (the "2023 Sales Agreement") with Leerink and Cantor Fitzgerald & Co. (the "Sales Agents"), pursuant to which we may, from time to time, sell up to an aggregate amount of $200.0 million of our common stock through the Sales Agents in an "at-the-market" offering (the "ATM Offering") pursuant to the 2025 Shelf Registration. We are not required to sell shares under the 2023 Sales Agreement. Sales of our common stock, if any, under the 2023 Sales Agreement may be made in any transactions that are deemed to be "at the market offerings" as defined in Rule 415 under the Securities Act. We will pay a given designated Sales Agent a commission of up to 3.0% of the aggregate gross proceeds of any shares of common stock sold through it pursuant to the 2023 Sales Agreement. As of September 30, 2025, we have not issued any securities pursuant to the 2023 Sales Agreement.

Based on our current and anticipated level of operations and cash generated from sales of our approved medicines, we believe our existing unrestricted cash, cash equivalents and investments will be sufficient to fund current operations through at least the next 12 months from the filing of this Quarterly Report on Form 10-Q and beyond.

While we generated net income in the third quarter of 2025, we anticipate that we will continue to incur net losses for the foreseeable future as we continue research efforts and the development of our product candidates, continue commercialization activities for our approved medicines and potentially expand into additional markets, hire additional staff, including clinical, scientific, operational, financial and management personnel and pay potential development milestones. Net loss is also impacted by significant non-cash charges related to stock-based compensation and amortization of intangible assets.

Our primary use of cash is to fund operating expenses. Our cash flow from operating activities may experience material quarter to quarter fluctuations due to a number of factors, including the timing of inventory builds, accounts receivable collections, receipt and payment of invoices, development milestone payments to our license partners as well as the magnitude and timing of cash receipts from our product revenues associated with periodic orders from Takeda and our distributors.

Our principal source of liquidity is product revenue from sales of our approved medicines. In recent quarters, liquidity from product revenues has been sufficient to fund current operations. There can be no assurances that future revenues will continue to be sufficient to fund operations. Should product revenues from our currently approved medicines, our current product candidates or any future product candidates, if approved, be insufficient to fund operations, we would expect to finance our cash needs through a combination of equity offerings, debt financings and potential collaboration, license or development agreements. Our primary cash needs are for day-to-day operations and to fund our working capital requirements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect rights as a stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. Additionally, if the equity and credit markets deteriorate from adverse geopolitical and macroeconomic developments or otherwise, it may make any necessary debt or equity financing more difficult, more costly and more dilutive. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce

or terminate our drug development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Material Cash Requirements

In addition to ongoing capital needs to fund our ongoing operations, our material cash requirements include the following contractual and other obligations.

In April 2023, we completed an offering of $316.3 million aggregate principal of the Notes, which includes the exercise of the initial purchasers' option in full. The offering resulted in net proceeds of $305.3 million after deducting the initial purchasers' discounts and commissions and offering expenses. The Notes are our senior, unsecured obligations and accrue interest at a rate of 4.00% per annum, payable semi-annually in arrears on May 1 and November 1 of each year. The Notes will mature on May 1, 2029, unless earlier converted, redeemed or repurchased by us. The terms of these Notes are further described in Note 9 to our unaudited condensed consolidated financial statements.

During the third quarter of 2025, the last reported sale price of our common stock exceeded 130% of the conversion price of the Notes for more than 20 trading days during the 30 consecutive trading days ended September 30, 2025. As a result, the Notes are convertible at the option of the holders of the Notes during the fourth quarter of 2025, the quarter immediately following the quarter when the conditions were met, as stated in the terms of the Notes. If holders of the Notes elect to convert their Notes, we may elect to settle such conversions by paying or delivering, as applicable, cash, shares of our common stock or a combination of cash and shares of our common stock.

Under the Shire License Agreement, the Asset Purchase Agreement with Travere and license agreement with Enthorin as well as our other license and acquisition agreements, we have payment obligations that are contingent upon future events such as our achievement of specified development, regulatory and commercial milestones and are required to make royalty payments in connection with the sale of products developed under those agreements. The amount and timing of milestone obligations are unknown or uncertain as we are unable to estimate the timing or likelihood of achieving the milestone events. Additionally, the amount of royalty payments are based upon future product sales, which we are unable to predict with certainty. These potential obligations are further described in Note 6 to our unaudited condensed consolidated financial statements.

We additionally have contractual obligations for our operating leases for our corporate headquarters. These obligations are further described in Note 8 to our unaudited condensed consolidated financial statements.

We enter into contracts in the normal course of business with clinical research organizations and clinical sites for the conduct of clinical trials, non-clinical research studies, professional consultants for expert advice and other vendors for clinical supply manufacturing or other services. These contracts generally provide for termination on notice, and therefore are cancellable contracts.

We enter into commercial inventory supply agreements that obligate us to firm commitments for the purchase of minimum order quantities, which may be material to our financial statements.

Cash Flows

The following table provides a summary of the net cash flow activity for the periods indicated (in thousands):

	Nine Months Ended September 30,	
	2025	2024
Net cash provided by operating activities	$ 49,760	$ 15,393
Net cash used in investing activities	(24,960)	(90,766)
Net cash provided by financing activities	31,143	12,359
Effect of exchange rate on cash, cash equivalents and restricted cash	3,630	82
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 59,573	$ (62,932)

Net Cash Provided by Operating Activities

Net cash provided by operating activities was $49.8 million for the nine months ended September 30, 2025, reflecting our net loss of $17.6 million offset by adjustments of $73.1 million. Adjustments consisted primarily of stock-based compensation, depreciation and amortization of our intangible assets and fixed assets, and charges associated with excess and obsolete inventory and firm commitment losses. Additionally, cash provided by operating activities reflected a cash outflow for changes in net operating assets of $5.7 million, primarily related to an increase in accounts receivable and payments made for inventory and prepaid assets. The net use of cash was partially offset by an increase in accrued sales

deductions and royalties due to the growth from our product sales in the nine months ended September 30, 2025, an increase in accrued clinical trial expenses during the period and an increase in accrued interest in relation to the Notes.

Net cash provided by operating activities was $15.4 million for the nine months ended September 30, 2024, reflecting our net loss of $64.2 million partially offset by adjustments of $55.3 million. Adjustments consisted primarily of stock-based compensation, depreciation and amortization of our intangible assets and fixed assets, and charges associated with excess and obsolete inventory and firm commitment losses. Additionally, cash provided by operating activities reflected changes in net operating assets of $24.3 million, primarily related to the increase in accounts payable, accrued expenses and other liabilities resulting primarily from an increase in accrued sales deductions and royalties due to the growth from our product sales in the nine months ended September 30, 2024, and an increase in accrued expenses driven by our growth, including accrued expenses related to clinical studies and contract manufacturing activities, offset by payments made for the purchase of inventory.

Net Cash Used in Investing Activities

Net cash used in investing activities was $25.0 million for the nine months ended September 30, 2025, primarily due to purchases of investments offset by proceeds from maturities of investments.

Net cash used in investing activities for the nine months ended September 30, 2024 was $90.8 million primarily due to purchases of investments resulting from the changing interest rate environment and milestone payments associated with the approval of Livmarli for cholestatic pruritus in patients with PFIC in the U.S. and for the treatment of PFIC in the EU, offset by proceeds from maturities of investments.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $31.1 million for the nine months ended September 30, 2025, due to proceeds from employee equity award exercises.

Net cash provided by financing activities was $12.4 million for the nine months ended September 30, 2024, due to proceeds from employee equity award exercises.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

Interest Rate Risk

Our cash, cash equivalents and investments as of September 30, 2025 consist of readily available checking and money market funds and investments. The primary objective of our investment activities is to preserve our capital to fund operations. We may invest in highly liquid and high-quality government and debt securities. As a result, our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the strategies we employ (including the short-term nature of the instruments in our portfolio and the low risk profile of our investments), as of the date of this Quarterly Report on Form 10-Q, we do not expect anticipated changes in interest rates to have a material effect on our interest rate risk in future reporting periods. For example, a hypothetical change in interest rates of 10% would not have a material impact on the fair market value of our cash equivalents and investments as of September 30, 2025. In addition, we maintain significant amounts of cash and cash equivalents at one financial institution that is in excess of federally insured limits.

We have outstanding $316.2 million aggregate principal of the Notes as of September 30, 2025. The interest rates on these Notes are fixed and therefore they do not expose us to risk related to rising interest rates. As of September 30, 2025, the approximate fair value of our Notes was $760.9 million.

Foreign Currency Rate Risk

Our operations include activities in the U.S., the Netherlands, Switzerland and certain other countries in Europe. As a result, our financial results may be affected by factors such as changes in foreign currency exchange rates or weak economic conditions in the foreign markets in which we sell our products. Our operating results are exposed to changes in foreign currency exchange rates between the U.S. Dollar (“USD”) and various foreign currencies, primarily the Euro and Swiss Franc. When the USD strengthens against these currencies, the relative value of the sales and operating expenses made in the respective foreign currency decreases. Conversely, when the USD weakens against these currencies, the relative value of such sales and operating expenses increases.

Based on our overall foreign currency denominated exposures as of September 30, 2025, we believe that a near-term 10% fluctuation of the USD exchange rate could result in a potential change in the fair value of our net assets and liabilities denominated in foreign currency by approximately $2.6 million. We expect to continue to enter into transactions based in foreign currencies that could be impacted by changes in the USD exchange rate.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation and supervision of our principal executive officer and our principal financial officer, have evaluated our disclosure controls and procedures (as defined in Rules 13a-15(e) or 15d-15(e) under the Exchange Act) as of the end of the period covered by this Quarterly Report on Form 10-Q. Based on that evaluation, our principal executive officer and our principal financial officer have concluded that as of the end of the period covered by this Quarterly Report on Form 10-Q, our disclosure controls and procedures were effective to provide reasonable assurance that information we are required to disclose in reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in SEC rules and forms, and that such information is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

Changes in Internal Control over Financial Reporting

Under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, we carried out an evaluation of any potential changes in our internal control over financial reporting during the fiscal quarter covered by this Quarterly Report on Form 10-Q.

There were no changes in our internal control over financial reporting during the three months ended September 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.

From time to time, we may become involved in legal proceedings relating to claims arising from the ordinary course of business. Our management believes that there are currently no claims or actions pending against us, the ultimate disposition of which could have a material adverse effect on our results of operations, financial condition or cash flows.

Item 1A. Risk Factors.

An investment in shares of our common stock involves a high degree of risk. You should carefully consider the following risk factors, as well as the other information in this Quarterly Report on Form 10-Q, before deciding whether to purchase, hold or sell shares of our common stock. The occurrence of any of the following risks could harm our business, financial condition, results of operations and/or growth prospects or cause our actual results to differ materially from those contained in forward-looking statements we have made in this Quarterly Report on Form 10-Q and those we may make from time to time. You should consider all of the risk factors described when evaluating our business.

Risks Related to Commercialization of our Approved Medicines and Development of our Product Candidates

The success of our business depends, in part, on our ability to market and sell our approved medicines profitably.

The success of our business depends, in part, on our ability to commercialize our approved medicines profitably. Our successful commercialization of our approved medicines depends on a number of factors, including, among others, the following:

- our ability to grow and maintain our sales team in the U.S., Canada, and certain countries in Europe, as well as scale our distribution capabilities in these locations and others where our products are available;
- the availability of adequate reimbursement and a commercially viable sales price of our approved medicines;
- acceptance by physicians, payors and patients of the benefits, safety and efficacy of our approved medicines, including relative to alternative and competing treatments;
- a continued acceptable safety profile of our approved medicines;
- the effect of health care legislation and regulatory changes in the locations where our approved medicines are authorized;
- our ability to successfully obtain the substances and materials used in manufacturing our medicines from third parties and to have finished product manufactured by third parties in accordance with regulatory requirements and in sufficient quantities for our commercial needs;
- our ability to establish and enforce intellectual property rights in and to our approved medicines and avoid or successfully defend third-party patent interference or intellectual property infringement claims;
- our ability to compete successfully with the marketing and sale of compounded and generic versions of our medicines; and
- sufficient patient population that would benefit from our approved medicines as they are intended for use in rare diseases for which the patient population is small.

If one or more of the above factors is not present, many of which are beyond our control, in a timely manner or at all, we could experience significant delays or an inability to market and sell our approved medicines profitably, which would harm our business, financial condition, operating results and prospects.

If we are unable to adequately grow, maintain and scale our marketing and sales capabilities or enter into or maintain rights pursuant to agreements with third parties to market and sell our approved medicines, we may not be able to generate viable revenues.

To successfully commercialize our approved medicines, we must grow, maintain and appropriately scale our marketing, sales, distribution, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. We have established our own commercial capabilities in the U.S. to commercialize our approved medicines. We are also in the process of further establishing our capabilities related to Livmarli in certain major European

markets and Canada and have entered into a limited number of partner and distributor agreements in other select geographies. We plan to continue to evaluate opportunities to partner with pharmaceutical companies that have established sales and marketing capabilities to commercialize our approved medicines and our product candidates, if approved, outside of these geographies. Our projections of the commercial and sales needs to target these markets may not be accurate. If we are materially off from our projections, our business and operating results would be harmed.

Growing and maintaining our own sales force to market Livmarli, Cholbam and Ctexli is expensive and time-consuming. Moreover, we may not be able to successfully or adequately develop this capability for our product candidates in development. We compete with other pharmaceutical and biotechnology companies to recruit, hire, train and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our approved medicines, and any agreements with such third parties may not be on terms that are favorable to us. To the extent we do rely on third parties to commercialize our approved medicines and our other product candidates, if approved, we may have little or no control over the marketing and sales efforts of such third parties and our revenues from product sales may be lower than if we had commercialized our product candidates ourselves. In addition, we have entered into a limited number of partner and distributor agreements. Any loss, commercial failure, or termination of rights pursuant to these agreements could delay or hinder our commercialization efforts.

In the event we are unable to successfully grow and maintain our marketing and sales force or collaborate with necessary third-party marketing and sales organizations, we would not be able to commercialize our approved medicines and our business, results of operations, financial condition, and prospects would be materially adversely affected.

Our commercial success may be severely hindered if we are unable to obtain and/or maintain adequate coverage and reimbursement for our approved medicines and any future product candidates, if approved.

The availability of coverage and adequate reimbursement from private third-party payors such as pharmacy benefit managers and commercial insurers, and governmental healthcare programs, such as Medicaid in the U.S. and equivalent programs in foreign countries, is critical to the commercial success of our approved medicines in the U.S. and in international markets. Coverage may be adversely affected by a number of factors, including, but not limited to:

- increasing and intense pressure from political, social, competitive and other sources to reduce drug unit costs, access drugs from other countries to achieve better pricing or limit changes in list price;
- changes in federal, state or foreign government regulations or private third-party payors' reimbursement policies, including changes that may result from government administration changes;
- implementation of federal or state regulations;
- reimbursement decisions and price negotiations with foreign government payors;
- consolidation and increasing assertiveness of commercial payors seeking net price reduction via drug rebates and other forms of discounts linked to the placement of our approved medicines on their formularies; and
- the imposition of restrictions on access or coverage of particular drugs or pricing.

A trend in the healthcare industry is cost containment. Third-party payors are developing increasingly sophisticated methods of controlling healthcare costs by, among other methods, limiting or preventing (for example via prior authorization, prior therapy or step edit requirements) coverage for particular medications, requiring drug companies to provide them with varying levels of discounts from list prices and/or challenging the value of list prices charged for medical products. Similarly, the containment of healthcare costs has become a priority for federal, national, and state governments around the world. Coverage decisions may depend upon the size of a patient population, perceptions of clinical efficacy and economic standards that may disfavor new drug products when more established or lower cost therapeutic alternatives are already available or subsequently become available.

Coverage and reimbursement for drug products can differ significantly across payors. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our approved medicines to each third-party payor separately, with no assurance that coverage will be obtained or maintained. Additionally, coverage policies and third-party reimbursement rates may change at any time. For example, rebate payments may increase, or prices be adjusted, under value-based purchasing arrangements based on evidence-based measures or outcomes-based measures for a patient or beneficiary based on use of our drug. Thus, even if favorable coverage and reimbursement status is attained for one or more drug products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

In many foreign countries, including EU Member States, the pricing of prescription drugs is subject to governmental control and the proposed pricing for a drug must be approved before it may be lawfully marketed. In such countries, pricing negotiations with governmental authorities can take considerable time after receipt of regulatory approval for a product and varies between countries. In addition, there can be considerable pressure from governments and other stakeholders on prices and reimbursement levels. For instance, governmental authorities in the EU Member States and third-party payors could base pricing and reimbursement terms on what they perceive to be comparable products, even if approved for different indications. In addition, EU Member States may restrict the range of medicines for which their national health insurance systems provide reimbursement and to control the prices of medicines for human use. An EU Member State, such as France and Germany, may approve a specific price for the medicine, it may refuse to reimburse a product at the price set by the manufacturer or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicine on the market. For example, some countries, like France, may impose a total revenue cap on a product, limiting maximum sales potential at a certain threshold regardless of unit price. These pricing and reimbursement decisions may impact the pricing and reimbursement of our approved medicines in such jurisdictions. Many EU Member States also periodically review their reimbursement procedures for medicines, which could have an adverse impact on the reimbursement status of our approved medicines in the future. Moreover, political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations often continue after coverage and reimbursement have been obtained. Reference pricing or pricing comparisons to our competitors used by various countries and parallel distribution, or arbitrage between low-priced and high-priced countries, can further reduce prices. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries.

We expect that legislators, policymakers and healthcare insurance funds in the EU Member States will continue to propose and implement cost-containing measures, such as lower maximum prices, lower or lack of reimbursement coverage and incentives to use cheaper, usually generic, products as an alternative to branded products, and/or branded products available through parallel import to keep healthcare costs down. Moreover, in December 2021, Regulation No 2021/2282 on Health Technology Assessment, ("HTA"), amending Directive 2011/24/EU ("HTA Regulation"), was adopted in the EU. The Regulation entered into application on January 12, 2025 through a phased implementation. It is intended to boost cooperation among EU Member States in assessing health technologies, including new medicines, and providing the basis for cooperation at the EU level for joint clinical assessments in these areas. The Regulation permits EU Member States to use common HTA tools, methodologies, and procedures across the EU, working together in four main areas, including joint clinical assessment of the innovative health technologies with the most potential impact for patients, joint scientific consultations whereby developers can seek advice from HTA authorities, identification of emerging health technologies to identify promising technologies early, and continuing voluntary cooperation in other areas. Individual EU Member States will continue to be responsible for assessing non-clinical (e.g., economic, social, ethical) aspects of health technologies, and making decisions on pricing and reimbursement. If we are unable to maintain favorable pricing and reimbursement status in EU Member States for product candidates that we may successfully develop and for which we may obtain regulatory approval, any anticipated revenue from and growth prospects for those product candidates in the EU could be negatively affected.

Historically, products launched in the EU and other foreign countries do not follow the price structures of the U.S. and prices can be significantly lower and the time to obtain pricing and reimbursement approvals is significantly longer. If pricing is set at unsatisfactory levels or if reimbursement of our approved medicines and any future product candidates, if approved, is unavailable or limited in scope or amount, our revenues from sales by us or our partners and the potential profitability of our approved medicines or any future product candidates, if approved, in those countries would be negatively affected.

Our approved medicines or any one of our product candidates, if approved, may fail to achieve the market acceptance among physicians, patients and others in the medical community necessary for commercial success.

The commercial success of our approved medicines or any one of our product candidates, if approved, depends significantly on the market acceptance among physicians, patients, tertiary care centers, transplant centers and others in the medical community. The degree and rate of market acceptance depends on a number of factors, including, among other things:

- patient demand;
- the availability of adequate reimbursement from private third-party payors and government authorities;
- the willingness of patients to pay out-of-pocket in the absence of coverage and adequate reimbursement by third-party payors and government authorities;

- the cost of treatment in relation to alternative treatments and patients willingness to pay for our approved medicines, including relative to discretionary items;
- our ability to successfully compete with available off-label therapies, future approved therapies, and therapies in development and available for use through expanded access programs;
- acceptance by physicians, patients, tertiary care centers, transplant centers and others in the medical community that our approved medicines are safe and effective treatments;
- physician and patient willingness to adopt a new therapy over other available therapies;
- limitations, warnings or adverse drug reactions contained in the labeling or product inserts approved by the FDA, European Commission or comparable foreign regulatory authorities, and patients' and physicians' assessment of these limitations and warnings;
- overcoming any biases physicians or patients may have toward particular therapies for the treatment of the indications our approved medicines are approved for (or, if applicable, deemed medically necessary for);
- patients and caregivers properly using our approved medicines as instructed;
- the prevalence and severity of side effects from the use or potential misuse of our approved medicines;
- relative convenience and ease of administration, including as compared to alternative treatments and competitive therapies, and patient satisfaction with the overall treatment experience;
- the ability of specialty pharmacies we contract with to process prescriptions and dispense our approved medicines and the processes required to place orders with those pharmacies;
- our ability to successfully internalize operation of our patient services hub from our third-party vendor;
- the ability of our patient services hub to provide adequate support for patients and physicians to prescribe and access our approved medicines;
- the timing of market introduction of any of our approved medicines as well as competitive products;
- the effectiveness of our sales, marketing and distribution efforts and those of the third parties with whom we contract;
- adverse publicity about our approved medicines or favorable publicity about competitive products;
- potential product liability claims;
- our ability to manage our growth and operations to effectively support our commercialization activities; and
- patient satisfaction leading to a high percentage of patients deriving clinical benefit and staying on our approved medicines chronically.

If any of our approved medicines fail to achieve the market acceptance among physicians, patients, tertiary care centers, transplant centers or others in the medical community necessary for commercial success, our operating results and financial condition will be adversely affected, which may delay, prevent or limit our ability to generate revenue and continue our business.

Our approved medicines and our product candidates may cause undesirable side effects or have other properties that could limit their commercial profile, expose us to product liability claims, delay or prevent regulatory approval of our product candidates or additional indications, or result in significant negative consequences following any additional marketing approval, any of which may adversely impact our business, financial condition, operating results and prospects.

As is the case with biopharmaceuticals generally, it is likely that there may be side effects and adverse events ("AEs") associated with use of our approved medicines and product candidates. Results of our clinical trials and expanded access program could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval of our product candidates by the FDA, European Commission or comparable foreign regulatory authorities. The drug-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. Any of these occurrences may harm our business, financial condition and prospects significantly.

In clinical trials of Livmarli in ALGS, the most commonly reported AEs were diarrhea, abdominal pain and vomiting, and were mostly mild to moderate in severity and transient in nature. Additionally, AEs reported in greater than 5% of patients included fat-soluble vitamin deficiency, nausea, liver transaminase increases, and bone fracture. The frequency of observed AEs has not increased over time. In the pivotal trial of Livmarli in PFIC, adverse reactions reported in greater than 5% of patients and greater than placebo included diarrhea, abdominal pain, increased transaminases, hematochezia or rectal hemorrhage, and bone fractures. Prescribing information for Livmarli includes warnings and precautions related to monitoring for and the risk of hepatotoxicity, gastrointestinal adverse reactions, fat-soluble vitamin deficiency and risk of propylene glycol toxicity (pediatric patients less than 5 years of age). In clinical trials of volixibat, the most common AEs reported were mild to moderate GI events (diarrhea, abdominal pain, nausea and vomiting) observed in the volixibat groups. In the interim analysis of the PBC VANTAGE study, the incidence of diarrhea in patients on volixibat was 77% with all cases mild to moderate; one patient discontinued the study due to an AE of diarrhea. The most common adverse reactions for Cholbam (≥1%) are diarrhea, reflux esophagitis, malaise, jaundice, skin lesion, nausea, abdominal pain, intestinal polyp, urinary tract infection, and peripheral neuropathy. The most common (≥ 20%) AEs seen in patients on chenodiol in the RESTORE clinical trial included diarrhea, constipation and headache.

Additionally, in respect of our approved medicines or if one or more of our product candidates receives marketing approval, and we or others (including regulatory approval authorities) later identify undesirable side effects caused by our approved medicines or such product candidates or other products with the same or related active ingredients, a number of potentially significant negative consequences could result, including, among other things:

- regulatory authorities may withdraw, suspend, or vary approvals of such product, including the FDA, European Commission or comparable foreign regulatory authorities withdrawing approval for the affected medicine;
- regulatory authorities may require additional warnings on the label;
- regulatory authorities may require a recall or we or our potential partners may voluntarily recall such product;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients at significant cost or instate a Risk Evaluation and Mitigation Strategies (“REMS”) or Risk Management Plan (“RMP”);
- regulatory authorities may require the addition of warnings, such as black box or other warnings, or contraindications in the product labeling that could diminish the usage of the product or otherwise limit the commercial success of the affected product;
- our ability to promote our approved medicines may be limited and we could be required to change administration of, or modify, such product in some other way;
- regulatory authorities may require us to modify, suspend or terminate our clinical trials, conduct additional clinical trials or engage in costly post-marketing testing and surveillance to monitor the safety or efficacy of such product;
- undesirable side effects may limit physicians’ or patients’ willingness to initiate or continue therapy with such product;
- sales may decrease significantly;
- we could be sued and held liable for harm caused to patients; and
- our corporate brand and reputation or the reputation of our approved medicines may suffer.

Such events could prevent us from achieving or maintaining market acceptance of our approved medicines, and could significantly harm our business, results of operations and prospects.

If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs in the U.S., we could be subject to additional reimbursement requirements, fines, sanctions and exposure under other laws which could have a material adverse effect on our business, results of operations and financial condition.

We participate in, or are subject to, the Medicaid Drug Rebate Program, as administered by Centers for Medicare & Medicaid Services (“CMS”), and other federal and state government pricing programs in the U.S., and we may participate, or be asked to participate, in additional government pricing programs or supplemental rebates in the future. These programs generally require us to pay rebates or otherwise provide discounts to government payors in connection

with drugs that are dispensed to beneficiaries/recipients of these programs. In some cases, such as with the Medicaid Drug Rebate Program, the rebates are based on pricing that we report to the government agencies that administer the programs. Pricing requirements and rebate/discount calculations are complex, vary among products and programs, and are often subject to interpretation by governmental or regulatory agencies and the courts. The requirements of these programs, including, by way of example, their respective terms and scope, change frequently. For example, on March 11, 2021, the American Rescue Plan Act of 2021 was signed into law, which eliminated the statutory Medicaid drug rebate cap, currently set at 100% of a drug's average manufacturer's price ("AMP"), for single source and innovator multiple source drugs, effective January 1, 2024. Responding to current and future changes may increase our costs, and the complexity of compliance will be time consuming. Invoicing for rebates is provided in arrears, and there is frequently a time lag of up to several months between the sales to which rebate notices relate and our receipt of those notices, which further complicates our ability to accurately estimate and accrue for rebates related to the Medicaid program as implemented by individual states. Thus, there can be no assurance that we will be able to identify all factors that may cause our discount and rebate payment obligations to vary from period to period, and our actual results may differ significantly from our estimated allowances for discounts and rebates. Changes in estimates and assumptions may have a material adverse effect on our business, results of operations and financial condition. In addition, the U.S. Department of Health and Human Services ("HHS") Office of Inspector General and other Congressional, enforcement and administrative bodies have recently increased their focus on pricing requirements for products, including, but not limited to the methodologies used by manufacturers to calculate AMP, and best price, for compliance with reporting requirements under the Medicaid Drug Rebate Program. Additionally, several states have a practice of asking, or are increasing activity in requesting supplemental rebates, for covered products. We are liable for errors associated with our submission of pricing data and for any overcharging of government payors. For example, failure to submit monthly/quarterly AMP and best price data on a timely basis could result in significant civil monetary penalties for each day the submission is late beyond the due date. Failure to make necessary disclosures and/or to identify overpayments could result in allegations against us under the civil False Claims Act and other laws and regulations. Any required refunds to the U.S. government or responding to a government investigation or enforcement action would be expensive and time consuming and could have a material adverse effect on our business, results of operations and financial condition. In addition, in the event that the CMS were to terminate our rebate agreement, no federal payments would be available under Medicaid or Medicare for our covered outpatient drugs.

We may face product liability exposure, and if claims are brought against us, we may incur substantial liability if our insurance coverage for those claims is inadequate.

We face an inherent risk of product liability suits for our approved medicines and product candidates. Our approved medicines and our product candidates are designed to affect important bodily functions and processes. Any side effects, manufacturing defects, failure to follow instructions, misuse or abuse associated with our approved medicines or our product candidates could result in injury to a patient or even death. In addition, a liability claim may be brought against us even if our approved medicines or our product candidates merely appear to have caused an injury. Product liability claims may be brought against us by, among others, consumers, their family members, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our approved medicines or product candidates. If we are the target of product liability claims, we will incur substantial legal costs, potential liabilities and could incur reputational harm if we do not successfully defend ourselves.

In addition, regardless of merit or eventual outcome, product liability claims may result in, among other things:

- the inability to commercialize our approved medicines or product candidates, if approved;
- decreased demand for our approved medicines or product candidates;
- termination of clinical trial sites or entire trial programs;
- product recall or withdrawal from the market or labeling, marketing or promotional restrictions;
- impairment of our business reputation and negative media attention;
- substantial costs of any related litigation or similar disputes;
- distraction of management's attention and other resources and employees from our primary business;
- substantial monetary awards to patients or other claimants against us that may not be covered by insurance; and
- loss of revenue.

Large judgments have been awarded in class action and individual lawsuits based on drugs that had anticipated or unanticipated side effects. Although we have obtained product liability insurance coverage, our insurance coverage may

not be sufficient to cover all of our product liability related expenses or losses and may not cover us for any expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive and, in the future, we may not be able to maintain insurance coverage at a reasonable cost, in sufficient amounts or upon adequate terms to protect us against losses due to product liability. A successful product liability claim or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could decrease our cash and could harm our business, financial condition, operating results and prospects.

If we are found to have improperly promoted off-label uses of our approved medicines, or unapproved uses of our product candidates, if approved, or if we are found to be the cause of physician misuse or off-label use of our approved medicines or our product candidates, if approved, we may become subject to prohibitions on the sale or marketing of such products, product liability claims and significant fines, penalties and sanctions, and our brand and reputation could be harmed.

The FDA, European Commission, Health Canada, competent authorities of individual EU Member States, and comparable foreign regulatory authorities strictly regulate the marketing and promotional claims that are made about drug and biologic products. In particular, a product may not be promoted for uses or indications that are not approved by the FDA, European Commission, Health Canada or comparable foreign regulatory authorities as reflected in the product's approved labeling and comparative safety or efficacy claims cannot be made without direct comparative clinical data. For example, although Livmarli may appeal to individuals who have not been diagnosed with cholestatic pruritus associated with ALGS or PFIC or suffer from other forms of cholestatic pruritus like those included in our PHASE 3 EXPAND trial, we (and our collaborators, where applicable) are only able to promote Livmarli:

- in the U.S. for cholestatic pruritus associated with ALGS in patients three months of age and older and for cholestatic pruritus in PFIC patients twelve months of age and older;
- in the EU for the treatment of cholestatic pruritus in patients with ALGS two months of age and older and for the treatment of PFIC in patients three months of age and older; and
- in Japan for the treatment of cholestatic pruritus in patients with ALGS and PFIC.

Additionally, Ctexli is only indicated for adults with CTX, not for individuals under the age of 18, even though it may be prescribed by healthcare providers for that population. If we are found to have promoted off-label uses of our approved medicines or product candidates, we may receive warning or untitled letters and become subject to significant criminal and civil liability, which would materially harm our business. Further, in the U.S., both federal and state governments have levied large civil and criminal fines against companies for alleged improper off-label promotion and have enjoined several companies from engaging in off-label promotion and to undertake corrective remedies.

If we become the target of such an investigation or prosecution based on our marketing and promotional practices, we could face similar sanctions, which would materially harm our business. In addition, management's attention could be diverted from our business operations, significant legal expenses could be incurred and our brand and reputation could be damaged. In some instances, the FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed. If we are deemed by the FDA to have engaged in the promotion of off-label uses, we could be subject to FDA regulatory or enforcement actions as well as by other federal, state or foreign enforcement authorities that might take action if they consider our business activities to constitute promotion of an off-label use, which could result in significant penalties, including criminal, civil or administrative penalties, damages, fines, disgorgement, exclusion from participation in government healthcare programs and the curtailment or restructuring of our operations. For example, if such off-label promotion results in the submission of a reimbursement claim to a governmental healthcare program, we could be found liable under the U.S. False Claims Act. In cases where off-label promotion has resulted in violations of other statutes, the U.S. Department of Justice ("DOJ") has also required companies to enter into deferred prosecution agreements or corporate integrity agreements.

Our approved medicines and our product candidates are subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense. We may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with any product.

Any regulatory approvals that we receive may be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including post-market studies or clinical trials, and surveillance to monitor safety and effectiveness. The FDA may also require a REMS in order to approve a product candidate, which could entail requirements for a medication guide, physician communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. Similarly, the European Commission may require a RMP in order to collect additional information on a medicine's safety profile which may include plans for pharmacovigilance activities and

measures to minimize risks. In addition, if the FDA, European Commission or comparable foreign regulatory authorities approve a product candidate, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, post-marketing obligations, storage, advertising, promotion, import, export and recordkeeping for the approved product will be subject to extensive and ongoing regulatory requirements. For example, we are subject to ongoing FDA and European Commission obligations and continued regulatory review with respect to, among other things, the manufacturing, processing, labeling, packaging, distribution, AE reporting, storage, advertising, promotion and recordkeeping for Livmarli, which requirements include submissions of safety and other post-marketing information and reports and registration, as well as continued compliance with current good manufacturing practices ("cGMP") requirements and with the FDA's and equivalent foreign good clinical practice ("GCP"). We are also subject to post-marketing obligations for Cholbam including the conduct and submission of registry studies.

In addition, Livmarli was the subject of a marketing authorization granted by the European Commission under exceptional circumstances in accordance with Article 14.8 of Regulation (EC) No 726/2004 relating to the authorization and supervision of medicines for human and veterinary use and establishing the EMA. This type of authorization is reviewed annually to reassess the risk-benefit balance of the medicine. The purpose of any specific procedures/obligations imposed as part of the marketing authorization granted in exceptional circumstances is to contribute to the provision of information on the safe and effective use of the product. Grant of a marketing authorization in exceptional circumstances is renewable for one-year periods and will normally not lead to the completion of a full dossier/approval.

We are subject to various FDA and EU post-marketing requirements across our approved medicines, including the conduct and submission of non-clinical, clinical and registry studies and the FDA's and EU's prohibition against marketing medicines in uses that are not approved. These and similar requirements could be imposed by the FDA, European Commission or comparable foreign regulatory authorities for any approved product.

In addition, manufacturers of drug and biologic products and their facilities are subject to continual review and periodic inspections by the FDA, the competent authorities of the individual EU Member States, or comparable foreign regulatory authorities for compliance with cGMP regulations. If we or a regulatory authority discovers previously unknown problems with a product, such as AEs of unanticipated severity or frequency, or problems with the facility where, or processes by which, the product is manufactured, such events may result in, among other things:

- restrictions on the marketing or manufacturing of the product, withdrawal of the product from the market, or voluntary or mandatory product recalls;
- fines, untitled letters, warning letters or holds on clinical trials;
- refusal by the FDA or European Commission to approve pending applications or supplements to approved applications filed by us or suspension or revocation of license approvals;
- product seizure or detention, or refusal to permit the import or export of a product; and
- injunctions or the imposition of civil or criminal penalties.

The occurrence of any event or penalty described above or any similar event or penalty may inhibit our ability to commercialize our approved medicines or our product candidates and generate revenue and could require us to expend significant time and resources in response and could generate negative publicity.

The FDA's, European Commission's and comparable foreign regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval for our product candidates or restrict marketing of any then-approved product. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would adversely affect our business, prospects, financial condition and results of operations. For example, in April 2023 the European Commission adopted a proposal for a new Directive and Regulation to revise the existing pharmaceutical legislation. In April 2024, the Parliament adopted its related position and in June 2025, the European Council agreed on its position. The Council, the Parliament and the European Commission have begun trilogue negotiations with a view to reaching an agreement on the package. Regulatory exclusivities are a key topic of the negotiations. A decrease in data and market exclusivity opportunities for our product candidates in the EU could make them open to generic or biosimilar competition earlier than is currently the case with a related reduction in reimbursement status.

We may pursue approval in the U.S., Canada or certain countries in Europe using accelerated, exceptional circumstances or conditional approval pathways, which typically require commitments to complete additional clinical trials. The additional clinical trials may not confirm the treatment effect, which may result in the loss of marketing authorization under accelerated approval, exceptional circumstances or conditional approval.

Our business depends, in part, on the success of our product candidates, each of which requires significant clinical testing before we can seek regulatory approval and potentially launch commercial sales.

Our business and future success depends, in part, on our ability to obtain regulatory approval for, and then successfully commercialize our product candidates. Our product candidates will require clinical development, regulatory review and approval in multiple jurisdictions, substantial investment, access to sufficient manufacturing capacity and significant marketing efforts before we can generate any revenue from product sales. Further, we are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA, European Commission or comparable foreign regulatory authorities, and we may never receive such regulatory approvals for our product candidates.

Our clinical trials may not be successful and may not be completed on time or at all, and the FDA, EMA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials. For example, in certain of our ongoing clinical trials, the primary efficacy endpoint is a patient-reported outcome or a caregiver-reported outcome measuring the decrease in severity of pruritus. The FDA, EMA or comparable foreign regulatory authorities may not accept such patient-reported outcomes or caregiver-reported outcomes as validated. If modifications are needed for our study design to support the submission of an application for marketing approval, incorporating such modifications may be costly and could lead to delays in obtaining approval from the FDA, European Commission or comparable foreign regulatory authorities, which may significantly, adversely and materially affect our ability to successfully commercialize our product candidates. Further, even if we make changes to the study design to address these considerations, the FDA, European Commission or comparable foreign regulatory authorities may not approve our product candidates.

Even if such regulatory authorities agree with the design and implementation of our clinical trials, such regulatory authorities may change their requirements in the future. In addition, even if the clinical trials are successfully completed, the FDA, EMA or comparable foreign regulatory authorities may not interpret the results as we do, and more clinical trials could be required before we submit our product candidates for approval.

To the extent that the results of our clinical trials are not satisfactory to the FDA, EMA or comparable foreign regulatory authorities for support of a marketing application, approval for our product candidates may be significantly delayed or prevented, or we may be required to expend significant additional resources, which may not be available to us, to conduct additional clinical trials in support of potential approval for our product candidates.

We have encountered and may continue to encounter delays and difficulties enrolling patients in our clinical trials, and as a result, our clinical development activities could be delayed or otherwise adversely affected.

Patient enrollment, a significant factor in the timing of clinical trials, is generally affected by many factors including, but not limited to, the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the clinical trial, the design of the clinical trial, competing clinical trials and clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating.

For example, each indication for which we are evaluating Livmarli and volixibat is a rare cholestatic liver disease with limited patient populations from which to draw participants in clinical trials. We will be required to identify and enroll a sufficient number of patients with the disease under investigation for each of our ongoing and planned clinical trials of Livmarli and volixibat. Potential patients may not be adequately diagnosed or identified with the diseases which we are targeting or may not meet the entry criteria for our trials. In addition, patients may ultimately decide not to enroll in a particular clinical trial for reasons outside of our control. We may seek to conduct clinical trials in countries in which we have not previously conducted trials for our product candidates and in which we have not yet worked with the competent regulatory authorities. As a result, we could face patient recruitment issues in certain countries where such foreign regulatory authorities are not familiar with our product candidates. Additionally, other pharmaceutical companies targeting the same cholestatic liver diseases are recruiting clinical trial patients from these patient populations, and have expanded access programs available, which have delayed enrollment in our clinical trials. Our inability to enroll a sufficient number of patients for any of our current or future clinical trials would result in significant delays. As a result, we may need to delay the completion of such trials beyond our expected timelines or abandon one or more clinical trials altogether.

Our clinical trials may fail to adequately demonstrate the safety and efficacy of our product candidates, which could prevent or delay regulatory approval and commercialization.

Before obtaining regulatory approvals for the commercial sale of a product candidate, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that a product candidate is both safe and effective for use in each target indication. Our clinical trials have in the past and could in the future fail to demonstrate

safety and efficacy of the product candidate studied for the target indication. For example, in December 2023, we announced that our Phase 2b EMBARK clinical trial evaluating Livmarli in patients with biliary atresia ("BA") did not meet its primary or key secondary endpoints. Most product candidates that commence clinical trials are never approved by regulatory authorities for commercialization. In the case of our product candidates, we are seeking to develop treatments for rare diseases for which there is limited clinical experience, and our planned clinical trials use novel end points and measurement methodologies, which add complexity to the conduct of and analysis of data from our clinical trials and may delay or prevent regulatory approval.

Clinical drug development involves a lengthy and expensive process with uncertain outcomes, and results of earlier studies and trials may not be predictive of future trial results.

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or safety profiles, notwithstanding promising results in earlier trials. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses. Further, if patients drop out of our clinical trials, miss scheduled doses or follow-up visits or otherwise fail to follow clinical trial protocols, or if our clinical trials are otherwise disrupted, the integrity of data from our clinical trials may be compromised or not accepted by the FDA, EMA or comparable foreign regulatory authorities, which would represent a significant setback for the applicable program. Additional safety data generated from our expanded access program and post-marketing studies could be different from, including less favorable than, the data generated and discussed with regulatory authorities to date. Our clinical trials may not be successful, and any safety concerns observed in any one of our clinical trials in our targeted indications could limit the prospects for regulatory approval of our product candidates in other indications.

Any delays in the commencement or completion, or termination or suspension, of our clinical trials could result in increased costs for us, delay or limit our ability to generate revenue and adversely affect our commercial prospects.

Before we can initiate clinical trials for our product candidates, we must submit the results of preclinical studies to the FDA, EMA or comparable foreign regulatory authorities along with other information, including information about product candidate chemistry, manufacturing and controls, and our proposed clinical trial protocol, as part of an IND application or similar regulatory filing. Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates in humans. Clinical testing is expensive, time consuming and uncertain as to outcome.

We do not know whether our planned clinical trials will begin on time or be completed on schedule, if at all. The commencement and completion of clinical trials can be delayed for a number of reasons, including delays related to:

- the FDA, EMA or comparable foreign regulatory authorities disagreeing as to the design or implementation of our clinical trials or agreement to commence our clinical trials;
- the FDA's, EU Member State competent authorities', or comparable foreign regulatory authorities' failure to accept our proposed manufacturing processes and suppliers and/or requirement to provide additional information regarding our manufacturing processes before providing marketing authorization;
- any failure or delay in reaching an agreement with CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;
- obtaining approval from one or more institutional review boards ("IRBs") or positive ethics committee opinions;
- IRBs or ethics committees refusing to approve or provide positive opinions, suspending or terminating the clinical trial at an investigational site, precluding enrollment of additional subjects, or withdrawing their approval of the clinical trial;
- changes to clinical trial protocol;
- selection of clinical end points that require prolonged periods of clinical observation or analysis of the resulting data;
- sites deviating from clinical trial protocol or dropping out of a clinical trial;

- manufacturing sufficient quantities of product candidate or obtaining sufficient quantities of combination therapies for use in clinical trials;
- subjects failing to enroll or remain in our trial at the rate we expect, or failing to return for post-treatment follow-up;
- subjects choosing an alternative treatment for the indication for which we are developing our product candidates, or participating in competing clinical trials;
- lack of adequate funding to continue the clinical trial;
- subjects experiencing severe or unexpected drug-related AEs;
- occurrence of serious adverse events ("SAEs") in clinical trials of the same class of agents conducted by other companies;
- a facility manufacturing our product candidates or any of their components being ordered by the FDA, EU Member State competent authorities, or comparable foreign regulatory authorities to temporarily or permanently shut down due to violations of cGMP, regulations or other applicable requirements, or infections or cross-contaminations of product candidates in the manufacturing process;
- any changes to our manufacturing process, suppliers or formulation that may be necessary or desired;
- the impact of geopolitical and macroeconomic developments on our ongoing and planned clinical trials; and
- failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA, EMA, competent authorities of individual EU Member States or comparable foreign regulatory authorities resulting in the imposition of a clinical hold, suspension or termination.

Further, conducting clinical trials in foreign countries presents additional risks that may delay completion of our clinical trials. These risks include the failure of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries.

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authorities may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA or comparable foreign regulatory authorities may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA, European Commission or comparable foreign regulatory authorities, as the case may be, and may ultimately lead to the denial of marketing approval of one or more of our product candidates.

If we experience delays in the completion of, or termination of, any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, and our ability to generate product revenue from any of these product candidates will be delayed. Moreover, any delays in completing our clinical trials will increase our costs, slow down our product candidate development and approval process and jeopardize our ability to commence product sales and generate revenue. In addition, many of the factors that cause, or lead to, termination or suspension of, or a delay in the commencement or completion of, clinical trials may also ultimately lead to the denial of regulatory approval of a product candidate. Any delays to our clinical trials that occur as a result could shorten any period during which we may have the exclusive right to commercialize our product candidates and our competitors may be able to bring products to market before we do, and the commercial viability of our product candidates could be significantly reduced. Any of these occurrences may harm our business, financial condition and prospects significantly.

Our product candidates are subject to extensive regulation and compliance, which is costly and time consuming, and such regulation may cause unanticipated delays or prevent the receipt of the required approvals to commercialize our product candidates.

The clinical development, manufacturing, labeling, storage, record-keeping, advertising, promotion, import, export, marketing and distribution of our product candidates are subject to extensive regulation by the FDA in the U.S., the EU and EU Member State competent authorities and by comparable foreign regulatory authorities in other markets. In the U.S., the EU and many foreign countries, we are not permitted to market our product candidates until we receive regulatory approval from the FDA, European Commission or comparable foreign regulatory authorities. The process of obtaining

regulatory approval is expensive, often takes many years following the commencement of clinical trials and can vary substantially based upon the type, complexity and novelty of the product candidates involved, as well as the target indications and patient population. Approval policies or regulations may change, and regulatory authorities have substantial discretion in the drug approval process, including the ability to delay, limit or deny approval of a product candidate for many reasons. Despite the time and expense invested in clinical development of product candidates, regulatory approval is never guaranteed.

Prior to obtaining approval to commercialize a product candidate in the U.S. or internationally, we must demonstrate with substantial evidence from adequate and well-controlled clinical trials, and to the satisfaction of the FDA, EMA or comparable foreign regulatory authorities, that such product candidates are safe and effective for their intended uses. Results from non-clinical studies and clinical trials can be interpreted in different ways. Even if we believe the non-clinical or clinical data for our product candidates are promising, such data may not be sufficient to support approval by the FDA, European Commission or comparable foreign regulatory authorities. The FDA, EMA or comparable foreign regulatory authorities, as the case may be, may also require us to conduct additional preclinical studies or clinical trials for our product candidates either prior to or post-approval, or may object to elements of our clinical development program. If we were required to conduct such additional preclinical studies or clinical trials, the FDA, EMA or comparable foreign regulatory authorities may not agree with our interpretation of the results and we may not receive approval for our product candidates or additional indications, or marketing of our approved medicines may be subject to additional requirements.

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

- the FDA, EMA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials or the validation of our caregiver and patient reported outcome instruments;
- serious and unexpected drug-related side effects may be experienced by participants in our clinical trials or by individuals using drugs similar to our product candidates;
- the population studied in the clinical trial may not be sufficiently broad or representative to assure safety in the full population for which we seek approval;
- the FDA, EMA or comparable foreign regulatory authorities may not accept clinical data from trials which are conducted at clinical facilities or in countries where the standard of care is potentially different from that of the U.S., the EU or the applicable foreign jurisdiction;
- we may be unable to demonstrate to the satisfaction of the FDA, EMA or comparable foreign regulatory authorities that a product candidate is safe and effective for any of its proposed indications;
- the results of clinical trials may not meet the level of statistical significance required by the FDA, EMA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA, EMA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to satisfy the FDA, EMA or comparable foreign regulatory authorities to support the submission of an NDA or other comparable submissions in the EU or other foreign jurisdictions or to obtain regulatory approval in the U.S. or elsewhere;
- approval or orphan status may be blocked or rejected by the FDA or the European Commission;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA, European Commission or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

Any of the above events could prevent us from achieving market approval of our product candidates and could substantially increase the costs of commercializing our product candidates. The demand for our product candidates could also be negatively impacted by any adverse effects of a competitor's product or treatment.

Of the large number of drugs in development, only a small percentage successfully complete the FDA, European Commission or comparable foreign regulatory approval processes and are commercialized. The lengthy approval

process 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, financial condition, results of operations and prospects.

Even if we eventually complete clinical trials and receive approval of an NDA or equivalent EU or foreign marketing application for our product candidates, the FDA, European Commission or comparable foreign regulatory authorities may grant approval contingent on the performance of costly additional clinical trials, including Phase 4 clinical trials, and/or the implementation of a REMS in the U.S. or RMP in the EU, which may be required to ensure safe use of the drug after approval. The FDA, European Commission or comparable foreign regulatory authorities also may approve a product candidate for a more limited indication or patient population than we originally requested, and the FDA, European Commission or comparable foreign regulatory authorities may not approve the labeling that we believe is necessary or desirable for the successful commercialization of a product. Any delay in obtaining, or inability to obtain, applicable regulatory approval would delay or prevent commercialization of that product candidate and would materially adversely impact our business and prospects.

If we fail to develop and commercialize additional product candidates, we may be unable to grow our business. Further, we may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

We plan to acquire rights to develop and commercialize product candidates in addition to our current approved medicines and our current product candidates. If we decide to pursue the development and commercialization of any additional product candidates, we may be required to invest significant resources to acquire or in-license the rights to such product candidates or to conduct drug discovery activities. We do not currently have the necessary drug discovery personnel or expertise adequate to discover and develop an additional product candidate on our own. Any other product candidates will require additional, time-consuming development efforts, and significant financial resources, prior to commercial sale, including preclinical studies, extensive clinical trials and approval by the FDA, European Commission or comparable foreign regulatory authorities. All product candidates are prone to the risks of failure that are inherent in pharmaceutical product development, including the possibility that the product candidate will not be shown to be sufficiently safe and/or effective for approval by regulatory authorities. Because we have limited financial and personnel resources, we focus on specific product candidates for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or other indications that later prove to have greater commercial potential. We may focus our efforts and resources on product candidates that ultimately prove to be unsuccessful.

In addition, we may not be able to acquire, discover or develop any additional product candidates, and any additional product candidates we may develop may not be approved, manufactured or produced economically, successfully commercialized or widely accepted in the marketplace or be more effective than other commercially available alternatives. Research programs to identify and efforts to acquire new product candidates require substantial technical, financial and human resources whether or not we ultimately identify or acquire any candidates. If we are unable to acquire, develop or commercialize any other product candidates on favorable terms or at all, our business and prospects will suffer.

Risks Related to Our Business and Industry

We have incurred substantial net losses since our inception and anticipate that we will continue to incur losses for the foreseeable future.

Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effectiveness in the targeted indication or an acceptable safety profile, gain regulatory approval and become commercially viable. While we have three medicines approved for commercial sale, we continue to incur significant research and development and other expenses related to our ongoing operations. As a result, we are not profitable and have incurred substantial net losses since our inception in May 2018. For the nine months ended September 30, 2025 and 2024, we reported a net loss of $17.6 million and $64.2 million, respectively. As of September 30, 2025, we had an accumulated deficit of $661.8 million.

While we generated net income in the third quarter of 2025, we expect to continue to incur net losses for the foreseeable future as we look to acquire products and product candidates, continue our clinical development of, and seek regulatory approvals for, our product candidates and as we continue commercializing our approved medicines in the U.S., Canada and in certain countries in Europe. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. Our prior net losses and expected future net

losses have had and will continue to have an adverse effect on our stockholders' equity and working capital. Because of the numerous risks and uncertainties associated with drug development, we are unable to accurately predict the timing or amount of increased expenses, or when, if at all, we will be able to achieve profitability.

If the market opportunities for our product candidates are smaller than we believe they are, our future revenue may be adversely affected, and our business may suffer.

If the size of the market opportunities in each of our target indications or for any assets or product candidates that we may acquire, such as the Bile Acid Medicines, is smaller than we anticipate, we may not be able to achieve profitability and growth. We focus the clinical development and commercialization of our approved medicines on rare diseases with relatively small patient populations. Given the small number of patients who have the diseases that we are targeting, it is critical to our ability to grow and become profitable that we continue to successfully identify patients with these rare diseases. In addition, our estimates of the patient populations for our target indications have been derived from a variety of sources, including scientific literature, surveys of clinics, patient foundations, and market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. The effort to identify patients with diseases we seek to treat is in early stages, and we cannot accurately predict the number of patients for whom treatment might be possible. Additionally, the potentially addressable patient population for each of our product candidates may be limited or may not be amenable to treatment with our product candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business. Lastly, the potentially addressable patient population for any of our potential indications may even be further reduced if gene therapy products become more widely accepted and approved.

Obtaining and maintaining regulatory approval for a product candidate in one jurisdiction does not mean that we will be successful in obtaining regulatory approval for that product candidate in other jurisdictions.

Obtaining and maintaining regulatory approval for a product candidate in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction, while a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. For example, even if the FDA grants marketing approval for a product candidate, comparable foreign regulatory authorities in foreign jurisdictions must also approve the manufacturing, marketing and promotion of the product candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the U.S., including additional preclinical studies or clinical trials as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the U.S., a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our product candidates is also subject to approval.

Regulatory authorities in jurisdictions outside of the U.S. and the EU have requirements for approval for product candidates with which we must comply prior to marketing in those jurisdictions, and the regulatory approval process outside the U.S. and EU generally includes all of the risks associated with obtaining FDA and European Commission approval. Consequently, approval by the FDA does not ensure approval by the European Commission, approval by the European Commission does not assure approval by the FDA, and approval of either or both of the FDA and European Commission does not assure approval by regulatory authorities in other countries or jurisdictions. Further, marketing approvals in countries outside the U.S., including in the EU, do not ensure pricing approvals in those countries or in any other countries, and marketing approvals and pricing approvals do not ensure that reimbursement will be obtained. If we fail to comply with the regulatory requirements in international markets and/or receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of any of our approved medicines or product candidates, if approved, will be harmed, which would adversely affect our business, prospects, financial condition and results of operations.

Disruptions at the FDA, EMA and other foreign regulatory authorities caused by layoffs, funding shortages or global health concerns could negatively impact our business.

The ability of the FDA, the competent authorities of EU Member States and other foreign regulatory authorities to review and approve proposed clinical trials or new product candidates can be affected by a variety of factors, including, but not limited to, government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, statutory, regulatory, and policy changes, and other events that may otherwise affect these regulatory agencies' ability to perform routine functions. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the

political process, including executive and congressional priorities, the impacts of which are inherently fluid and unpredictable.

Disruptions at the FDA, European Commission, EMA and other foreign regulatory authorities may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary regulatory authorities, which would adversely affect our business. For example, over the last several years, including beginning on October 1, 2025 and continuing to the present, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities to the extent they are not funded by existing available user fees. Repeated or prolonged government shutdowns or global health concerns could prevent the FDA, EMA or comparable foreign regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities and could significantly impact the ability of the FDA, European Commission, EMA or comparable foreign regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business. In addition, the current U.S. administration has enacted substantial reductions in force at various U.S. government agencies that, if applied to the FDA in a material way, could significantly reduce the FDA's capacity to perform its functions in a manner consistent with its past practices and could negatively impact our business.

Recently enacted legislation, future legislation and healthcare reform measures may increase the difficulty and cost for us to obtain marketing approval for and commercialize our product candidates and may affect the prices we may set.

In the U.S., certain European countries, and some other foreign jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes to the healthcare system, including cost-containment measures that may reduce or limit coverage and reimbursement for newly approved drugs and affect our ability to profitably sell any product candidates for which we obtain marketing approval. For example, Germany introduced changes to its laws governing reimbursement of medicines that impact, among others, orphan designation medicines. Previously, orphan designation drugs were presumed to provide an additional benefit upon obtaining a marketing authorization and, therefore, subject to a "limited assessment" by the German authority competent for reimbursement (G-BA) as long as the sales of the orphan designation medicine remained under the threshold of € 50 million in 12 months. Above this threshold, an orphan designation medicine would be subject to a full assessment by the G-BA regarding its benefits compared to an appropriate comparator therapy, just like any other medicine. This threshold will be lowered to € 30 million in the future, and if we exceed this threshold, we will need to undergo a full assessment in accordance with the German laws governing reimbursement, which may impact the reimbursement of our approved medicines. Other countries may adopt similar or new approaches that may impact the reimbursement of our product(s), increase our operating costs and impact profitability. Additionally, if adopted in the form proposed, the recent European Commission proposals to revise the existing EU laws governing authorization of medicines may result in a decrease in data and market exclusivity for our product candidates in the EU.

There have also been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare. For example, in March 2010, the Patient Protection and Affordable Care Act ("Affordable Care Act") was enacted in the U.S. Among the provisions of the Affordable Care Act of importance to our potential product candidates, the Affordable Care Act: established an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents; expanded eligibility criteria for Medicaid programs; increased the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program; creates a Medicare Part D coverage gap discount program; established a Patient-Centered Outcomes Research Institute to oversee, identify priorities in and conduct comparative clinical effectiveness research, along with funding for such research; and established a Center for Medicare & Medicaid Innovation at CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending.

There have been amendments and executive, judicial and Congressional challenges to certain aspects of the Affordable Care Act. In addition, the Affordable Care Act has been subject to various health reform measures. On August 16, 2022, the Inflation Reduction Act of 2022 ("IRA") was signed into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in Affordable Care Act marketplaces through plan year 2025. The IRA also eliminates the "donut hole" under the Medicare Part D program beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost and through a newly established manufacturer discount program. It is unclear how any additional health care reform measures of the current presidential administration will impact the Affordable Care Act and our business.

Further, the IRA will, among other things, (i) allow HHS to negotiate the price of certain single-source drugs and biologics that have been on the market for at least 7 years covered under Medicare, and subject drug manufacturers to civil monetary penalties and a potential excise tax by offering a price that is not equal to or less than the negotiated "maximum fair price" under the law (the "Medicare Drug Price Negotiation Program") and (ii) impose rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation. The IRA permits HHS to

implement many of these provisions through guidance, as opposed to regulation, for the initial years. HHS has and will continue to issue and update guidance as these programs are implemented. These provisions took effect progressively starting in fiscal year 2023. On August 15, 2024, HHS announced the agreed-upon reimbursement prices for the first ten drugs that were subject to price negotiations, although the Medicare drug price negotiation program is currently subject to legal challenges. On January 17, 2025, HHS selected fifteen additional products covered under Part D for price negotiation in 2025. Each year thereafter more Part B and Part D products will become subject to the Medicare Drug Price Negotiation Program.

Other legislative changes have been proposed and adopted in the U.S. since the Affordable Care Act was enacted. On July 4, 2025, the OBBBA was signed into law, which is expected to reduce Medicaid spending and enrollment by implementing work requirements for some beneficiaries, capping state-directed payments, reducing federal funding, and limiting provider taxes used to fund the program. The OBBBA also narrows access to Affordable Care Act marketplace exchange enrollment and declines to extend the Affordable Care Act enhanced advanced premium tax credits, set to expire at the end of 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. Further, through the process created by the Budget Control Act of 2011,there are automatic reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and, due to subsequent legislative amendments to the statute, including the Bipartisan Budget Act of 2018 and the Consolidated Appropriations Act of 2023, will remain in effect until 2032 unless additional Congressional action is taken. Additionally, on March 11, 2021, the American Rescue Plan Act of 2021 was signed into law, which eliminates the statutory Medicaid drug rebate cap, previously set at 100% of a drug's average manufacturer price, for single source and innovator multiple source drugs, effective January 1, 2024.

Further, there has been heightened governmental scrutiny in the U.S. of pharmaceutical pricing practices in light of the rising cost of prescription drugs. Such scrutiny has resulted in several recent Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products.

On December 8, 2023, the National Institute of Standards and Technology published for comment a Draft Interagency Guidance Framework for Considering the Exercise of March-In Rights which for the first time includes the price of a product as one factor an agency can use when deciding to exercise march-in rights. While march-in rights have not previously been exercised, it is uncertain if that will continue under the new framework.

At the state level, individual states in the U.S. have also increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, results of operations, financial condition and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our approved medicines and our other product candidates, if approved, or put pressure on our product pricing, which could negatively affect our business, results of operations, financial condition and prospects.

We expect that the Affordable Care Act, the IRA and other healthcare reform measures that may be adopted in the future may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from third-party payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our approved medicines and our product candidates, if approved. Further, the overall funding of certain government programs such as Medicaid and Medicare is uncertain and there is no guarantee that funds approved by the U.S. Congress will be made available by the current administration. The current administration is pursuing policies to reduce regulations and expenditures across government including at HHS, the FDA, CMS and related agencies. These actions, presently directed by executive orders or memoranda from the Office of Management and Budget, may propose policy changes that create additional uncertainty for our business. These actions include, for example, (1) directives to reduce agency workforce; (2) rescinding a Biden administration executive order tasking the Center for Medicare and Medicaid Innovation to consider new payment and healthcare models to limit drug spending; (3) eliminating the Biden administration's executive order that directed HHS to establish an AI task force and developing a strategic plan; (4) directing HHS and other agencies to lower prescription drug costs through a variety of initiatives, including by improving upon the Medicare Drug Price Negotiation Program and establishing Most-Favored-Nation pricing for pharmaceutical products; (5) imposing tariffs on imported pharmaceutical

products; (6) directing certain federal agencies to enforce existing law regarding hospital and plan price transparency and by standardizing prices across hospitals and health plans; and (7) as part of the Make America Healthy Again Commission's recent Strategy Report, working across government agencies to increase enforcement on direct-to-consumer pharmaceutical advertising. These actions and policies may significantly reduce U.S. drug prices, potentially impacting manufacturers' global pricing strategies and profitability, while increasing their operational costs and compliance risks. In September 2025, the current administration announced the first agreement with a major pharmaceutical company that requires the drug manufacturer to offer, through a direct to consumer platform, U.S. patients and Medicaid programs prescription drug Most-Favored Nation pricing equal to or lower than those paid in other developed nations, with additional mandates for direct-to-patient discounts and repatriation of foreign revenues. In the event Most-Favored-Nation pricing for pharmaceutical products is implemented and applicable to the approved medicines that we commercialize outside of the U.S., our revenue opportunities may be adversely affected, as we expect that our U.S. pricing would have to be reduced to the lowest price paid for the applicable product outside of the U.S. In such event, we may choose to forgo the ex-U.S. market to preserve more favorable U.S. pricing. Additionally, in its June 2024 decision in *Loper Bright Enterprises v. Raimondo* ("Loper Bright"), the U.S. Supreme Court overturned the longstanding Chevron doctrine, under which courts were required to give deference to regulatory agencies' reasonable interpretations of ambiguous federal statutes. The Loper Bright decision could result in additional legal challenges to current regulations and guidance issued by federal agencies applicable to our operations, including those issued by the FDA. Congress may introduce and ultimately pass health care related legislation that could, among others, impact the drug approval process, modify the Medicare Drug Price Negotiation Program, and reduce Medicaid enrollment and funding. We expect additional health reform measures may be implemented in the future.

A variety of risks associated with marketing our product candidates internationally could materially adversely affect our business.

We already have and plan to seek further regulatory approval for our product candidates internationally and, accordingly, we are subject to additional risks related to operating in foreign countries if and when we obtain the necessary approvals, including:

- differing regulatory requirements in foreign countries, including differing reimbursement, pricing and insurance regimes;
- the potential for regulatory approvals in other countries to result in re-examination of previously approved regulatory submissions in other countries;
- the potential for so-called parallel importing, which is what happens when a local seller, either with government approval or faced with high or higher local prices, opts to import goods from a foreign market (with low or lower prices) rather than buying them locally;
- changes in tariffs (including tariffs imposed by the U.S. and retaliatory tariffs, if any, imposed by U.S. trading partners), trade barriers, price and exchange controls and other regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets, including as a result of high interest rates and ongoing military conflicts, as well as any related political or economic responses and counter-responses or otherwise by various global actors;
- compliance with tax, employment, immigration and labor laws for employees living or traveling internationally;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the U.S.;
- potential liability under the FCPA or comparable foreign regulations;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the U.S.;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities internationally; and
- business interruptions resulting from geo-political actions, including war and terrorism.

In addition, some countries, such as Brazil, Israel and Chile, require that clinical trial participants receive the product at no cost even after the clinical trial has ended. We would not be able to recover any profit for these patients and depending on the number of patients, duration of the treatment and numerous other factors, such regulations could harm our business, prospects, financial condition and results of operations significantly. These and other risks associated with our international operations may materially adversely affect our ability to attain or maintain profitable operations.

We face significant competition from other biotechnology and pharmaceutical companies with products that may directly or indirectly compete with ours, and our operating results will suffer if we fail to compete effectively.

The biopharmaceutical industry is characterized by intense competition and rapid innovation. Our potential competitors include major multinational pharmaceutical companies, established biotechnology companies, specialty pharmaceutical companies, generic pharmaceutical companies and universities and other research institutions who are active in rare disease. Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations and well-established sales forces. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors may succeed in developing, acquiring or licensing, on an exclusive basis, drug products that are more effective or less costly than our product candidates, which may negatively affect our commercial opportunities. We believe the key competitive factors that will affect the development and commercial success of our product candidates are efficacy, safety and tolerability profile, reliability, convenience of dosing, price and reimbursement. There may also be competitors for our product candidates that we are unaware of at this point. Additionally, while our product candidates and approved medicines are generally protected for a defined period per jurisdiction by various patents, the loss of patent or regulatory exclusivity for our pharmaceutical products may open such products to competition from generic substitutes that are typically priced significantly lower than the original products. The introduction of generic versions of a pharmaceutical product frequently leads to a swift and substantial decline in the sales of the original product. Our continued innovation efforts cannot fully mitigate the impact of competition from generics.

We are aware of two other companies pursuing clinical development of therapies that reduce sBA levels via the IBAT pathway. GlaxoSmithKline plc (“GSK”) and Ipsen have IBATis in clinical development for cholestatic liver diseases.

We are aware Ipsen has received approval for odevixibat (Bylvay) for the treatment of pruritus in patients with PFIC 3 months of age and older, and cholestatic pruritus in patients with ALGS 12 months of age and older, in the U.S., and for the treatment of PFIC in patients 6 months of age and older in the EU and for odevixibat (Kayfanda) for the treatment of cholestatic pruritus in ALGS in patients 6 months of age and older. In the EU, Bylvay and Kayfanda are authorized under exceptional circumstances. Ipsen has opened enrollment in their ALGS open-label extension study to infants 11 months or younger and is also conducting a study of odevixibat in BA and plans to pursue other cholestatic liver diseases. GSK announced in November 2024 that the Phase 3 GLISTEN trial with linerixibat in PBC met its primary pruritus endpoint and that GSK has submitted a marketing applications to the U.S. and EU in the first half of 2025 with potential approvals expected in the first half of 2026.

Other off-label medications are also used in ALGS, PFIC, PSC and PBC for cholestatic pruritus such as Ursodeoxycholic acid (“UDCA”), cholestyramine and other bile salt resins, rifampin, naltrexone and other agents, such as selective serotonin reuptake inhibitors. Despite the lack of FDA approval, these older, generic agents are perceived as part of the standard of care for treating ALGS, PFIC, PSC and PBC patients suffering from cholestatic pruritus. Further, we may compete with companies that are developing gene therapy for the treatment of PFIC. Additionally, surgical interventions, such as partial external biliary diversion and nasobiliary drainage, and extracorporeal liver support, such as Molecular Adsorbent Recirculation System, are also employed in an attempt to lower bile acid levels, manage pruritus and improve measures of liver function.

In adult settings of cholestasis, similar to pediatric settings, cholestyramine, UDCA, rifampin and naltrexone are commonly used agents. We are aware that Alfasigma S.p.’s (formerly Intercept Pharmaceuticals, Inc.) Ocaliva, Gilead Science’s Livdelzi, and Ipsen’s Iqirvo are approved as a second-line treatment for PBC in patients with inadequate response to ursodeoxycholic acid. We are aware of several agents in clinical development for the treatment of PBC including Alfasigma’s Ocaliva and bezafibrate, Zydus Therapeutics Inc.’s saroglitazar magnesium, Calliditas Therapeutics AB’s setanaxib, COUR Pharmaceuticals’ CNP-104, Ascletis Pharma’s ASC42, Umecrine Cognition’s golexanolone, Kowa Company Ltd’s K-808, HighTide Therapeutics Inc.’s HTD-1801, Hepagene Therapeutics Inc’s HPG-1860, Tharimmune Inc’s TH-104, Cascade Pharmaceuticals Inc.’s CS-0159, and GSK’s linerixibat, another IBATi.

We are not aware of FDA or European Commission approved therapeutics for the treatment of PSC. We are aware of several agents in clinical development for the treatment of PSC, including Dr. Falk Pharma's Norucholic acid, HighTide Therapeutics Inc.'s HTD1801, Alfasigma's Ocaliva, or obeticholic acid, Ipsen's elafibranor and ritivixibat, NGM Biopharmaceuticals Inc.'s NGM282, Chemomab Therapeutics Ltd.'s CM-101, Cascade Pharmaceuticals Inc.'s CS-0159, and LISCure Biosciences Inc.'s LB-P8.

Symptomatic treatment with antipruritics, such as cholestyramine, typically provides only modest relief. Bristol Myers Squibb Company has discontinued its brand name cholestyramine, but generic versions of the drug are marketed by Upsher-Smith Laboratories, Inc., Par Pharmaceutical Companies, Inc., Sandoz Inc., the generic pharmaceuticals division of Novartis AG and others. UDCA, also known as ursodiol, is marketed by a number of generic pharmaceutical companies such as Mylan Inc., Actavis Inc., Lannett Company, Inc. and Par Pharmaceutical Companies, Inc.

There are other approved chenodeoxycholic acid products available outside of the U.S. Both Dr. Falk Pharma GmbH and Leadiant Biosciences, Inc. have FDA Orphan Drug Designations granted for the treatment of CTX (granted in 2004 and 2007, respectively).

There are currently no FDA-approved treatments in the U.S. that compete with Cholbam. There are other approved cholic acid products available outside of the U.S. and Laboratoires CTRS has received approval from the EMA for a version of cholic acid.

Under the Hatch-Waxman Amendments of the Federal Food, Drug, and Cosmetic Act, a pharmaceutical manufacturer may file an ANDA seeking approval of a generic copy of an approved innovator product or an NDA under Section 505(b)(2) that relies on the FDA's prior findings of safety and effectiveness in approving the innovator product. A Section 505(b)(2) NDA may be for a new or improved version of the original innovator product. Certain of our approved medicines, including Chenodal, Ctexli and Cholbam, are or may be subject to immediate competition from compounded and generic entrants, as the ANDA and NDA for these drug products have no remaining or current patent or non-patent exclusivity.

In December 2019, the CREATES Act was enacted, which provides a legislatively defined private right of action under which generic companies can bring suit against companies who refuse access to product for the bioequivalence testing needed to support approval of a generic product. It is our policy, which is in compliance with the CREATES Act, to evaluate requests for samples of our branded products, and to provide samples in response to bona fide requests from qualified third parties, including generic manufacturers, subject to specified conditions. We have provided samples to certain generic manufacturers.

We are not aware of FDA or European Commission approved therapeutics for the treatment of FXS. We are aware of one other company, Shionogi & Co., LTD., pursuing clinical development of a PDE4D inhibitor (zatomilast/BPN14770) in FXS. We are aware of several other companies pursuing clinical development of therapies for FXS including Harmony Biosciences Inc.'s ZYN002, Allos Pharma Inc.'s Arbaclofen, Healx Ltd.'s Gabaxodol, Spinogenix Inc.'s SPG601, Connecta Therapeutics S.L.'s CTH120, and Kaerus Therapeutics Inc.'s KER-0193.

Even though we have obtained orphan drug designation for several of our product candidates, we may not be able to obtain or maintain the benefits associated with orphan drug status, including market exclusivity.

Regulatory authorities in some jurisdictions, including the U.S. and the EU, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the U.S., or a patient population of greater than 200,000 individuals in the U.S., but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. In the EU, the European Commission, on the basis of the opinion of the EMA Committee for Orphan Medicinal Products ("COMP"), grants orphan drug designation for medicines to be developed for the diagnosis, prevention or treatment of diseases that are life-threatening or chronically debilitating, for which either no satisfactory method of diagnosis, prevention, or treatment exists, or if such method exists, the medicine is of significant benefit to those affected by such condition. To benefit from such designation, either the prevalence of such condition must not be more than five in 10,000 people across the EU or, if more prevalent, it must be unlikely that the marketing of the medicine would generate sufficient returns to justify the investment needed for its development. In September 2013, the FDA granted orphan drug status to Livmarli for the treatment of patients with PFIC and ALGS in the U.S. We also received orphan drug designation for Livmarli for PFIC and ALGS in the EU. In 2025, the FDA and COMP granted orphan drug designation for volixibat for PBC and PSC. The FDA granted orphan drug designation for chenodiol for the treatment of CTX in 2010. Generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug may be entitled to a period of marketing exclusivity, which precludes the FDA or the European Commission from approving another marketing application for the same drug (or, in the case of the European Commission, a similar drug) for

the same indication for that time period. The applicable period is seven years in the U.S. and ten years in the EU, which may be extended by six months and two years, respectively, in the case of product candidates that have complied with the respective regulatory authority's agreed upon pediatric investigation plan ("PIP"). There is, however, a legislative proposal pending in the EU that may modify the length of orphan market exclusivity, change the way in which market exclusivity is awarded to drugs with more than one approved orphan indication. The exclusivity period in the EU can be reduced to six years if at the end of the fifth year a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or European Commission determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. In addition, even after a drug is granted orphan exclusivity and approved, the FDA can subsequently approve another drug for the same condition before the expiration of the seven-year exclusivity period including the same active ingredient, if the FDA concludes 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. In the EU, the European Commission may approve another drug for the same indication despite its orphan exclusivity on the basis that it is not a similar medicinal product or if it is considered safer, more effective, or otherwise clinically superior. Conversely, the European Commission may deny marketing approval for a product candidate if it determines such product candidate is structurally similar to an approved product for the same indication. In addition, if an orphan designated product receives marketing approval for an indication broader than or different from what is designated, such product may not be entitled to orphan exclusivity. Even though the FDA has granted orphan drug designation to Livmarli for the treatment of PFIC and ALGS, and for volixibat for the treatment of PBC and PSC, our current orphan drug designations may not provide exclusivity for approval for Livmarli or volixibat for modified or different indications.

Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process in the U.S. or the EU. Also, regulatory approval for any product candidate may be withdrawn, and other product candidates may obtain approval before us and receive orphan drug exclusivity, which could block us from entering the market.

Even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the candidate from competition because different drugs can be approved for the same condition before the expiration of the orphan drug exclusivity period.

We have formed and may continue to form or seek strategic alliances or enter into additional licensing arrangements in the future, and we may not realize the benefits of such alliances or licensing arrangements.

We have formed and may continue to form or seek strategic alliances, create joint ventures or collaborations or enter into additional licensing arrangements with third parties that we believe will complement or augment our development and commercialization efforts with respect to our approved medicines, our product candidates and any future product candidates that we may develop. We also have commercial partnerships outside of North America as well as in major European markets.

Any of our existing relationships or any future relationships we enter into may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders or disrupt our management and business. In addition, we face significant competition in seeking appropriate strategic partners and the negotiation process is time-consuming and complex. Moreover, we may not be successful in our efforts to establish a strategic partnership or other alternative arrangements for volixibat because it may be deemed to be at too early of a stage of development for collaborative effort, and third parties may not view volixibat as having the requisite potential to demonstrate safety and efficacy. If we license products or businesses, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture. Following a strategic transaction or license, we may not achieve the revenues or specific net income that justifies such transaction. Any delays in entering into new strategic partnership agreements related to our product candidates could delay the development and commercialization of our product candidates in certain geographies for certain indications, which would harm our business prospects, financial condition and results of operations.

Our failure to successfully in-license, acquire, develop and market additional product candidates or approved medicines would impair our ability to grow our business.

Although a substantial amount of our efforts are focused on the clinical development, potential regulatory approval and commercialization of our approved medicines and product candidates, a key element of our long-term strategy is to in-license, acquire, develop, market and commercialize a portfolio of products to treat patients with rare diseases. Because we do not have the necessary internal research and development capabilities, unless we build such capabilities internally, we will be dependent upon pharmaceutical companies, academic scientists and other researchers to

sell or license products or technology to us. The success of this strategy depends partly upon our ability to identify and select promising biopharmaceutical product candidates and products, negotiate licensing or acquisition agreements with their current owners and finance these arrangements. The process of proposing, negotiating and implementing a license or acquisition of a product candidate or approved product is lengthy and complex. Other companies, including some with substantially greater financial, marketing, sales and other resources, may compete with us for the license or acquisition of product candidates and approved medicines. We have limited resources to identify and execute the acquisition or in-licensing of third-party products, businesses and technologies and integrate them into our current infrastructure. Moreover, we may devote resources to potential acquisitions or licensing opportunities that are never completed, or we may fail to realize the anticipated benefits of such efforts. We may not be able to acquire the rights to additional approved medicines or product candidates on terms that we find acceptable, or at all. Further, any product candidate that we acquire may require additional development efforts prior to commercial sale, including preclinical or clinical testing and approval by the FDA, the European Commission and other similar regulatory authorities. All product candidates are prone to risks of failure during biopharmaceutical product development, including the possibility that a product candidate will not be shown to be sufficiently safe and effective for approval by regulatory authorities. In addition, any approved medicines that we acquire may not have the market potential we believe, be manufactured or sold profitably, or achieve market acceptance.

We may fail to realize all of the anticipated benefits of our commercial and product candidate acquisitions or those benefits may take longer to realize than expected.

We believe that there are significant benefits that may be realized from the Bile Acid Portfolio Acquisition or any other product or product candidate acquisition. For example, the full benefits of the Bile Acid Portfolio Acquisition, including the anticipated financial contribution of newly acquired assets, may not be realized as expected, may be diminished due to competition or may not be achieved within the anticipated time frame, or at all. Failure to achieve the anticipated benefits of the Bile Acid Portfolio Acquisition or any other product or product candidate acquisition, could adversely affect our results of operations or cash flows, divert needed resources away from our current approved medicines and product candidates, decrease or delay any accretive effect of an acquisition and negatively impact the price of our common stock.

We may not be able to integrate the acquired assets successfully. We have hired additional employees as part of the transaction and may not successfully integrate or deploy them for commercialization of the acquired Bile Acid Medicines. We have transferred and entered into new contracts for a number of vendors that support the manufacture and distribution of the acquired assets and there are several services still being provided by Travere after the transition services agreement expired. If we fail to successfully integrate the acquired assets, our results of operations could be adversely affected. The integration process will continue to require significant time and resources, require significant attention from management and may disrupt the ordinary functioning of our business, and we may not be able to manage the process successfully, which could harm our business.

We are highly dependent on our key personnel, and if we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.

Our ability to compete in the highly competitive biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. The loss of the services of any of our executive officers or other key employees and our inability to find suitable replacements could potentially harm our business, prospects, financial condition or results of operations.

We conduct many of our operations at our facility in Foster City, California. This region serves as the headquarters to many other biopharmaceutical companies and many academic and research institutions. Competition for skilled personnel in our market is intense and may limit our ability to hire and retain highly qualified personnel on acceptable terms or at all.

To induce valuable employees to remain at our company, in addition to salary and cash incentives, we have provided stock awards that vest over time. The value to employees of stock awards that vest over time may be significantly affected by movements in our stock price that are beyond our control and may at any time be insufficient to counteract more lucrative offers from other companies. In addition, in response to competition, high inflation rates and labor shortages, we may need to adjust employee cash compensation, which would affect our operating costs and our margins, or equity compensation, which would affect our outstanding share count and cause dilution to existing stockholders. Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us on short notice. Although we have offer letters with our key employees, these offer letters provide for at-will employment, which means that any of our employees could leave our employment at any time, with or without notice. We do not maintain “key man” insurance policies on the lives of these individuals or the lives of any of our

other employees. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level, and senior managers as well as junior, mid-level, and senior scientific and medical personnel.

Many of the other biotechnology and pharmaceutical companies that we compete against for qualified personnel have greater financial and other resources, different risk profiles and a longer history in the industry than we do. They may also provide more diverse opportunities and better chances for career advancement. Some of these characteristics are more appealing to high quality candidates than what we can offer. If we are unable to continue to attract and retain high quality personnel, the rate and success at which we can discover, develop and commercialize product candidates will be limited.

We will need to grow the size of our organization, and we may experience difficulties in managing this growth.

As of September 30, 2025, we had 355 full-time employees. As our development and commercialization plans and strategies develop, we expect to need additional development, managerial, operational, financial, sales, marketing and other personnel. Future growth would impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, maintaining and motivating additional employees;
- managing our commercialization efforts while focusing on other areas of our business;
- managing our internal development efforts effectively, including the clinical and regulatory review process for our approved medicines and our product candidates, while complying with our contractual obligations to contractors and other third parties; and
- improving our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to commercialize our approved medicines, any then-approved product and product candidates depends, in part, on our ability to effectively manage any future growth, and our management may also have to divert a disproportionate amount of its attention away from day-to-day activities in order to devote a substantial amount of time to managing these growth activities. To date, we have used the services of outside vendors to perform tasks including clinical trial management, statistics and analysis, regulatory affairs, formulation development and other drug development functions. Our growth strategy may entail expanding our group of contractors or consultants to implement these tasks going forward. Because we rely on numerous consultants, effectively outsourcing many key functions of our business, we will need to be able to effectively manage these consultants to ensure that they successfully carry out their contractual obligations and meet expected deadlines. We may not be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all. Our growth strategy also includes transitioning certain outsourced functions of our business, such as our patient services hub, from third-party vendors to our employees. If we are unable to effectively manage our outsourced activities, if the quality or accuracy of the services provided by consultants is compromised for any reason, or if we are not able to successfully internalize certain functions of our business, our clinical trials may be extended, delayed or terminated, we may not successfully commercialize our approved medicines or obtain regulatory approval for our product candidates, and we may not otherwise advance our business. As we grow our organization to internalize operation of our patient services hub, we may experience adverse changes that we would not have experienced had our patient services hub remained with our third-party vendor, including the potential loss of patient data. If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to further develop and commercialize our approved medicines, any then-approved product and product candidates and, accordingly, may not achieve our research, development and commercialization goals.

Business disruptions could seriously harm our future revenue and financial condition and increase our costs and expenses.

Our operations, and those of our CROs and other contractors and consultants, could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man-made disasters or business interruptions, for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. We rely on third-party manufacturers to produce our approved medicines and product candidates. Our ability to obtain clinical supplies of these products could be disrupted if the operations of these suppliers are affected by a man-made or natural disaster or other business interruption. Our corporate headquarters is located in California near major earthquake faults and fire zones. The ultimate impact on us, our significant suppliers and our general infrastructure of being located near major earthquake faults and fire zones and being consolidated in certain geographical areas is unknown, but our operations and financial condition could suffer in the event of a major earthquake, fire or other natural disaster.

Our employees, independent contractors, principal investigators, CROs, consultants, strategic partners and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk that employees, independent contractors, principal investigators, CROs, consultants and vendors may engage in fraudulent or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violates: (1) the laws of the FDA, EU, individual EU Member States or comparable foreign regulatory authorities, including those laws that require the reporting of true, complete and accurate information to the FDA, EMA, the competent authorities of individual EU Member States or comparable foreign regulatory authorities; (2) manufacturing standards; (3) healthcare fraud and abuse laws in the U.S. and similar foreign fraudulent misconduct laws; or (4) laws that require the true, complete and accurate reporting of our financial information or data. These laws may impact, among other things, our current activities with principal investigators and research subjects, as well as proposed and future sales, marketing and education programs. In particular, the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, structuring and commission(s), certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials. If we obtain regulatory approval for any of our product candidates and begin commercializing those products in the U.S. or other foreign jurisdictions, our potential exposure under such laws will increase significantly, and our costs associated with compliance with such laws are also likely to increase. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, damages, disgorgement, monetary fines, imprisonment, possible exclusion from participation in the U.S. in Medicare, Medicaid and other federal healthcare programs and in equivalent foreign programs, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations.

Our relationships with customers, physicians and third-party payors may be subject, directly or indirectly, to federal, state and equivalent foreign healthcare fraud and abuse laws, false claims laws, transparency laws, health information privacy and security laws, monopoly and anti-trust laws, and other healthcare laws and regulations. If we or our employees, independent contractors, consultants, commercial partners or vendors violate these laws, we could face substantial penalties.

These laws may impact, among other things, our clinical research program, as well as sales, marketing and education programs. In particular, the promotion, sales and marketing of healthcare items and services is subject to extensive laws and regulations designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive and other business arrangements. We may also be subject to federal, state and foreign laws governing the privacy and security of identifiable patient information. The U.S. healthcare laws and regulations that may affect our ability to operate include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, any person or entity from knowingly and willfully, offering, paying, soliciting or receiving any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, the purchasing, leasing, ordering or arranging for the purchase, lease, or order of any item or service reimbursable under Medicare, Medicaid or other federal healthcare programs. The term “remuneration” has been broadly interpreted to include anything of value. Although there are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, the exceptions and safe harbors are drawn narrowly. Practices that may be alleged to be intended to induce prescribing, purchases or recommendations, include the provision of in-kind services, genetic testing services, or products or any payments of more than fair market value, and may be subject to scrutiny if they do not qualify for an exception or safe harbor. In addition, a person or entity does not need to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal civil False Claims Act and the civil monetary penalties statute;
- federal civil and criminal false claims laws, including the federal civil False Claims Act, and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or

causing to be presented, claims for payment or approval from Medicare, Medicaid, or other federal government programs that are false or fraudulent or knowingly making a false statement to improperly avoid, decrease or conceal an obligation to pay money to the federal government, including federal healthcare programs;

- the Health Insurance Portability and Accountability Act ("HIPAA"), which created new federal civil and criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, including private third-party payors and knowingly and willfully falsifying, concealing or covering up by any trick, scheme or device, a material fact or making any materially false, fictitious or fraudulent statements in connection with the delivery of, or payment for, healthcare benefits, items or services. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), and their respective implementing regulations, which impose requirements on certain healthcare providers, health plans, and healthcare clearinghouses, known as covered entities and their respective business associates that perform services for them as well as their covered subcontractors that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information;
- federal civil and criminal anti-trust or anti-monopoly laws that restrict or limit corporate actions and practices in order to regulate the conduct and organization of businesses in order to promote competition may apply to exclusive contractual relationships between manufacturers, distributors, and specialty pharmacies which dispense the manufactured products; and
- the federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to CMS information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other healthcare professionals (such as physician assistants and nurse practitioners) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members.

We may also be subject to state and foreign equivalents of each of the healthcare laws described above, among others, some of which may be broader in scope. For example, we may be subject to the following: state and foreign anti-kickback and false claims laws that may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third party payors, including private insurers, or that apply regardless of payor; state and foreign laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government; state and foreign laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers, marketing expenditures, or drug pricing; state and local laws requiring the registration of pharmaceutical sales representatives; and state and foreign laws, such as the EU's and the United Kingdom's General Data Protection Regulations (respectively, the "EU GDPR" and "UK GDPR", together, the "GDPR") governing the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Additionally, we may be subject to federal and comparable foreign consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available, it is possible that some of our business activities, or our arrangements with physicians, could be subject to challenge under one or more of such laws. If we or our employees, independent contractors, consultants, commercial partners and vendors violate these laws, we may be subject to investigations, enforcement actions and/or significant penalties. We have adopted a code of conduct and healthcare compliance policies, but it is not always possible to identify and deter employee misconduct or business noncompliance, and the precautions we take to detect and prevent inappropriate conduct may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. Efforts to ensure that our business arrangements will comply with applicable healthcare laws may involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other

healthcare laws and regulations. If any such actions are instituted against us, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, damages, disgorgement, monetary fines, imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs or comparable foreign programs, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations. In addition, the approval and commercialization of any of our product candidates outside the U.S. will also likely subject us to foreign equivalents of the healthcare laws mentioned above, among other foreign laws.

Our business is subject to complex, stringent and evolving U.S. and foreign laws, regulations, and rules, contractual obligations, industry standards, policies and other obligations relating to privacy and data protection. Our actual or perceived failure to comply with such obligations could result in regulatory investigations or actions, litigations (including class claims) and mass arbitration demands, fines and penalties, disruptions of and changes to our business practices, monetary penalties, reputational harm, loss of revenue or profits, and other adverse business consequences.

In the ordinary course of business, we collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, "process") personal data and other sensitive information, including proprietary and confidential business data, trade secrets, intellectual property, data we collect about trial participants in connection with clinical trials, and sensitive third-party data.

Our data processing activities subject us to numerous data privacy and security obligations, such as various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contracts, and other obligations that govern the processing of personal data by us and on our behalf. In the U.S., federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal data privacy laws, consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), and other similar laws (e.g., wiretapping laws). For example, HIPAA, as amended by HITECH, imposes specific requirements relating to the privacy, security, and transmission of individually identifiable health information. Additionally, the Controlling the Assault of Non-Solicited Pornography and Marketing Act of 2003 ("CAN-SPAM") and Telephone Consumer Protection Act ("TCPA") imposes specific requirements on communications with customers. In particular, the TCPA imposes various consumer consent requirements and other restrictions relating to marketing to individuals using technology such as telephones, mobile devices, and text messages. TCPA violations can result in significant financial penalties, including penalties or criminal fines imposed by the Federal Communications Commission or fines of up to $1,500 per violation imposed through private litigation or by state authorities.

Numerous U.S. states have enacted comprehensive privacy laws that impose certain obligations on covered businesses, including providing specific disclosures in privacy notices and affording residents with certain rights concerning their personal data. As applicable, such rights may include the right to access, correct, or delete certain personal data, and to opt-out of certain data processing activities, such as targeted advertising, profiling, and automated decision-making. The exercise of these rights may impact our business and ability to provide our products and services. Certain states also impose stricter requirements for processing certain personal data, including sensitive information, such as conducting data privacy impact assessments. These state laws allow for statutory fines for noncompliance. For example, at the state level, the California Consumer Privacy Act, as amended, ("CCPA"), applies to personal data of California consumers, business representatives and employees, and requires businesses to provide specific disclosures in privacy notices and honor requests of such individuals to exercise certain privacy rights. The CCPA provides for fines for violations and allows private litigants affected by certain data breaches to recover significant statutory damages. Although the CCPA and other comprehensive state privacy laws exempt some data processed in the context of clinical trials, these laws may increase compliance costs and potential liability for us and the third parties with whom we work. Similar laws are being considered in several other states, as well as at the federal and local levels, and we expect more states to pass similar laws in the future.

Outside the U.S., an increasing number of laws, regulations, and industry standards apply to data privacy and security. For example, the EU GDPR and the UK GDPR (together "GDPR"), Brazil's General Data Protection Law (Lei Geral de Proteção de Dados Pessoais, or "LGPD") (Law No. 13,709/2018), Canada's Personal Information Protection and Electronic Document Act ("PIPEDA") and China's Personal Information Protection Law ("PIPL") impose strict requirements for processing personal data, and violators of these laws may face significant penalties.

For example, the GDPR imposes stringent requirements for controllers and processors of personal data, including, for example, more robust disclosures to individuals and a strengthened individual data rights regime, mandatory data breach notifications in certain circumstances, limitations on retention of information, increased requirements pertaining to special categories of data, such as health data, and additional obligations when we contract with third-party

processors in connection with the processing of personal data. In addition, the definition of "personal data" under the GDPR is broad and captures "pseudonymized" or key-coded data that is commonly processed in a clinical trial-related context.

We are subject to the GDPR because of our data processing activities that involve the personal data of individuals residing in the EEA and UK, such as in connection with our clinical trials in Europe, and early access program in multiple EU countries, and because of certain processing of personal data carried out in the context of the activities of our relevant European subsidiaries. In addition, we maintain an office in Switzerland, which subjects us to privacy and data protection laws and regulations similar to the GDPR under the Swiss Federal Act on Data Protection, or the FADP. The FADP applies to the collection and processing of personal data, including health-related information, by companies located in Switzerland, or in certain circumstances, by companies located outside of Switzerland.

Furthermore, the EU GDPR provides that EEA Member States may introduce specific requirements related to the processing of "special categories of personal data", including the personal data related to health and genetic information, which we may process in connection with clinical trials or otherwise; as well as personal data related to criminal offenses or convictions where allowed under local law and confirmed by potential employee in employment situations. Under the GDPR, companies that do not comply may face temporary or definitive bans on data processing and other corrective actions; fines of up to 20 million Euros under the EU GDPR or 17.5 million pounds sterling under the UK GDPR, or in each case, 4% of annual global revenue, whichever is greater; or private litigation related to processing of personal data brought by classes of data subjects or consumer protection organizations authorized at law to represent their interests.

Additionally, in Canada, the Personal Information Protection and Electronic Documents Act ("PIPEDA") and various related provincial laws, as well as Canada's Anti-Spam Legislation ("CASL"), may apply to our operations.

In the ordinary course of business, we transfer personal data from Europe (including from our European subsidiaries) and other jurisdictions to the U.S. or other countries. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal data to other countries. In particular, the EEA and the UK have significantly restricted the transfer of personal data to the U.S. and other countries whose privacy laws it generally believes are inadequate. Other jurisdictions may adopt or have already adopted similarly stringent data localization and/or cross-border data transfer laws. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and UK to the U.S. in compliance with law, such as the EEA standard contractual clauses, the UK's International Data Transfer Agreement / Addendum, and the EU-U.S. Data Privacy Framework and the UK extension thereto (which allows for transfers to relevant U.S.-based organizations who self-certify compliance and participate in the Framework), these mechanisms are subject to legal challenges, and there is no assurance that we can satisfy or rely on these measures to lawfully transfer personal data to the U.S. If there is no lawful manner for us to transfer personal data from the EEA, the UK, or other jurisdictions, to the U.S., or if the requirements for a legally-compliant transfer are too onerous, we could face significant adverse consequences, including limitations of our ability to conduct clinical trial activities in Europe and/or elsewhere, the interruption or degradation of our operations, the need to relocate part of or all of our business or data processing activities to other jurisdictions (such as Europe) at significant expense, increased exposure to regulatory actions, substantial fines and penalties, the inability to transfer personal data and work with partners, vendors and other third parties, and/or injunctions against our processing or transferring of personal data necessary to operate our business. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers of personal data to recipients outside Europe for allegedly violating the EU GDPR's cross-border data transfer limitations. For example, in May 2023, the Irish Data Protection Commission determined that a major social media company's use of the standard contractual clauses to transfer personal data from Europe to the U. S. was insufficient and levied a 1.2 billion Euro fine against the company and prohibited the company from transferring personal data to the U.S. Additionally, companies that transfer personal data to recipients outside of the EEA and/or UK to other jurisdictions, particularly to the U.S., are subject to increased scrutiny from regulators, individual litigants and activist groups. Regulators in the U.S. such as the Department of Justice are also increasingly scrutinizing certain personal data transfers and have proposed and may enact certain data export restrictions and localization requirements, for example, the Preventing Access to U.S. Sensitive Personal Data and Government-Related Data by Countries of Concern or Covered Persons rule finalized by the Department of Justice in late 2024, enacting the Biden administration's executive order Preventing Access to Americans' Bulk Sensitive Personal Data and United States Government-Related Data by Countries of Concern, which went into effect April 8, 2025.

In addition, we are also bound by contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. Additionally, some of our customer contracts require us to host personal data locally. We also publish privacy policies, marketing materials and other statements, such as statements related to compliance with certain certifications or self-regulatory principles, concerning artificial intelligence, data privacy and security. Regulators in the United States may scrutinize these statements, and if these policies, materials or statements

are found to be deficient, lacking in transparency, deceptive, unfair, misleading or misrepresentative of our practices, we may be subject to adverse consequences.

Obligations related to data privacy and security (and consumers' data privacy expectations) are quickly changing, becoming increasingly stringent, and creating uncertainty. Additionally, these obligations may be subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions. Preparing for and complying with these obligations requires significant resources and may necessitate changes to our information technologies, systems, and practices and to those of any third parties that process personal data on our behalf.

Although we endeavor to comply with our data privacy and security obligations, we may at times fail (or be perceived to have failed) to do so. Moreover, despite our efforts, our personnel or third parties with whom we work may fail to comply with such obligations, which could negatively impact our business operations and compliance posture.

Our, or the third parties with whom we work, actual or perceived failure to adequately comply with applicable laws and regulations relating to privacy and data protection, or to protect personal data and other data we process or maintain, could result in adverse consequences, including regulatory fines and bans on processing personal data, government enforcement actions (e.g., investigations, fines, penalties, audits, inspections, and similar), litigation (including class action claims), mass arbitration demands, and other liabilities, claims for damages by affected individuals, orders to destroy or not use personal data, imprisonment of company officials, additional reporting requirements and/or oversight, interruptions or stoppages in our business operations (including, as relevant, clinical trials), and damage to our reputation. Any of these consequences could have a material adverse effect on our business, financial condition, results of operations and growth prospects, including but not limited to: loss of customers; interruptions or stoppages in our business operations (including, clinical trials); inability to process personal data or to operate in certain jurisdictions; limited ability to develop or commercialize our approved medicines; expenditure of time and resources to defend any claim or inquiry; adverse publicity; or substantial changes to our business model or operations. In particular, plaintiffs have become increasingly more active in bringing privacy-related claims against companies, including class claims and mass arbitration demands. Some of these claims allow for the recovery of statutory damages on a per violation basis, and, if viable, carry the potential for monumental statutory damages, depending on the volume of data and the number of violations.

Any collaboration arrangements that we have or may enter into in the future may not be successful or may result in product diversion, which could adversely affect our ability to develop and commercialize our approved medicines and any then-approved product.

Any existing or future collaborations that we enter into may not be successful. The success of our collaboration arrangements depends and will depend heavily on the efforts and activities of our collaborators. Collaborations are subject to numerous risks, which may include that:

- collaborators have significant discretion in determining the efforts and resources that they will apply to collaborations;
- collaborators may conduct their own clinical trials which may not be compliant, may not be successful or may generate contradictory results;
- collaborators may not pursue development and commercialization of our approved medicines and any then-approved product or may elect not to continue or renew development or commercialization programs based on trial or test results, changes in their strategic focus due to the acquisition of competitive products, availability of funding or other external factors, such as a business combination that diverts resources or creates competing priorities;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product or product candidates;
- a collaborator with marketing, manufacturing and distribution rights to one or more products may not commit sufficient resources to or otherwise not perform satisfactorily in carrying out these activities;
- we could grant exclusive rights to our collaborators that would prevent us from collaborating with others;
- 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;
- a collaborator or series of collaborators may improperly or unknowingly sell product directly (or indirectly to a potential customer) into the "gray market" whereby our branded products are diverted from authorized

sales channels into the hands of dealers, brokers or the open market, and may result in unauthorized sale of our product in a specific country or region;

- disputes may arise between us and a collaborator that causes the delay or termination of the research, development or commercialization of our current or future 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 current or future products;
- collaborators may own or co-own intellectual property covering our approved medicines and any then-approved product that results from our collaborating with them, and in such cases, we would not have the exclusive right to develop or commercialize such intellectual property; and
- a collaborator's sales and marketing activities or other operations may not be in compliance with applicable laws resulting in civil or criminal proceedings.

Risks Related to Our Reliance on Third Parties

We depend on intellectual property licensed from third parties and termination of any of these licenses could result in the loss of significant rights, which would harm our business.

We are dependent on patents, know-how and proprietary technology, both our own and licensed from others. For example, we entered into an assignment and license agreement with Shire pursuant to which we were assigned exclusive global rights to license intellectual property and know-how related to Livmarli and volixibat, rights to license know-how related to Livmarli from Pfizer, certain patents and know-how related to volixibat from Sanofi and certain patents and know-how related to Livmarli and volixibat from Satiogen, which we subsequently acquired in May 2022. We also acquired licensed rights to commercialize Cholbam and chenodiol from certain parties via the Bile Acid Portfolio Acquisition. We are required to use commercially reasonable efforts or diligent efforts to commercialize products based on the licensed rights and to pay certain royalties based off our net sales. We may not meet these requirements, which could result in a loss or termination of any rights under such agreements. Any termination of these licenses will result in the loss of significant rights and will restrict our ability to commercialize our product candidates.

We are generally also subject to all of the same risks with respect to protection of intellectual property that we license, as we are for intellectual property that we own, which are described below under "Risks Related to Our Intellectual Property." If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize products could suffer.

We rely on third parties to conduct our clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize our product candidates.

We currently rely on, and intend to continue relying on, third-party CROs in connection with our clinical trials. We control or will control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our clinical trials is conducted in accordance with applicable protocol, legal, regulatory and scientific standards, and our reliance on our CROs does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with GCPs, which are regulations and guidelines enforced by the FDA, the competent authorities of the individual EU Member States, or comparable foreign regulatory authorities for product candidates in clinical development. Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of these CROs fail to comply with applicable GCP regulations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, EMA, the competent authorities of the individual EU Member States, or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. Upon inspection, such regulatory authorities may not determine that any of our clinical trials comply with the GCP regulations. In addition, our clinical trials must be conducted with drug product produced under cGMP regulations and will require a large number of test subjects. Our failure or any failure by our CROs to comply with these regulations or to recruit a sufficient number of patients may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, our business may be implicated if any of our CROs violates federal, state or foreign fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

Our CROs are not our employees and, except for requirements and remedies available to us under our agreements with such CROs, we have limited control whether or not they devote sufficient time and resources to our ongoing preclinical, clinical and non-clinical programs. These CROs may also have relationships with other commercial

entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities, which could affect their performance on our behalf. If our CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to complete development of, obtain regulatory approval for or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed.

Switching or adding CROs involves substantial cost and requires extensive management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur when CROs are switched or added, which can materially impact our ability to meet our desired clinical development timelines. Although we carefully manage our relationships with our CROs, we may encounter challenges or delays in the future and these delays or challenges may have a material adverse impact on our business, prospects, financial condition and results of operations.

We rely completely on third parties to manufacture and distribute our clinical and commercial drug supplies, including certain sole-source suppliers and manufacturers. These third parties may fail to obtain and maintain regulatory approval for their facilities, fail to provide us with sufficient quantities of drug substance, drug product, or labeled finished product in a timely fashion, or fail to do so at acceptable quality levels or prices.

We do not currently have, nor do we plan to acquire, the infrastructure or capability internally to manufacture or distribute our clinical and commercial drug supplies. Instead, we rely on contracted third parties.

We do not currently have any long-term agreements or commitment with a manufacturer to produce raw materials, APIs and the finished products of our product candidates or the associated packaging. We will need to continue to identify and qualify a third-party manufacturer prior to commercialization of our product candidates, and we intend to enter into agreements for commercial production with third-party suppliers. For our approved medicines, we are reliant on third parties to successfully manufacture drug substance components and the finished drug product in accordance with regulatory requirements and in sufficient quantities for commercialization. We are also reliant on third parties for the manufacture of packaging, labeling and oral dosing dispensers for our approved medicines. As our approved medicines are intended to treat rare diseases, we will only require a low-volume of raw materials and APIs, and in some cases with single-source suppliers and manufacturers. Our reliance on third-party suppliers and manufacturers, including single-source suppliers, could harm our ability to develop our product candidates or to commercialize our currently approved medicines and any product candidates that are approved in the future.

Any of our existing or future suppliers or manufacturers may, among other things:

- fail to supply us with our approved medicines and product candidates on a timely basis or in the requested amount due to unexpected damage to or destruction of facilities, equipment or deliveries, labor disputes or otherwise, including “acts of God”;
- fail to increase manufacturing capacity and produce drug product and components in larger quantities and at higher yields in a timely or cost-effective manner, or at all, to sufficiently meet our clinical and commercial needs;
- be unable to meet our production demands, including due to issues related to their reliance on sole-source suppliers and manufacturers;
- become unavailable through business interruption or financial insolvency; or
- be unable or unwilling to supply or manufacture for us, or to renew current supply or manufacturing agreements when such agreements expire on a timely basis, on acceptable terms or at all.

In the event of any of the foregoing or in the event such third parties fail to meet our needs, if we do not have an alternative supplier or manufacturer in place, we would be required to expend substantial management time and expense to identify, qualify and transfer processes to alternative suppliers or manufacturers. Transferring technology to other sites may require additional processes, technologies and validation studies, which are costly, may take considerable amounts of time, may not be successful and, in most cases, require review and approval by the FDA, the competent authorities of the individual EU Member States or comparable foreign regulatory authorities. Any need to find and qualify new suppliers or manufacturers could adversely impact our ability to commercialize our approved medicines or our product candidates, if approved. Additionally, we and our manufacturers do not currently maintain significant inventory of drug substances and other materials. Any delay or interruption in the supply of clinical trial supplies could delay the completion of our clinical

trials, increase the costs associated with maintaining clinical trial programs and, depending upon the period of delay, require us to commence new clinical trials at additional expense or terminate clinical trials completely.

Although we are ultimately responsible for ensuring compliance with regulatory requirements such as cGMPs, we are dependent on our contract suppliers and manufacturers for day-to-day compliance with cGMP for production of both drug substances and finished products. Facilities used by our contract suppliers and manufacturers to produce the drug substances and materials or finished products for commercial sale must pass inspection and be approved by the FDA and other relevant regulatory authorities including the competent authorities of the individual EU Member States. A number of our contract suppliers and manufacturers must comply with cGMP requirements enforced by the FDA, the competent authorities of the individual EU Member States, and other equivalent foreign authorities, through their facilities inspection program and review of submitted technical information. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the FDA's, the competent authorities of the individual EU Member States, and other equivalent foreign authorities' strict regulatory requirements, they will not be able to secure or maintain FDA approval for the manufacturing facilities and our ability to secure supplies of our approved medicines or our product candidates will be negatively affected.

In addition, we have limited control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the safety of our approved medicines is compromised due to a failure to adhere to applicable laws or for other reasons, the manufacturing facilities may need to be closed for an extended period of time and we may need to find alternative manufacturing facilities, in which case we might not be able to identify manufacturers for clinical or commercial supply on acceptable terms, or at all, which would significantly impact our ability to develop, obtain regulatory approval for or market our approved medicines.

We and our third-party suppliers and manufacturers are vulnerable to geopolitical and macroeconomic developments, such as potential future bank failures, tariffs and trade tensions, the ongoing shutdown of the federal government and the resulting effects on its regulatory agencies, geopolitical tensions, the ongoing conflicts between Ukraine and Russia and in the Middle East, and increasing tensions between the U.S. and China, as well as any related political or economic responses and counter-responses or otherwise by various global actors or the general effect on the global economy and supply chain, future pandemics, high inflation rates and the responses by central banking authorities to control such inflation, which could negatively impact the availability or cost of materials and the third parties on which we rely. Similarly, the manufacturing facilities of a majority of our suppliers are located outside of the U.S. This may give rise to difficulties in importing our product into the U.S. or other countries as a result of, among other things, regulatory agency approval requirements, taxes, tariffs, local import requirements such as import duties or inspections, incomplete or inaccurate import documentation, defective packaging or negative impacts on global shipping due to geopolitical and macroeconomic developments. If such events result in any interruption in the supply of a drug substance or other material or in the manufacture of our approved medicines, such interruption could have a material adverse effect on our business, financial condition, operating results and prospects.

We rely on a specialty pharmacy for all of our sales of our approved medicines in each of the U.S. and Canada and use 3PLs, authorized distributors and licensed partners outside of the U.S. Switching or adding a specialty pharmacy or distributor involves substantial cost and requires extensive management time and focus. In addition, there is a natural transition period when a new specialty pharmacy or distributor commences work. If either the specialty pharmacy or the distributor becomes subject to bankruptcy or is acquired by a company that wants to terminate the relationship with us, and we are required to transition to a new specialty pharmacy or distributor, such transition may result in an inability for us to collect outstanding receivables and a decline in our revenue, results of operations and cash flows.

International trade policies, including tariffs, sanctions and trade barriers may adversely affect our business, financial condition, results of operations and prospects.

We operate in a global economy, and our business depends on a global supply chain for the development, manufacturing, and distribution of our pharmaceutical products, and for the advancement of our preclinical and clinical development programs. There is inherent risk, based on the complex relationships among the U.S. and the countries in which we conduct our business, that political, diplomatic, and national security factors can lead to global trade restrictions and changes in trade policies and export regulations that may adversely affect our business and operations. The current international trade and regulatory environment is subject to significant ongoing uncertainty.

Tariff policies, particularly those affecting China and pharmaceutical products, could increase our costs and reduce our profitability, including as a result of our inability to adjust pricing in formulary-based markets. Recent and potential future changes in international trade policies, particularly regarding U.S.-China trade relations and pharmaceutical-specific tariffs, present risks to our operations and financial performance.

The ongoing trade tensions between the U.S. and other jurisdictions have resulted in multiple rounds of tariffs and anticipated tariffs affecting pharmaceuticals and pharmaceutical ingredients, including finished products, manufacturing equipment, and related supplies. Tariffs on these inputs may increase our manufacturing costs for certain products. Moreover, the dynamic and unpredictable tariff and trade landscape creates uncertainty and planning challenges for our operations. Changes in tariff classifications, country-of-origin requirements, or customs procedures can occur with limited notice. Further, the Bureau of Industry and Security, U.S. Department of Commerce, has initiated an investigation to determine whether pharmaceutical ingredients, including finished drug product, manufactured outside the U.S. pose a national security risk and should be subject to additional tariffs. Unlike consumer goods, pharmaceuticals face unique regulatory constraints that make rapid supply chain adjustments particularly difficult and costly. This uncertainty complicates our long-term investment decisions regarding manufacturing facilities, supply chain optimization, and research and development locations.

Unlike many industries, our ability to pass increased costs to customers is limited by the structure of pharmaceutical pricing and reimbursement systems. Many of our products are included in formularies with pricing established through annual or multi-year contracts with commercial, third-party payors and pharmacy benefit managers, and reimbursement methodologies established by government programs, such as Medicare. These arrangements typically include fixed pricing terms that were negotiated prior to the implementation of the recently announced tariffs. As a result, and depending on the timing and scope of the implementation of these tariffs, cost increases due to tariffs may be difficult or impossible to pass through to customers until the next negotiation cycle, which could be several months or years away.

Current or future tariffs may also result in increased research and development expenses, including with respect to increased costs associated with APIs and raw materials. Trade restrictions affecting the import of materials necessary for clinical trials could result in delays to our development timelines. Increased development costs and extended development timelines could place us at a competitive disadvantage compared to companies operating in regions with more favorable trade relationships and could reduce investor confidence and negatively impact our business, results of operations, financial condition and growth prospects.

The complexity of announced or future tariffs may also increase the risk that we or our customers or suppliers may be subject to civil or criminal enforcement actions in the U.S. or foreign jurisdictions related to compliance with trade regulations. Foreign governments may also adopt non-tariff measures, such as procurement preferences or informal disincentives to engage with, purchase from or invest in U.S. entities, which may limit our ability to compete internationally and attract non-U.S. investment, employees, customers and suppliers. Foreign governments may also take other retaliatory actions against U.S. entities, such as decreased intellectual property protection, increased enforcement actions, or delays in regulatory approvals, which may result in heightened international legal and operational risks. In addition, the U.S. and other governments have imposed and may continue to impose additional sanctions, such as trade restrictions or trade barriers, which could restrict us from doing business directly or indirectly in or with certain countries or parties and may impose additional costs and complexity to our business.

Trade disputes, tariffs, restrictions and other political tensions between the U.S. and other countries may also exacerbate unfavorable macroeconomic conditions including inflationary pressures, foreign exchange volatility, financial market instability, and economic recessions or downturns. The ultimate impact of current or future tariffs and trade restrictions remains uncertain and could materially and adversely affect our business, financial condition, and prospects. While we actively monitor these risks, any prolonged economic downturn, escalation in trade tensions, or deterioration in international perception of U.S.-based companies could materially and adversely affect our business, ability to access the capital markets or other financing sources, results of operations, financial condition and prospects. In addition, tariffs and other trade developments have and may continue to heighten the risks related to the other risk factors described elsewhere in this report and in our Annual Report.

Risks Related to Our Financial Position and Capital Requirements

We may need substantial additional financing to continue our commercialization efforts for our approved medicines, develop our product candidates and implement our operating plans. If we fail to obtain additional financing when needed, we may be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

Our operations have consumed substantial amounts of cash since inception. We expect to continue to spend substantial amounts to continue the clinical development and seek regulatory approval of our product candidates. We will require significant additional amounts in order to continue our marketing and sales efforts for our approved medicines, prepare for commercialization for our product candidates, and, if approved, to launch and commercialize our product candidates.

Based on our current and anticipated level of operations, we believe our existing unrestricted cash, cash equivalents and investments will be sufficient to fund current operations through at least the next 12 months. However, changing circumstances may cause us to consume capital significantly faster than we currently anticipate, and we may need to spend more money than currently expected because of circumstances beyond our control. We may require additional capital for the further development and commercialization of our product candidates and may need to raise additional funds sooner if we choose to expand more rapidly than we presently anticipate.

Additional funding may not be available on acceptable terms, or at all. As a result of adverse geopolitical and macroeconomic developments, such as potential future disruptions in access to bank deposits or lending commitments due to bank failures, tariffs and trade tensions, the ongoing shutdown of the federal government and the resulting effects on its regulatory agencies, geopolitical tensions, the ongoing conflicts between Ukraine and Russia and in the Middle East, and increasing tensions between the U.S. and China, as well as any related political or economic responses and counter-responses or otherwise by various global actors or the general effect on the global economy and supply chain, actual and anticipated changes in interest rates, economic inflation and the responses by central banking authorities to control such inflation, the global credit and financial markets have experienced extreme volatility and disruptions, including severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates and uncertainty about economic stability. If the equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly and more dilutive. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of our approved medicines and product candidates or other research and development initiatives. We also could be required to seek collaborators for our product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to our product candidates in markets where we otherwise would seek to pursue development or commercialization ourselves.

Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

We may seek additional capital through a combination of public and private equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. For example, in April 2023, we issued and sold $316.3 million aggregate principal amount of the Notes. Further, in August 2023, in connection with and immediately prior to the closing of the Bile Acid Portfolio Acquisition, we completed a private placement of our common stock, pursuant to which we issued 8,000,000 shares of our common stock. Additionally, in November 2023, we entered into a Sales Agreement (the "2023 Sales Agreement") with Leerink Partners LLC and Cantor Fitzgerald & Co., pursuant to which we may elect to issue and sell, from time to time, shares of common stock having an aggregate offering price of up to $200.0 million. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our stockholders as such. For example, pursuant to the terms of the Notes and the related indenture (the "Indenture"), between us and U.S. Bank Trust Company, National Association, as trustee, our Notes are subject to conversion at the election of the holders for the quarterly period ending December 31, 2025, and if such an election is made and we elect to settle such conversion obligation under the Notes in shares of our common stock or a combination of cash and shares of our common stock as we have done in the past, the issuance of such common stock would dilute the ownership interests of our stockholders and sales in the public market could adversely affect prevailing market prices. The incurrence of indebtedness would result in increased fixed payment obligations and could involve certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or product candidates, or grant licenses on terms unfavorable to us.

If we raise additional funds through collaboration, strategic partnerships and licensing arrangements with third parties, we may have to relinquish valuable rights to our approved medicines and product candidates, our intellectual property, future revenue streams or grant licenses on terms that are not favorable to us. If our cash flows and capital resources are insufficient to allow us to make required payments, we may have to reduce or delay capital expenditures, sell assets or seek additional capital.

We may be unable to raise the funds necessary to repurchase the Notes for cash following a fundamental change, or to pay any cash amounts due upon conversion, and any future indebtedness may limit our ability to repurchase the Notes or pay cash upon their conversion.

Holders of the Notes may, subject to a limited exception described in certain provisions in the Notes and the related Indenture require us to repurchase the Notes following a fundamental change at a cash repurchase price generally equal to the principal amount of the Notes to be repurchased, plus accrued and unpaid interest, if any. In addition, upon conversion, we will satisfy part or all of our conversion obligation in cash unless we elect to settle conversions solely in shares of our common stock. We may not have enough available cash or be able to obtain financing at the time we are required to repurchase the Notes or pay any cash amounts due upon conversion. In addition, applicable law, regulatory authorities and agreements governing any future indebtedness may restrict our ability to repurchase the Notes or pay any cash amounts due upon conversion. Our failure to repurchase the Notes or to pay any cash amounts due upon conversion when required will constitute a default under the Indenture. A default under the Indenture or the fundamental change itself could also lead to a default under agreements governing any future indebtedness, which may result in any future indebtedness becoming immediately payable in full. We may not have sufficient funds to satisfy all amounts due under any future indebtedness and the Notes.

Our ability to utilize our net operating loss carryforwards and certain other tax attributes may be limited.

We have incurred substantial losses during our history and do not expect to become profitable in the near future, and we may never achieve profitability. As of December 31, 2024, we had federal and California and other state net operating loss ("NOL") carryforwards of approximately $156.1 million, $32.1 million and $62.1 million, respectively. The federal NOL carryforwards do not expire, and the California and other state NOL carryforwards will begin to expire in 2038 and 2032, respectively, unless previously utilized. Our ability to utilize our NOL carryforwards and certain other tax attributes may be limited. As of December 31, 2024, we also had federal and state research and development credit carryforwards totaling $44.8 million and $6.4 million, respectively. The federal research and development credit carryforwards will begin to expire in 2039, unless previously utilized. The state research and development credits do not expire.

Under the current U.S. federal income tax law, federal NOLs generated in taxable years beginning after December 31, 2017 may be carried forward indefinitely, but the deductibility of such federal NOLs in a taxable year is limited to 80% of taxable income in such year. Similar rules may apply under state tax laws. Our NOL carryforwards and other applicable tax attributes are subject to review and possible adjustment by the U.S. Internal Revenue Service and state tax authorities and may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant stockholders over a three-year period in excess of 50 percentage points (by value), as defined under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended. It is possible that we have experienced one or more such ownership changes in the past, and we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership. We may therefore be limited in the portion of NOL carryforwards and other applicable tax attributes that we can use in the future to offset future taxable income. At the state level, California has enacted legislation that, with certain exceptions, suspends the ability to use California NOLs to offset California income and limits the ability to use California business tax credits to offset California taxes, for taxable years beginning after 2023 and before 2027. Other states may also suspend or place limitation on the NOL utilization. We have recorded a full valuation allowance related to our NOLs and other deferred tax assets due to the uncertainty of the ultimate realization of the future benefits of those assets.

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

New income, sales, use, excise or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could affect the tax treatment of our (and our subsidiaries') domestic and foreign financial results. Any new taxes could adversely affect our domestic and international business operations, and our business and financial performance. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us. Future guidance from the U.S. Internal Revenue Service and other tax authorities with respect to such legislation may adversely affect us, and certain aspects of such legislation could be repealed or modified in the future, which could have an adverse effect on us. For example, the OBBBA made a number of changes to U.S. federal income tax law, including 100% bonus depreciation, domestic research cost expensing, and modifications to the international tax framework. We are currently evaluating the impact of the OBBBA upon our future tax liabilities and continuing to monitor changes in tax laws and regulations.

Our indebtedness and liabilities could limit the cash flow available for our operations, expose us to risks that could adversely affect our business, financial condition and results of operations and impair our ability to satisfy our obligations under our indebtedness.

As of September 30, 2025, we had $316.2 million aggregate principal amount of indebtedness under the Notes.

We may also incur additional indebtedness to meet future financing needs. Our indebtedness could have significant negative consequences for our security holders and our business, results of operations and financial condition by, among other things:

- increasing our vulnerability to adverse economic and industry conditions;
- limiting our ability to obtain additional financing;
- requiring the dedication of a substantial portion of our cash flow from operations to service our indebtedness, which will reduce the amount of cash available for other purposes;
- limiting our flexibility to plan for, or react to, changes in our business;
- diluting the interests of our existing stockholders as a result of issuing shares of our common stock upon conversion of the Notes; and
- placing us at a possible competitive disadvantage with competitors that are less leveraged than us or have better access to capital.

Our ability to make scheduled payments of the principal of, to pay interest on or to refinance our indebtedness, including the Notes, depends on our future performance, which is subject to economic, financial, competitive and other factors beyond our control. Our business may not generate sufficient funds, and we may otherwise be unable to maintain sufficient cash reserves, to pay amounts due under our indebtedness, including the Notes, and our cash needs may increase in the future.

The conditional conversion feature of the Notes may adversely affect our financial condition and operating results, and conversion of our outstanding Notes may result in the dilution of existing stockholders, create downward pressure on the price of our common stock, and restrict our ability to take advantage of future opportunities.

The conditional conversion feature of the Notes entitles holders of the Notes to convert the Notes at any time during specified periods at their option if such conditions are met. For example, the conditional conversion feature of the Notes has been met previously, including in September 2025. Consequently, for prior quarterly periods, the Notes were, and for the quarterly period ending December 31, 2025, the Notes are, subject to conversion at the election of the holders. If one or more holders elect to convert their Notes, unless we elect to satisfy our conversion obligation by delivering solely shares of our common stock (other than paying cash in lieu of delivering any fractional share), we would be required to settle a portion or all of our conversion obligation in cash, which could adversely affect our liquidity.

The Notes may be converted into cash, shares of our common stock or a combination of cash and shares of our common stock. If shares of our common stock are issued to the holders of the Notes upon conversion as they have been in the past, there will be dilution to our stockholders' equity and the market price of our shares may decrease due to the additional selling pressure in the market. Any downward pressure on the price of our common stock caused by the sale or potential sale of shares issuable upon conversion of the Notes could also encourage short sales by third parties, creating additional selling pressure on our stock. The existence of the Notes and the obligations that we incurred by issuing them may restrict our ability to take advantage of certain future opportunities, such as engaging in future debt or equity financing activities.

Also, ASU No. 2020-06 requires the application of the if-converted method to calculate the impact of convertible instruments on diluted earnings per share when the instruments may be settled in cash or shares. See Note 2, Summary of Significant Accounting Policies. During the three months ended September 30, 2025, the conditional conversion feature of the Notes was triggered and the Notes are convertible, in whole or in part, at the option of the holders between October 1, 2025 through December 31, 2025. We use the if-converted method for calculating any potential dilutive effect of the conversion options embedded in the Notes on diluted net income per share.

The accounting method for the Notes could adversely affect our reported financial condition and results.

In August 2020, the Financial Accounting Standards Board published an Accounting Standards Update, which we refer to as ASU 2020-06, which simplifies certain of the accounting standards that apply to convertible notes. In accordance with ASU 2020-06, the Notes are reflected as a liability on our balance sheets, with the initial carrying amount equal to the principal amount of the Notes, net of issuance costs. The issuance costs are treated as a debt discount for

accounting purposes, which will be amortized into interest expense over the term of the Notes. As a result of this amortization, the interest expense that we expect to recognize for the Notes for accounting purposes will be greater than the cash interest payments we will pay on the Notes, which will result in lower reported income.

In addition, the shares underlying the Notes are reflected in our diluted earnings per share using the "if converted" method, in accordance with ASU 2020-06. Under that method, diluted earnings per share would generally be calculated assuming that all the Notes were converted solely into shares of common stock at the beginning of the reporting period, unless the result would be anti-dilutive. The application of the if-converted method may reduce our reported diluted earnings per share.

In the future, we may, in our sole discretion, irrevocably elect to settle the conversion value of the Notes in cash up to the principal amount being converted. Following such an irrevocable election, if the conversion value of the Notes exceeds their principal amount for a reporting period, then we will calculate our diluted earnings per share by assuming that all of the Notes were converted at the beginning of the reporting period and that we issued shares of our common stock to settle the excess, unless the result would be anti-dilutive.

Risks Related to Our Intellectual Property

We do not currently have patent protection or regulatory exclusivity for certain of our approved medicines or rely on regulatory exclusivity. If we are unable to obtain and maintain sufficient intellectual property protection for our approved medicines and our product candidates, or if the scope of the intellectual property protection is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our approved medicines and our other product candidates, if approved, may be adversely affected.

Our commercial success will depend in part on obtaining and maintaining a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our technologies. We do not have, and do not expect to obtain, patent protection for any commercial form of chenodiol or Cholbam. Any unauthorized 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 our market.

The patent positions of biotechnology and pharmaceutical companies can be highly uncertain and involve complex legal and factual questions for which important legal principles remain unresolved. No consistent policy regarding the breadth of claims allowed in pharmaceutical patents has emerged to date in the U.S. or in many jurisdictions outside of the U.S. Changes in either the patent laws or interpretations of patent laws in the U.S. and other jurisdictions may diminish the value of our intellectual property. Accordingly, we cannot predict the breadth of claims that may be enforced in the patents that may be issued from the applications we currently or may in the future own or license from third parties. Further, if any patents we obtain or license are deemed invalid and unenforceable, our ability to commercialize or license our technology could be adversely affected.

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or any of our actual or potential future collaborators will be successful in protecting our approved medicines or product candidates, proprietary technologies and their uses by obtaining and defending patents. These risks and uncertainties include the following:

- the U.S. Patent and Trademark Office ("USPTO") and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process, the noncompliance with which can result in abandonment or lapse of a patent or patent application, and partial or complete loss of patent rights in the relevant jurisdiction;
- patent applications may not result in any patents being issued;
- patents that may be issued or in-licensed have been and may again in the future be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable or otherwise may not provide any competitive advantage;
- our competitors, many of whom have substantially greater resources than we do and many of whom have made significant investments in competing technologies, may seek or may have already obtained patents that will limit, interfere with or eliminate our ability to make, use, import, and sell our approved medicines or our product candidates;
- other parties may have designed around our claims or developed technologies that may be related or competitive to our platform, may have filed or may file patent applications and may have received or may

receive patents that overlap or conflict with our patent applications, either by claiming the same methods or devices or by claiming subject matter that could dominate our patent position;

- any successful opposition or other post-grant proceeding to any patents owned by or licensed to us could deprive us of rights necessary for the practice of our technologies or the successful commercialization of any products or product candidates that we may develop;
- because patent applications in the U.S. and most other jurisdictions are confidential for a period of time after filing, we cannot be certain that we or our licensors were the first to file any patent application related to our approved medicines or our product candidates, proprietary technologies and their uses;
- an interference proceeding can be provoked by a third party or instituted by the USPTO to determine who was the first to invent any of the subject matter covered by the patent claims of our applications for any application with an effective filing date before March 16, 2013;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the U.S. for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns; and
- jurisdictions other than the U.S. may have patent laws less favorable to patentees than those upheld by U.S. courts, allowing foreign competitors a better opportunity to create, develop and market competing products.

The patent prosecution process is also expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach such agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection. We may also rely on trade secrets to protect our technology, especially where we do not believe patent protection is appropriate or feasible. However, trade secrets are difficult to protect. Although we use reasonable efforts to protect our trade secrets, our employees, consultants, contractors, outside scientific collaborators and other advisors may unintentionally or willfully disclose our information to competitors. Enforcing a claim that a third party illegally obtained and is using any of our trade secrets is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the U.S. are sometimes less willing to protect trade secrets. Moreover, our competitors may independently develop equivalent knowledge, methods and know-how.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. Only limited protection may be available and may not adequately protect our rights or permit us to gain or keep any competitive advantage. If we do not adequately protect our intellectual property and proprietary technology, competitors may be able to use our approved medicines, our product candidates and proprietary technologies and erode or negate any competitive advantage we may have, which could have a material adverse effect on our financial condition and results of operations. For example:

- others may be able to make compounds that are similar to our approved medicines and our product candidates but that are not covered by the claims of our patents;
- we might not have been the first to make the inventions covered by our pending patent applications;
- we might not have been the first to file patent applications for these inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies;
- any patents that we obtain may not provide us with any competitive advantages;
- we may not develop additional proprietary technologies that are patentable;
- our competitors might conduct research and development activities in jurisdictions 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 cannot ensure that any of our patents, or any of our pending patent applications, if issued, or those of our licensors, will include claims having a scope sufficient to protect our approved medicines and any then-approved medicine;

- we cannot ensure that we will be able to successfully commercialize our approved medicines and any then-approved product on a substantial scale, if approved, before the relevant patents that we own or license expire; or
- the patents of others may have an adverse effect on our business.

Others have filed, and in the future are likely to file, patent applications covering products and technologies that are similar, identical or competitive to ours or important to our business. We cannot be certain that any patent application owned by a third party will not have priority over patent applications filed or in-licensed by us, or that we or our licensors will not be involved in additional interference, opposition or other patent office proceedings before the USPTO or non-U.S. patent offices.

We cannot be certain that the claims in our issued patents and pending patent applications covering our approved medicines or our product candidates will be considered patentable by the USPTO, courts in the U.S., or by patent offices and courts in foreign jurisdictions. Furthermore, the laws of some foreign jurisdictions do not protect proprietary rights to the same extent or in the same manner as the laws of the U.S. As a result, we may encounter significant problems in protecting and defending our intellectual property.

The strength of patents in the biotechnology and pharmaceutical fields involves complex legal and scientific questions and can be uncertain. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our approved medicines or our product candidates in the U.S. or in foreign jurisdictions. Even if such patents do successfully issue, third parties have and may again in the future challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. Any successful opposition or other post-grant proceeding to our patents could deprive us of exclusive rights necessary for the successful commercialization of our approved medicines or our product candidates. Furthermore, even if they are unchallenged, our patents may not adequately protect our intellectual property, provide exclusivity for approved medicines or our product candidates or prevent others from designing around our claims. If the breadth or strength of protection provided by the patents we hold with respect to our approved medicines or our product candidates is threatened, it could dissuade companies from collaborating with us to develop, or threaten our ability to commercialize, our approved medicines or our product candidates.

Further, if we encounter delays in our development efforts, including our clinical trials, the period of time during which we could market our approved medicines or our product candidates under patent protection would be reduced. In addition, patents have a limited lifespan. In the U.S., the natural expiration of a patent is generally 20 years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. A patent term extension of up to five years based on regulatory delay may be available in the U.S. under the Hatch-Waxman Act. However, only a single patent can be extended for each marketing approval, and any patent can be extended only once, for a single product. Moreover, the scope of protection during the period of the patent term extension does not extend to the full scope of the claim, but instead only to the scope of the product as approved. Further, a patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only those claims covering such approved drug product, an approved method for using it or a method for manufacturing it may be extended. Laws governing analogous patent term extensions in foreign jurisdictions vary widely, as do laws governing the ability to obtain multiple patents from a single patent family. Additionally, we may not receive an extension if we fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration, or the term of any such extension is less than we request, 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.

For U.S. patent applications in which claims are entitled to a priority date before March 16, 2013, an interference proceeding can be provoked by a third party or instituted by the USPTO to determine who was the first to invent any of the subject matter covered by the patent claims of our patents or patent applications. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our participation in an interference proceeding may fail and, even if successful, may result in substantial costs and distract our management and other employees.

For U.S. patent applications containing a claim not entitled to priority before March 16, 2013, there is greater level of uncertainty in the patent law. In September 2011, the Leahy-Smith America Invents Act, or America Invents Act, was signed into law. The America Invents Act includes a number of significant changes to U.S. patent law, including provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. The USPTO has developed regulations and procedures to govern the administration of the America Invents Act, and many of the

substantive changes to patent law associated with the America Invents Act, and in particular, the "first to file" provisions, were enacted on March 16, 2013. The America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable, or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our approved medicines and our product candidates and drug discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. We require all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology, such as third parties involved in the manufacture of our approved medicines and our product candidates and third parties involved in our clinical trials to enter into confidentiality agreements. We cannot be certain that all such agreements have been duly executed, that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets could impair our competitive position and may have a material adverse effect on our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. For example, the FDA, as part of its Transparency Initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all. If we are unable to prevent unauthorized material disclosure of our intellectual property to third parties, we may not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, operating results and financial condition.

We currently rely on method-of-use and formulation patents to protect Livmarli and composition-of-matter and method-of-use patents to protect volixibat.

We currently have rights to patents and patent applications in the U.S., Europe and other jurisdictions covering methods of treating certain cholestatic liver diseases using certain IBATis, including maralixibat (the API of Livmarli) and volixibat. Patent applications may never issue as patents. We do not have patents or patent applications covering maralixibat as a composition-of-matter. Therefore, the primary patent-based intellectual property protection for our Livmarli program are granted method-of-use patents and any patents that may grant on currently pending method-of-use and formulation patent applications.

Composition-of-matter patents on APIs are generally considered to be the strongest form of intellectual property protection for pharmaceutical products, as such patents provide protection without regard to any method of use. Method-of-use patents protect the use of a product for the specified method. Method-of-use patents do not prevent a competitor from making and marketing a product that is identical to our product for an indication that is outside the scope of the patented method. Moreover, even if competitors do not actively promote their products for our targeted indication(s), physicians may prescribe these products "off-label." Although off-label prescriptions may infringe or contribute to the infringement of method-of-use patents, the practice is common and such infringement is difficult to prevent or prosecute.

Obtaining and maintaining our 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.

The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent process. Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on any issued patents and/or applications are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patents and/or applications. We have systems in place to remind us to pay these fees, and we employ an outside firm and rely on our outside counsel to pay these fees due to foreign patent agencies. While an inadvertent lapse may sometimes be cured by payment of a late fee or by other means in accordance with the applicable rules, 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 enter the market earlier than should otherwise have been the case, which would have a material adverse effect on our business.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect Livmarli and our product candidates.

As is the case with other biotechnology and pharmaceutical companies, our success is heavily dependent on intellectual property, particularly on obtaining and enforcing patents. Our patent rights may be affected by developments or uncertainty in U.S. or foreign patent statutes, patent case law, USPTO rules and regulations or the rules and regulations of foreign patent offices. Obtaining and enforcing patents in the biotechnology industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. In addition, the U.S. may, at any time, enact changes to U.S. patent law and regulations, including by legislation, by regulatory rule-making, or by judicial precedent, that adversely affect the scope of patent protection available and weakened the rights of patent owners to obtain patents, enforce patent infringement and obtain injunctions and/or damages. For example, the scope of patentable subject matter under 35 U.S.C. 101 has evolved significantly over the past several years as the Court of Appeals for the Federal Circuit and the Supreme Court issued various opinions, and the USPTO modified its guidance for practitioners on multiple occasions. Other jurisdictions may likewise enact changes to their patent laws in ways that adversely diminish the scope of patent protection and weaken the rights of patent owners to obtain patents, enforce patent infringement and obtain injunctions and/or damages. Further, the U.S. and other governments may, at any time, enact changes to laws and regulations that create new avenues for challenging the invalidity of issued patents. For example, the America Invents Act created new administrative post-grant proceedings, including post-grant review, inter partes review, and derivation proceedings that allow third parties to challenge the validity of issued patents. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

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

Patents are of national or regional effect. Filing, prosecuting and defending patents on Livmarli and our product candidates in all jurisdictions throughout the world would be prohibitively expensive. In addition, the laws of some foreign jurisdictions do not protect intellectual property rights in the same manner and to the same extent as laws in the U.S. Consequently, we may not be able to prevent third parties from practicing our inventions in all jurisdictions outside the U.S. 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 enforcement of such patent protection is not as strong as that in the U.S. These products may compete with Livmarli and any then-approved product and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

The requirements for patentability may differ in certain jurisdictions. For example, unlike other jurisdictions, China has a heightened requirement for patentability, and specifically requires a detailed description of medical uses of a claimed drug. In India, unlike the U.S., there is no link between regulatory approval for a drug and its patent status. In addition to India, certain jurisdictions in Europe and developing jurisdictions, including China, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In those jurisdictions, we may have limited remedies if patents are infringed or if we are compelled to grant a license to a third party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we own or license.

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, trade secrets and other intellectual property protection, particularly those relating to biotechnology or pharmaceutical products, 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 costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly, and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

In addition, geo-political actions in the U.S. and in foreign countries could increase the uncertainties and costs surrounding the prosecution or maintenance of our patent applications or those of any current or future licensors and the maintenance, enforcement or defense of our issued patents or those of any current or future licensors. For example, due to the Russia-Ukraine conflict, the U.S. and other foreign governments have implemented various economic sanctions and

trade and activity restrictions involving Russia and Belarus. It is possible that additional sanctions and restrictions will be imposed by the U. S. or other jurisdictions as the Russia-Ukraine conflict continues, and such actions may include limiting or preventing filing, prosecution, and/or maintenance of patent applications in Russia. Government actions may also prevent maintenance of issued patents in Russia. These actions could result in abandonment or lapse of our patents or patent applications, resulting in partial or complete loss of patent rights in Russia. If such an event were to occur, it could have a material adverse effect on our business. In addition, a decree was adopted by the Russian government in March 2022, allowing Russian companies and individuals to exploit inventions owned by patentees from the U.S. without consent or compensation. Consequently, we would not be able to prevent third parties from practicing our inventions in Russia or from selling or importing products made using our inventions in and into Russia. Accordingly, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

Finally, Europe's Unified Patent Court may in particular present uncertainties for our ability to protect and enforce our patent rights against competitors in Europe. On June 1, 2023, the EU unitary patent system was launched, providing a single pan-European Unitary Patent and a new European Unified Patent Court ("UPC"), for litigation involving European patents. Under the UPC, all European patents, including those issued prior to ratification of the European Patent Package, will by default automatically fall under the jurisdiction of the UPC. The UPC will provide our competitors with a new forum to centrally revoke our European patents that have not been opted out of the UPC, and allow for the possibility of a competitor to obtain pan-European injunctions. It will be several years before we will understand the scope of patent rights that will be recognized and the strength of patent remedies that will be provided by the UPC. Under the EU unitary patent system, we will have the right to opt our patents out of the UPC over the first seven years of the court's existence, but doing so may preclude us from realizing the benefits of the new unified court.

If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We are a party to a number of license agreements under which we are granted intellectual property rights that are important to our business. For example, certain trade secrets related to maralixibat are licensed from Pfizer, and patents, patent applications and trade secrets related to volixibat are licensed from Sanofi. Our existing license agreements as related to our approved medicines and product candidates impose various development, regulatory and/or commercial diligence obligations, payment of milestones and/or royalties and other obligations. If we fail to comply with our obligations under a license agreement, or we are subject to a bankruptcy, the license agreement may be terminated, in which event we would not be able to develop, commercialize or market our approved medicines or other product candidates, as the case may be.

Licensing of intellectual property rights is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise between us and our licensors regarding intellectual property rights subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property rights of the licensor that are not subject to the licensing agreement;
- our right to sublicense intellectual property rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our approved medicines and our product candidates, and what activities satisfy those diligence obligations; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

If disputes over intellectual property rights that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, our business, results of operations, financial condition and prospects may be adversely affected. We may enter into additional licenses in the future and if we fail to comply with obligations under those agreements, we could suffer adverse consequences.

We may become subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may be subject to claims that former employees (including former employees of our licensors), collaborators or other third parties have an interest in our patents or other intellectual property as an inventor or co-inventor. The failure to name the proper inventors on a patent application can result in the patents issuing thereon being unenforceable. Inventorship disputes may arise from conflicting views regarding the contributions of different individuals named as inventors, the effects of foreign laws where foreign nationals are involved in the development of the subject matter of the patent, conflicting obligations of third parties involved in developing Livmarli or our product candidates or as a result of questions regarding co-ownership of potential joint inventions. Litigation may be necessary to resolve these and other claims challenging inventorship and/or ownership. Alternatively, or additionally, we may enter into agreements to clarify the scope of our rights in such intellectual property. 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. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

We may not be successful in obtaining or maintaining necessary rights to product components and processes for our development pipeline through acquisitions and in-licenses.

Presently we have intellectual property rights, through licenses from third parties related to our approved medicines and our product candidates. For example, we have license agreements with Shire and Satiogen for both maralixibat and volixibat. We have our license agreement with Shire, Satiogen and Pfizer for our intellectual property rights covering maralixibat. Further, we have our license agreement with Sanofi for our intellectual property rights covering volixibat. Because our programs may require the use of additional proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license or use these proprietary rights. In addition, Livmarli or our product candidates may require specific formulations to work effectively and efficiently and these rights may be held by others. We may be unable to acquire or in-license proprietary rights related to any compositions, formulations, methods of use, processes or other intellectual property rights from third parties that we identify as being necessary for Livmarli or our product candidates. Even if we are able to obtain a license to such proprietary rights, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement technology.

Where we obtain licenses from or collaborate with third parties, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from third parties, or such activities, if controlled by us, may require the input of such third parties. We may also require the cooperation of our licensors and collaborators to enforce any licensed patent rights, and such cooperation may not be provided. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business, in compliance with applicable laws and regulations, which may affect the validity and enforceability of such patents or any patents that may issue from such application. Moreover, if we do obtain necessary licenses, we will likely have obligations under those licenses, including making royalty and milestone payments, and any failure to satisfy those obligations could give our licensor the right to terminate the license. Termination of a necessary license, or expiration of licensed patents or patent applications, could have a material adverse impact on our business. Our business would suffer if any such licenses terminate, if the licensors fail to abide by the terms of the license, if the licensors fail to enforce licensed patents against infringing third parties, if the licensed patents or other rights are found to be invalid or unenforceable, or if we are unable to enter into necessary licenses on acceptable terms. Furthermore, if any licenses terminate, or if the underlying patents fail to provide the intended exclusivity, competitors or other third parties may gain the freedom to seek regulatory approval of, and to market, products identical to ours. Moreover, our licensors may own or control intellectual property that has not been licensed to us and, as a result, we may be subject to claims, regardless of their merit, that we are infringing or otherwise violating the licensor's rights. In addition, while we cannot currently determine the amount of the royalty obligations we would be required to pay on sales of future products, if any, the amounts may be significant. The amount of our future royalty obligations will depend on the technology and intellectual property we use in products that we successfully develop and commercialize, if any. Therefore, even if we successfully develop and commercialize products, we may be unable to achieve or maintain profitability.

The licensing and acquisition of third-party proprietary rights is a competitive area, and companies, which may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third-party proprietary rights that we may consider necessary or attractive in order to commercialize our approved medicines or our product candidates. More established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

For example, we have collaborated and may in the future collaborate with U.S. and foreign academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate an exclusive license to any of the institution's proprietary rights in technology resulting from the collaboration. Regardless of such option to negotiate a license, we may be unable to negotiate a license within the specified time frame or under terms that are acceptable to us. If we are unable to do so, the institution may offer, on an exclusive basis, their proprietary rights to other parties, potentially blocking our ability to pursue our program.

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us, either on reasonable terms, or at all. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment, or at all. If we are unable to successfully obtain rights to required third-party intellectual property rights on commercially reasonable terms, our ability to commercialize our approved medicines and any then-approved product, and our business, financial condition and prospects for growth could suffer.

Third-party claims alleging intellectual property infringement may prevent or delay our drug discovery and development efforts.

Our success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the U.S., involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including inter partes review, post-grant proceedings, interference and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. The America Invents Act introduced new procedures including inter partes review and post grant review. The implementation of these procedures brings uncertainty to the possibility of challenges to our patents in the future and the outcome of such challenges. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are marketing our approved medicines and developing our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our activities related to our approved medicines and our product candidates may give rise to claims of infringement of the patent rights of others.

The pharmaceutical and biotechnology industries have produced a proliferation of patents, and it is not always clear to industry participants, including us, which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. Any of our approved medicines or our current or future product candidates may infringe existing or future patents. We may not be aware of patents that have already issued that a third party might assert are infringed by our approved medicines or one of our current or future product candidates. Nevertheless, we are not aware of any issued patents that will prevent us from marketing our approved medicines or our product candidates.

Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents of which we are currently unaware with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our approved medicines or our product candidates. Because patent applications can take many years to issue and may be confidential for 18 months or more after filing, there may be currently pending third-party patent applications which may later result in issued patents that our approved medicines, our product candidates or our technologies may infringe, or which such third parties claim are infringed by the use of our technologies. Parties making claims against us for infringement or misappropriation of their intellectual property rights may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our approved medicines or one or more of our product candidates. Defense of these claims, regardless of their merit, could involve substantial expenses and could be a substantial diversion of employee resources from our business.

If we collaborate with third parties in the development of technology in the future, our collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to litigation or potential liability. Further, collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability. In the future, we may agree to indemnify our commercial collaborators against certain intellectual property infringement claims brought by third parties.

Any claims of patent infringement asserted by third parties would be time consuming and could:

- result in costly litigation;
- divert the time and attention of our technical personnel and management;

- cause development delays;
- prevent us from commercializing our approved medicines or our product candidates until the asserted patent expires or is held finally invalid or not infringed in a court of law;
- require us to develop non-infringing technology, which may not be possible on a cost-effective basis;
- require us to pay damages to the party whose intellectual property rights we may be found to be infringing, which may include treble damages if we are found to have been willfully infringing such intellectual property;
- require us to pay the attorney's fees and costs of litigation to the party whose intellectual property rights we may be found to be infringing; and/or
- require us to enter into royalty or licensing agreements, which may not be available on commercially reasonable terms, or at all.

If we are sued for patent infringement, we would need to demonstrate that our approved medicines and any then-approved product or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid, and we may not be able to do either. Proving invalidity is difficult. For example, in the U.S., proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and divert management's time and attention in pursuing these proceedings, which could have a material adverse effect on us. If we are unable to avoid infringing the patent rights of others, we may be required to seek a license, which may not be available, defend an infringement action or challenge the validity of the patents in court. Patent litigation is costly and time consuming. We may not have sufficient resources to bring these actions to a successful conclusion. In addition, if we do not obtain a license, develop or obtain non-infringing technology, fail to defend an infringement action successfully or have infringed patents declared invalid, we may incur substantial monetary damages, encounter significant delays in bringing our product candidates to market and be precluded from manufacturing or selling our approved medicines or our product candidates.

We do not always conduct independent reviews of pending patent applications of and patents issued to third parties. We cannot be certain that others have not filed patent applications for technology covered by our pending applications, or that we were the first to invent the technology, because:

- some patent applications in the U.S. may be maintained in secrecy until the patents are issued;
- patent applications in the U.S. and elsewhere can be pending for many years before issuance, or unintentionally abandoned patents or applications can be revived;
- pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our technologies, our approved medicines, our product candidates or the use thereof;
- identification of third-party patent rights that may be relevant to our technology is difficult because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims;
- patent applications are typically not published until 18 months after the priority date; and
- publications in the scientific literature often lag behind actual discoveries.

Furthermore, the scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history and can involve other factors such as expert opinion. Our interpretation of the relevance or the scope of claims in a patent or a pending application may be incorrect, which may negatively impact our ability to market our approved medicines and any then-approved product. Further, we may incorrectly determine that our technologies, our approved medicines and any then-approved product, or product candidates are not covered by a third-party patent or may incorrectly predict whether a third party's pending patent application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the U.S. or internationally that we consider relevant may be incorrect, which may negatively impact our ability to develop and market our approved medicines or our product candidates.

Our competitors may have filed, and may in the future file, patent applications covering technology similar to ours, and others may have or obtain patents or proprietary rights that could limit our ability to make, use, sell, offer for sale or import our approved medicines, our product candidates and future approved medicines or impair our competitive position. Numerous third-party U.S. and foreign issued patents and pending patent applications exist in the fields in which

we are marketing our approved medicines and developing product candidates. 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 our approved medicines and our product candidates. Any such patent application may have priority over our patent applications, which could further require us to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on inventions similar to ours, we may have to participate in an interference proceeding declared by the USPTO to determine priority of invention in the U.S. The costs of these proceedings could be substantial, and it is possible that such efforts would be unsuccessful if, unbeknownst to us, the other party had independently arrived at the same or similar invention prior to our own invention, resulting in a loss of our U.S. patent position with respect to such inventions. Other jurisdictions have similar laws that permit secrecy of patent applications and may be entitled to priority over our applications in such jurisdictions.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations.

If a third party prevails in a patent infringement lawsuit against us, we may have to stop making and selling the infringing product, pay substantial damages, including treble damages and attorneys' fees if we are found to be willfully infringing a third party's patents, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, 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. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of our approved medicines and our product candidates. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize our approved medicines and our product candidates, which could harm our business significantly. Even if we were able to obtain a license, the rights may be nonexclusive, which may give our competitors access to the same intellectual property.

We may be subject to claims that we have wrongfully hired an employee from a competitor or that we or our employees have wrongfully used or disclosed alleged confidential information or trade secrets of their former employers.

As is common in the biotechnology and pharmaceutical industries, in addition to our employees, we engage the services of consultants to assist us in the development of our product candidates. Many of these consultants, and many of our employees, were previously employed at, or may have previously provided or may be currently providing consulting services to, other pharmaceutical companies including our competitors or potential competitors. We may become subject to claims that we, our employees or consultants inadvertently or otherwise used or disclosed trade secrets or other information proprietary to their former employers or their former or current clients. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely affect our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to our management team and other employees.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time consuming, and unsuccessful. Further, our issued patents could be found invalid or unenforceable if challenged in court, and we may incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights.

Third parties including competitors may infringe, misappropriate or otherwise violate our patents, patents that may issue to us in the future, or the patents of our licensors that are licensed to us. To counter infringement or unauthorized use, we may need to or choose to file infringement claims, which can be expensive and time-consuming. We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in jurisdictions where the laws may not protect those rights as fully as in the U.S. Further, because of the expense and uncertainty of litigation, we may conclude that even if a third party is infringing our issued patent, any patents that may be issued as a result of our pending or future patent applications or other intellectual property rights, the risk-adjusted cost of bringing and enforcing such a claim or action may be too high or not in the best interest of our company or our stockholders. In such cases, we may decide that the more prudent course of action is to simply monitor the situation or initiate or seek some other non-litigious action or solution.

If we choose to go to court to stop another party from using the inventions claimed in our patents, that individual or company has the right to ask the court to rule that such patents are invalid, unenforceable, or should not be enforced against that third party for any number of reasons. In patent litigation in the U.S., defendant counterclaims

alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge include an alleged failure to meet any of several statutory requirements for patentability, including lack of novelty, obviousness, lack of written description, indefiniteness, or non-enablement. Grounds for an unenforceability assertion could include an allegation that someone connected with prosecution of the patent withheld material information from the USPTO or made a misleading statement during prosecution, i.e. committed inequitable conduct. Third parties may also raise similar claims before the USPTO, even outside the context of litigation. Similar mechanisms for challenging the validity and enforceability of a patent exist in foreign patent offices and courts and may result in the revocation, cancellation, or amendment of any foreign patents we or our licensors hold now or in the future. The outcome following legal assertions of invalidity and unenforceability is unpredictable, and prior art could render our patents or those of our licensors invalid. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on such product or product candidate. Such a loss of patent protection would have a material adverse impact on our business.

Interference or derivation proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms or at all, or if a non-exclusive license is offered and our competitors gain access to the same technology. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties or enter into development or manufacturing partnerships that would help us bring our product candidates to market.

Even if resolved in our favor, litigation or other legal proceedings relating to our intellectual property rights may cause us to incur significant expenses and could distract our 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 the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace.

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

Our ability to enforce our patent rights depends on our ability to detect infringement. It may be difficult to detect infringers who do not advertise the components or methods that are used in connection with their products and services. Moreover, it may be difficult or impossible to obtain evidence of infringement in a competitor's or potential competitor's product or service. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded if we were to prevail may not be commercially meaningful.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed. Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

We rely on trade secrets to protect our proprietary technologies, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect. We rely in part on confidentiality agreements with our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors, and inventions agreements with employees, consultants and advisors, to protect our trade secrets and other proprietary information. In addition to contractual measures, we try to protect the confidential nature of our proprietary information using commonly accepted physical and technological security measures. Despite these efforts, we cannot provide any assurances that all such agreements have been duly executed, and these agreements may not effectively prevent disclosure of confidential information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its Transparency Initiative, is currently considering whether to make additional information publicly

available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all. Costly and time-consuming litigation could be necessary to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

In addition, such security measures may not provide adequate protection for our proprietary information, for example, in the case of misappropriation of a trade secret by an employee, consultant, customer or third party with authorized access. Our security measures may not prevent an employee, consultant or customer from misappropriating our trade secrets and providing them to a competitor, and recourse we take against such misconduct may not provide an adequate remedy to protect our interests fully. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. Unauthorized parties may also attempt to copy or reverse engineer certain aspects of our approved medicines and any then-approved product that we consider proprietary. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret can be difficult, expensive and time-consuming, and the outcome is unpredictable. Even though we use commonly accepted security measures, the criteria for protection of trade secrets can vary among different jurisdictions.

Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the U.S. are less willing or unwilling to protect trade secrets. Moreover, third parties may still obtain this information or may come upon this or similar information independently, and we would have no right to prevent them from using that technology or information to compete with us. Trade secrets may over time be disseminated within the industry through independent development, the publication of journal articles and the movement of personnel skilled in the art from company to company or academic to industry scientific positions. Though our agreements with third parties typically restrict the ability of our advisors, employees, collaborators, licensors, suppliers, third-party contractors and consultants to publish data potentially relating to our trade secrets, our agreements may contain certain limited publication rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position. Because from time to time we expect to rely on third parties in the development, manufacture, and distribution of our approved medicines and any then-approved product and provision of our services, we must, at times, share trade secrets with them. Despite employing the contractual and other security precautions described above, the need to share trade secrets increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. If any of these events occur or if we otherwise lose protection for our trade secrets, the value of this information may be greatly reduced and our competitive position would be harmed. If we do not apply for patent protection prior to such publication or if we cannot otherwise maintain the confidentiality of our proprietary technology and other confidential information, then our ability to obtain patent protection or to protect our trade secret information may be jeopardized.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Our current or future trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. We have and may continue to license our trademarks and trade names to third parties, such as distributors. Though these license agreements may provide guidelines for how our trademarks and trade names may be used, a breach of these agreements or misuse of our trademarks and tradenames by our licensees may jeopardize our rights in or diminish the goodwill associated with our trademarks and trade names. Our efforts to enforce or protect our proprietary rights related to trademarks, trade names, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely affect our financial condition or results of operations.

Moreover, any name we have proposed to use with our product or product candidates in the U.S. must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark.

The FDA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA (or an equivalent administrative body in a foreign jurisdiction) objects to

any of our proposed proprietary product names, we may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark or regulatory laws, not infringe the existing rights of third parties and be acceptable to the relevant administrative body. Furthermore, in many jurisdictions, owning and maintaining a trademark registration may not provide an adequate defense against a subsequent infringement claim asserted by the owner of a senior trademark. At times, competitors or other third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. If we assert trademark infringement claims, a court may also determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights denying our claim. In this case, we could ultimately be forced to cease use of such trademarks. Similar requirements exist in most jurisdictions worldwide.

Risks Related to Ownership of Our Common Stock

The trading price of our common stock may be volatile, and you could lose all or part of your investment.

The trading price of our common stock is likely to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. For example, the closing price of our common stock since January 1, 2025 to November 3, 2025 has ranged from a low of $38.39 to a high of $77.69. In addition to the factors discussed in this "Risk Factors" section, these factors include, among others:

- the degree of physician and patient adoption of our approved medicines and use of our approved medicines necessary for commercial success;
- our failure to grow and maintain our own sales force to market our approved medicines;
- our ability to market and sell our approved medicines, where approved;
- any delay in our regulatory filings for Livmarli, Ctexli or volixibat and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such filings, including without limitation the FDA's issuance of a "refusal to file" letter or a request for additional information;
- our ability to scale our distribution capabilities;
- any delay in our regulatory filings for our product candidates and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such filings, including without limitation the FDA's issuance of a "refusal to file" letter or a request for additional information;
- our failure to commercialize our product candidates;
- the commencement, enrollment or results of our ongoing clinical trials of our product candidates or any future clinical trials we may conduct, or changes in the development status of our product candidates;
- adverse results or delays in clinical trials;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- adverse regulatory decisions, including failure to receive regulatory approval for our product candidates;
- changes in laws or regulations applicable to our approved medicines and our product candidates, including but not limited to clinical trial requirements for approvals;
- changes in the structure of health care payment systems;
- the failure to obtain coverage and adequate reimbursement of our approved medicines and our product candidates, if approved;
- adverse developments concerning our manufacturers;
- our inability to obtain adequate product supply for any approved drug product or inability to do so at acceptable prices;
- our inability to maintain or establish collaborations if needed;
- our ability to in-license, acquire, develop and market additional product candidates or approved medicines;

- management transitions and additions or departures of key scientific or management personnel;
- unanticipated serious safety concerns related to the use of our approved medicines or our product candidates;
- introduction of new products or services offered by us or our competitors;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- our ability to effectively manage our growth;
- the size and growth, if any, of the markets for our approved medicines with approved indications;
- our ability to successfully enter new markets or develop additional product candidates;
- actual or anticipated variations in quarterly operating results;
- our cash position;
- our failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;
- publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- changes in the market valuations of similar companies;
- overall performance of the equity markets;
- issuances of debt or equity securities;
- sales of our common stock by us or our stockholders in the future;
- trading volume of our common stock;
- changes in accounting practices;
- ineffectiveness of our internal controls;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- geopolitical and macroeconomic developments, including the ongoing military conflicts, economic slowdowns, recessions, inflation, tariffs and trade tensions, the ongoing shutdown of the federal government and the resulting effects on its regulatory agencies, bank failures, high interest rates and tightening of credit markets; and
- other events or factors, many of which are beyond our control.

Volatility in the trading price of our common stock could also prohibit or delay us from executing on our strategy, including in-licensing or acquiring additional product candidates or approved medicines using our common stock as consideration or raising additional capital on favorable terms or at all, any of which could exacerbate the volatility of the trading price of our common stock. In addition, the stock market in general, and Nasdaq-listed and biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. In the past, securities class action litigation has often been instituted against companies following periods of volatility in the market price of a company's securities. This type of litigation, if instituted, could result in substantial costs and a diversion of management's attention and resources, which would harm our business, operating results or financial condition.

We do not intend to pay dividends on our common stock so any returns will be limited to the value of our stock.

We have never declared or paid any cash dividend on our common stock. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will therefore be limited to the appreciation of their stock.

Our principal stockholders and management own a significant percentage of our stock and are able to exert significant control over matters subject to stockholder approval.

Our executive officers and directors, combined with our stockholders who own more than 5% of our outstanding capital stock, beneficially own shares representing a significant percentage of our common stock. Therefore, these stockholders have the ability to influence us through this ownership position. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

Future sales and issuances of our common stock or rights to purchase common stock could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

We expect that significant additional capital may be needed in the future to continue our planned operations, including conducting clinical trials, commercialization efforts, expanded research and development activities and costs associated with operating a public company, including costs resulting from our no longer qualifying as an emerging growth company and a smaller reporting company and becoming a large accelerated filer. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time, including through our automatic shelf registration statement on Form S-3 filed with the SEC in September 2025. For example, in November 2023, we entered into the 2023 Sales Agreement, pursuant to which we may elect to issue and sell, from time to time, shares of common stock having an aggregate offering price of up to $200.0 million through the sales agents. The remaining capacity under the 2023 Sales Agreement was $200.0 million as of September 30, 2025. Further, in connection with and immediately prior to the closing of the Bile Acid Portfolio Acquisition, we completed a private placement of our common stock, pursuant to which we issued 8,000,000 shares of our common stock, and we filed a registration statement registering 7,937,448 of these shares for resale. If these additional shares of common stock are resold, or if it is perceived that they will be resold, in the public market, the trading price of our common stock could decline. Subject to the limitations on our ability to sell common stock described above, if we sell common stock, convertible securities or other equity securities, investors may be materially diluted by subsequent sales. Such sales may also result in material dilution to our existing stockholders, including noteholders who have received shares of our common stock upon conversion of their notes, and new investors could gain rights, preferences and privileges senior to the holders of our common stock.

Pursuant to our 2019 Equity Incentive Plan (“2019 Plan”), our management is authorized to grant equity incentive awards to our employees, directors and consultants. We also maintain a 2019 Employee Stock Purchase Plan (“ESPP”) pursuant to which our management is authorized to grant options to purchase shares of our common stock to our employees. In addition, pursuant to our 2020 Inducement Plan, our board of directors, or a committee thereof, is authorized to grant inducement awards to new hires as a material inducement to their employment with us.

Additionally, the number of shares of our common stock reserved for issuance under our 2019 Plan is subject to an automatic increase on January 1 of each year through and including January 1, 2029, by 5.0% of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by our board of directors. The number of shares of our common stock reserved for issuance under our ESPP is subject to an automatic increase on January 1 of each year through and including January 1, 2029, by the lesser of (i) 1.0% of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, and (ii) 1,500,000 shares of common stock. Unless our board of directors elects not to increase the number of shares available for future grant each year, our stockholders may experience additional dilution, which could cause our stock price to fall. Shares of common stock that are either subject to outstanding options or reserved for future issuance under our employee benefit plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, Rule 144 and Rule 701 under the Securities Act of 1933, as amended (the “Securities Act”). If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

Further, certain holders of our common stock are entitled to rights with respect to the registration of their shares under the Securities Act. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares held by affiliates, as defined in Rule 144 under the

Securities Act. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

Our business could be negatively affected as a result of actions by activist stockholders, and such activism could impact the trading value of our securities.

Stockholders may, from time to time, engage in proxy solicitations or advance stockholder proposals, or otherwise attempt to effect changes and assert influence on our board of directors and management. Activist campaigns that contest or conflict with our strategic direction or seek changes in the composition of our board of directors could have an adverse effect on our operating results and financial condition. A proxy contest would require us to incur significant legal and advisory fees, proxy solicitation expenses and administrative and associated costs and require significant time and attention by our board of directors and management, diverting their attention from the pursuit of our business strategy. Any perceived uncertainties as to our future direction and control, our ability to execute on our strategy, or changes to the composition of our board of directors or senior management team arising from a proxy contest could lead to the perception of a change in the direction of our business or instability which may result in the loss of potential business opportunities, make it more difficult to pursue our strategic initiatives, or limit our ability to attract and retain qualified personnel and business partners, any of which could adversely affect our business and operating results. If individuals are ultimately elected to our board of directors with a specific agenda, it may adversely affect our ability to effectively implement our business strategy and create additional value for our stockholders. We may choose to initiate, or may become subject to, litigation as a result of the proxy contest or matters arising from the proxy contest, which would serve as a further distraction to our board of directors and management and would require us to incur significant additional costs. In addition, actions such as those described above could cause significant fluctuations in our stock price based upon temporary or speculative market perceptions or other factors that do not necessarily reflect the underlying fundamentals and prospects of our business.

Our failure to meet Nasdaq's continued listing requirements could result in a delisting of our common stock.

If we fail to satisfy the continued listing requirements of Nasdaq, such as the corporate governance requirements or the minimum closing bid price requirement, Nasdaq may take steps to delist our common stock. Such a delisting would likely have a negative effect on the price of our common stock and would impair your ability to sell or purchase our common stock when you wish to do so. In the event of an actual or threatened delisting, we may take actions in an attempt to restore compliance with listing requirements, but any such actions may not allow our common stock to become listed again, stabilize the market price or improve the liquidity of our common stock, prevent our common stock from dropping below the Nasdaq minimum bid price requirement or prevent future non-compliance with the listing requirements of Nasdaq.

Anti-takeover provisions under our charter documents and Delaware law could delay or prevent a change of control, which could limit the market price of our common stock and may prevent or frustrate attempts by our stockholders to replace or remove our current management.

Our amended and restated certificate of incorporation and amended and restated bylaws contain provisions that could delay or prevent a change of control of our company or changes in our board of directors that our stockholders might consider favorable. Some of these provisions include:

- a board of directors divided into three classes serving staggered three-year terms, such that not all members of the board will be elected at one time;
- a prohibition on stockholder action through written consent, which requires that all stockholder actions be taken at a meeting of our stockholders;
- a requirement that special meetings of stockholders be called only by the chairman of the board of directors, the chief executive officer or by a majority of the total number of authorized directors;
- advance notice requirements for stockholder proposals and nominations for election to our board of directors;
- a requirement that no member of our board of directors may be removed from office by our stockholders except for cause and, in addition to any other vote required by law, upon the approval of not less than two-thirds of all outstanding shares of our voting stock then entitled to vote in the election of directors;
- a requirement of approval of not less than two-thirds of all outstanding shares of our voting stock then entitled to vote in the election of directors to amend our amended and restated bylaws by stockholder action or to amend specific provisions of our amended and restated certificate of incorporation; and

- the authority of the board of directors to issue preferred stock on terms determined by the board of directors without stockholder approval and which preferred stock may include rights superior to the rights of the holders of common stock.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the General Corporation Law of the State of Delaware ("DGCL"), which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These anti-takeover provisions and other provisions in our amended and restated certificate of incorporation and amended and restated bylaws could make it more difficult for stockholders or potential acquirors to obtain control of our board of directors or initiate actions that are opposed by the then-current board of directors and could also delay or impede a merger, tender offer or proxy contest involving our company. These provisions could also discourage proxy contests and make it more difficult for you and other stockholders to elect directors of your choosing or cause us to take other corporate actions you desire. Any delay or prevention of a change of control transaction or changes in our board of directors could cause the market price of our common stock to decline.

In addition, certain provisions in the Notes and the related Indenture could make a third party's attempt to acquire us more difficult or expensive. For example, if a takeover constitutes a fundamental change under our Indenture, then noteholders will have the right to require us to repurchase their Notes for cash. In addition, if a takeover constitutes a make-whole fundamental change under our Indenture, then we may be required to temporarily increase the conversion rate. In either case, and in other cases, our obligations under the Notes and the Indenture could increase the cost of acquiring us or otherwise discourage a third party from acquiring us or removing incumbent management, including in a transaction that noteholders or holders of our common stock may view as favorable.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that the Court of Chancery of the State of Delaware and the federal district courts of the U.S. will be the exclusive forums for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: (i) any derivative action or proceeding brought on our behalf; (ii) any action or proceeding asserting a claim of breach of a fiduciary duty owed by any of our current or former directors, officers or other employees to us or our stockholders; (iii) any action or proceeding asserting a claim against us or any of our current or former directors, officers or other employees, arising out of or pursuant to any provision of the DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws; (iv) any action or proceeding to interpret, apply, enforce or determine the validity of our amended and restated certificate of incorporation or our amended and restated bylaws; (v) any action or proceeding as to which the DGCL confers jurisdiction to the Court of Chancery of the State of Delaware; and (vi) any action asserting a claim against us or any of our directors, officers or other employees governed by the internal affairs doctrine, in all cases to the fullest extent permitted by law and subject to the court's having personal jurisdiction over the indispensable parties named as defendants. These provisions would not apply to suits brought to enforce a duty or liability created by the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or any other claim for which the federal courts have exclusive jurisdiction. Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our amended and restated certificate of incorporation and amended and restated bylaws further provides that the federal district courts of the U.S. will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act. While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our amended and restated certificate of incorporation and amended and restated bylaws. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions.

These exclusive-forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees and may discourage these types of lawsuits. If a court were to find either exclusive-forum provision in our amended and restated certificate of incorporation or amended and restated bylaws to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving the dispute in other jurisdictions, all of which could seriously harm our business.

General Risk Factors

Unfavorable geopolitical and macroeconomic developments could adversely affect our business, financial condition or results of operations.

Our business could be adversely affected by conditions in the U.S. and global economies, the U.S. and global financial markets and adverse geopolitical and macroeconomic developments, including potential future disruptions in access to bank deposits or lending commitments due to bank failures, tariffs and trade tensions, the ongoing shutdown of the federal government and the resulting effects on its regulatory agencies, geopolitical tensions and military conflicts, such as the ongoing conflicts between Ukraine and Russia and in the Middle East, and increasing tensions between the U.S. and China. The effects caused by these factors could be exacerbated by any related political or economic responses and counter-responses or otherwise by various global actors or the general effect on the global economy and supply chain. General business and economic conditions that could affect our business, financial condition or results of operations include fluctuations in economic growth, inflation and interest rates, debt and equity capital markets, liquidity of the global financial markets, the availability and cost of credit, investor and consumer confidence, and the strength of the economies in which we, our manufacturers, suppliers and other collaborators operate. A weak or declining global economy could also strain our suppliers and manufacturers, possibly resulting in supply disruption. 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 business.

If our information technology systems, or those used by our CMOs, CROs, commercial vendors or other contractors, consultants or third parties with whom we work, or our data are or were compromised, we could experience material adverse consequences, including but not limited to regulatory investigation, actions, litigation, fines and penalties, disruptions of our business operations, reputation harm, loss of revenue or profits, and other adverse consequences.

In the course of our business, we and the third parties with whom we work, process proprietary, confidential and sensitive information, including personal data (such as health-related data), intellectual property and trade secrets (collectively, sensitive information).

The sensitive information processed and stored in our technology systems, and those of our research collaborators, CROs, contractors, consultants and other third parties with whom we work, may be vulnerable to cyberattacks, malicious internet-based activity, online and offline fraud and other similar activities. These threats are prevalent and continue to rise, are increasingly difficult to detect, and come from a variety of sources, including traditional computer "hackers," threat actors, "hacktivists," organized criminal threat actors, personnel (such as through theft or misuse), sophisticated nation states, and nation-state-supported actors. Some actors now engage and are expected to continue to engage in cyberattacks, including without limitation nation-state actors for geopolitical reasons and in conjunction with military conflicts and defense activities. During times of war and other major conflicts, we and the third parties with whom we work may be vulnerable to a heightened risk of these attacks, including cyberattacks that could materially disrupt our systems and operations, supply chain, and ability to produce, sell and distribute our goods and services. We and the third parties with whom we work may be subject to a variety of threats, including but not limited to errors or malfeasance by our personnel or the personnel of the third parties, malware (including as a result of advanced persistent threat intrusions), malicious code (such as viruses and worms), software vulnerabilities, hacking, denial of service attacks, credential stuffing, social engineering (including through deep fakes, which may be increasingly more difficult to identify as fake, and phishing attacks), ransomware, supply-chain attacks, server malfunctions, software or hardware failure, loss of data or other information technology assets, adware, telecommunications failures, attacks enhanced or facilitated by artificial intelligence (AI) and other similar threats. Threat actors are continuing to develop and use more sophisticated tools and techniques (including AI) that are specifically designed to circumvent security controls, evade detection, and obfuscate forensic evidence, making it more difficult for us to identify, investigate and recover from incidents. Ransomware attacks, including by organized criminal threat actors, nation-states, and nation-state-supported actors, are becoming increasingly prevalent and severe and can lead to significant interruptions in our operations, loss of data and income, reputational harm, and diversion of funds. Extortion payments may alleviate the negative impact of a ransomware attack, but we may be unwilling or unable to make such payments due to, for example, applicable laws or regulations prohibiting such payments. Remote work has increased risks to our information technology systems and data, as our employees utilize network connections, computers and devices outside our premises or network, including working at home, while in transit and in public locations.

Future or past business transactions (such as acquisitions or integrations) could also expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. Furthermore, we may discover security issues that were not found during due

diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

We work with third parties and technologies that operate critical business systems to process sensitive information in a variety of contexts, including, without limitation, third-party providers of cloud-based infrastructure, encryption and authentication technology, employee communications, and other functions. Likewise, we work with third-party research institution collaborators, CMOs, CROs, other contractors and consultants for many aspects of our business, including research and development activities and manufacturing of our approved medicines and our product candidates, and similar events relating to their computer systems or data could also have a material adverse effect on our business. Our ability to monitor these third parties' information security practices is limited, and these third parties may not have adequate information security measures in place. If the third parties with whom we work experience a security incident or other interruption, we could experience adverse consequences. While we may be entitled to damages if the third parties with whom we work fail to satisfy their privacy or security-related obligations to us, any award may be insufficient to cover our damages, or we may be unable to recover such award. Similarly, supply-chain attacks have increased in frequency and severity, and we cannot guarantee that third parties and infrastructure in our supply chain or the supply chains of the third parties with whom we work have not been compromised.

While we have implemented information security measures designed to protect against security incidents, we cannot assure you that our (or the third parties with whom we work) security measures will be effective. It may be difficult and/or costly to detect, investigate, mitigate, contain, and remediate a security incident. Our efforts to detect, investigate, mitigate, contain, and remediate a security incident may not be successful. Actions taken by us or the third parties with whom we work to detect, investigate, mitigate, contain, and remediate a security incident could result in outages, data losses, and disruptions of our business. Threat actors may also gain access to other networks and systems after a compromise of our networks and systems. We take steps designed to detect, mitigate and remediate vulnerabilities in our information systems (such as our hardware and/or software, including that of third parties with whom we work). We have not and may not in the future, however, be able to detect and remediate all such vulnerabilities, including on a timely basis. Further, we have (and may in the future) experienced delays in developing and deploying remedial measures and patches designed to address identified vulnerabilities. Vulnerabilities could be exploited and result in a security incident.

Certain of the previously identified or similar threats have in the past and could in the future cause a security incident or other interruption that could result in unauthorized, unlawful, or accidental acquisition, modification, destruction, loss, alteration, encryption, disclosure of, or access to our sensitive information or our information technology systems, or those of the third parties with whom we work. For example, in February through March of 2024, along with many others in our industry, we became aware of a security incident at Change Healthcare which impacted the ability of patient claims to be adjudicated and patient prescriptions to be filled. Our internal team had to work closely with our specialty pharmacy and logistic providers to assess and resolve the matter. A security incident or other interruption could disrupt our ability (and that of third parties with whom we work) to provide our approved medicines and services.

We may expend significant resources or modify our business activities (including our clinical trial activities) to try to protect against security incidents. Certain data privacy and security obligations require us to implement and maintain specific security measures, industry-standard or reasonable security measures to protect our information technology systems and sensitive information. Applicable data privacy and security obligations may require us, or we may choose, to notify relevant stakeholders, including affected individuals, customers, regulators, and investors, of security incidents, or to take other actions, such as providing credit monitoring and identity theft protection services. Such disclosures and compliance with applicable requirements are costly, and the disclosure or the failure to comply with such requirements could lead to adverse consequences. If a material security incident was to occur, or we (or a third party with whom we work) are perceived to have experienced such an event, we may experience adverse consequences. These consequences may include: government enforcement actions (for example, investigations, fines, penalties, audits, and inspections), additional reporting requirements and/or oversight, restrictions on processing sensitive information (including personal data), litigation (including class claims), indemnification obligations, negative publicity, reputational harm, monetary fund diversions, interruptions in our operations (including availability of data), financial loss, and other similar harms. Security incidents and attendant consequences may negatively impact our ability to grow and operate our business. More specifically, for example, the loss of clinical trial data from completed, ongoing or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Security incidents and any unauthorized access or disclosure of our sensitive information could also compromise our intellectual property and patent portfolio, expose sensitive business information, expose the personal data of our employees, require us to incur significant remediation costs, disrupt key business operations and divert attention of management and key information technology resources.

We cannot be sure that our insurance coverage will be adequate or sufficient to protect us from or to mitigate liabilities arising out of our privacy and cybersecurity practices, that such coverage will continue to be available to us on

commercially reasonable terms, or at all, or that such coverage will pay future claims. The successful assertion of one or more large claims against us that exceed available insurance coverage, or the occurrence of changes in our insurance policies, including premium increases or the imposition of large deductible or co-insurance requirements, could adversely affect our reputation, business, financial condition and results of operations. Additionally, our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our data privacy and security obligations.

In addition to experiencing a security incident, third parties may gather, collect, or infer sensitive information about us from public sources, data brokers, or other means that reveals competitively sensitive details about our organization and could be used to undermine our competitive advantage or market position.

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

We are subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations, and various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls, and anti-corruption and anti-money laundering laws and regulations, including the FCPA, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, clinical research organizations, contractors and other collaborators and partners from authorizing, promising, offering, providing, soliciting or receiving, directly or indirectly, improper payments or anything else of value to recipients in the public or private sector. We may engage third parties for clinical trials outside of the U.S., to sell our approved medicines and any then-approved product internationally once we enter a commercialization phase, and/or to obtain necessary permits, licenses, patent registrations and other regulatory approvals. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities and other organizations. We can be held liable for the corrupt or other illegal activities of our employees, agents, clinical research organizations, contractors and other collaborators and partners, even if we do not explicitly authorize or have actual knowledge of such activities. Any violations of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm and other consequences.

If we or our third-party manufacturers use hazardous and biological materials in a manner that causes injury or violates applicable law, we may be liable for damages.

Our research and development activities involve the controlled use of potentially hazardous substances, including chemical and biological materials, by our third-party manufacturers. Our manufacturers are subject to federal, state and local laws and regulations in the U.S. governing the use, manufacture, storage, handling and disposal of medical, radioactive and hazardous materials. Although we believe that our manufacturers' procedures for using, handling, storing and disposing of these materials comply with legally prescribed standards, we cannot completely eliminate the risk of contamination or injury resulting from medical, radioactive or hazardous materials. As a result of any such contamination or injury, we may incur liability or local, city, state or federal authorities may curtail the use of these materials and interrupt our business operations. In the event of an accident, we could be held liable for damages or penalized with fines, and the liability could exceed our resources. We do not have any insurance for liabilities arising from medical radioactive or hazardous materials. Compliance with applicable environmental laws and regulations is expensive, and current or future environmental regulations may impair our research, development and production efforts, which could harm our business, prospects, financial condition or results of operations.

We have identified material weaknesses in our internal control over financial reporting in the past. If we identify additional material weaknesses in the future or otherwise fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired, which could adversely impact our investors' confidence in our financial reports and our stock price could be adversely affected.

We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act and the rules and regulations of Nasdaq. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal controls over financial reporting. Each fiscal year, we must perform system and process evaluation and testing of our internal controls over financial reporting to allow management to report on the effectiveness of our internal controls over financial reporting in our Form 10-K filing for that year, as required by Section 404 of the

Sarbanes-Oxley Act. This requires that we incur substantial professional fees and internal costs related to our accounting and finance functions and that we expend significant management efforts.

In connection with the audit of our consolidated financial statements as of and for the year ended December 31, 2023, management identified material weaknesses in the design of controls and level of evidence retained over the existence and valuation of inventory, including the controls over existence of inventory located at third parties and the net realizable value assessment of on-hand inventory and future purchases under firm commitments, and over the precision of management review controls and the sufficiency of control evidence related to prospective financial information used to determine the fair value of acquired developed technology. A material weakness is a deficiency, or combination of deficiencies, in internal control over financial reporting such that there is a reasonable possibility that a material misstatement of our annual or interim consolidated financial statements will not be prevented or detected on a timely basis.

Although we have been and are taking steps to improve our internal control over financial reporting and remediated these material weaknesses, the measures we have taken to date may not be sufficient to avoid potential future material weaknesses. Additionally, a control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we identify new material weaknesses in our internal control over financial reporting, if we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner, if we are unable to maintain proper and effective internal controls, or if our independent registered public accounting firm is unable to express an opinion that our internal control over financial reporting is effective in future periods, we may not be able to produce timely and accurate financial statements and investors may lose confidence in the accuracy and completeness of our financial reports. If that were to happen, the market price of our stock could decline and we could be subject to sanctions or investigations by Nasdaq, the SEC or comparable foreign regulatory authorities.

We have incurred and will continue to incur significant increased costs to comply with changing laws, rules, regulations and standards relating to various aspects of our business, including corporate governance, workforce initiatives and public disclosure, and failure to comply with such laws, rules, regulations and standards could adversely affect our business.

We have incurred and will continue to incur significant legal, accounting and other expenses to comply with changing laws, rules, regulations and standards relating to various aspects of our business, including corporate governance, workforce initiatives and public disclosure. Sources of these changing laws, rules, regulations and standards include new SEC regulations, Nasdaq rules and executive orders, which are creating uncertainty for companies such as ours. These laws, rules, regulations and standards are subject to varying interpretations in some cases due to their lack of specificity, and as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies, which could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure, policies and governance practices. For example, we are subject to the reporting requirements of the Exchange Act, which require, among other things, that we file with the SEC annual, quarterly and current reports with respect to our business and financial condition. In addition, the Sarbanes-Oxley Act, as well as rules subsequently adopted by the SEC and Nasdaq to implement provisions of the Sarbanes-Oxley Act, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and changes in corporate governance practices. Further, in July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act (the "Dodd-Frank Act") was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as "say on pay" and proxy access. Legislation permits emerging growth companies to implement many of these requirements over a longer period. As of June 30, 2023, the market value of our common stock held by non-affiliates exceeded $700.0 million. Consequently, we are no longer an emerging growth company or a smaller reporting company, and we are no longer able to avail ourselves of certain transition rules for companies that recently transitioned out of being a smaller reporting company. As a result, we are subject to certain disclosure and compliance requirements that apply to other public companies but did not previously apply to us due to our status as an emerging growth company or a smaller reporting company (or due to our transitioning out of being a smaller reporting company, which transition rules entitled us to certain scaled disclosure requirements) and expect to incur additional legal and financial compliance costs as a result. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new laws, rules, regulations and standards. If we fail, or are perceived to fail, to comply with these laws, rules, regulations and standards, our reputation may be harmed and we might be subject to litigation, sanctions, investigations or other regulatory proceedings by regulatory authorities, such as the SEC. Any such action could adversely affect our financial results and the market price of our common stock.

We expect the laws, rules, regulations and standards applicable to companies, especially public companies, to continue to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. If these requirements divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition and results of operations. The increased costs will decrease our net income or increase our consolidated net loss and may require us to reduce costs in other areas of our business or increase the prices of our approved medicines and any then-approved product or services. For example, we expect these laws, rules, regulations and standards to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain the same or similar coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

If securities or industry analysts publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our common stock depends 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 covers us no longer covers us, downgrades our stock or publishes inaccurate or unfavorable research about our business, our stock price may decline. If one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

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

Recent Sales of Unregistered Securities

There were no sales of equity securities during the period covered by this report that were not registered under the Securities Act and were not previously reported in a Current Report on Form 8-K filed by the Company.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers

None.

Item 3. Defaults Upon Senior Securities.

Not applicable.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

During our last fiscal quarter, our directors and officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted or terminated the contracts, instructions or written plans for the purchase or sale of our securities set forth in the table below.

			Type of Trading Arrangement			
Name and Position	**Action**	**Adoption/ Termination Date**	**Rule 10b5-1***	**Non-Rule 10b5-1****	**Total Shares of Common Stock to be Sold**	**Expiration Date**
Michael Grey, Director	Adopted	August 22, 2025	X		Up to 150,000	June 30, 2027
Saira Ramasastry, Director	Adopted	September 18, 2025	X		Up to 20,977	September 18, 2026
* Contract, instruction or written plan intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) under the Exchange Act.						
** “Non-Rule 10b5-1 trading arrangement” as defined in Item 408(c) of Regulation S-K under the Exchange Act.						

Item 6. Exhibits.

Exhibit Number	Description
2.1+¥	Asset Purchase Agreement, dated July 16, 2023, by and between Mirum Pharmaceuticals, Inc. and Travere Therapeutics, Inc. (incorporated by reference to Exhibit 2.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on July 17, 2023).
3.1	Amended and Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on July 25, 2019).
3.2	Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed with the SEC on July 25, 2019).
10.1	Mirum Pharmaceuticals, Inc. 2020 Inducement Plan, as amended September 4, 2025 (incorporated by reference to Exhibit 99.1 to the Registrant's Registration Statement on Form S-8 (File No. 333-290137), filed with the SEC on September 9, 2025).
31.1*	Certification of Principal Executive Officer pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Financial Officer pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1*#	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2*#	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS*	Inline XBRL Instance Document
101.SCH*	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents
104*	Cover Page Interactive Data File (embedded within the Inline XBRL document)

* Filed herewith.

+ Pursuant to Item 601(b)(10) of Regulation S-K, as applicable, certain portions of this exhibit have been omitted (indicated by "[*]") because the Registrant has determined that the information is not material and is the type that the Registrant treats as private or confidential.

¥ Schedules have been omitted pursuant to Item 601(a)(5) of Regulation S-K. The registrant undertakes to furnish supplemental copies of any of the omitted schedules upon request by the SEC.

The information in Exhibits 32.1 and 32.2 shall not be deemed "filed" for purposes of Section 18 of the Exchange Act, or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act or the Exchange Act (including this Quarterly Report on Form 10-Q), unless the Registrant specifically incorporates the foregoing information into those documents by reference.

SIGNATURES

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

Mirum Pharmaceuticals, Inc.

Date: November 4, 2025 By: /s/ Christopher Peetz

Christopher Peetz
Chief Executive Officer
(Principal Executive Officer)

Mirum Pharmaceuticals, Inc.

Date: November 4, 2025 By: /s/ Eric Bjerkholt

Eric Bjerkholt
Chief Financial Officer
(Principal Financial Officer)

Exhibit 31.1

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Christopher Peetz, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: November 4, 2025

By: /s/ Christopher Peetz

Christopher Peetz
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934,
AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Eric Bjerkholt, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: November 4, 2025

By: /s/ Eric Bjerkholt

Eric Bjerkholt
Chief Financial Officer
(Principal Financial 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 Mirum Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the quarter ended September 30, 2025, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

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

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

Date: November 4, 2025

By: /s/ Christopher Peetz

Christopher Peetz
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 Mirum Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the quarter ended September 30, 2025, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

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

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

Date: November 4, 2025

By: /s/ Eric Bjerkholt

Eric Bjerkholt
Chief Financial Officer
(Principal Financial Officer)