

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 June 30, 2023

OR

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

For the transition period from _____ to _____

Commission File Number 001-38981

Mirum Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware

(State or other jurisdiction of
incorporation or organization)

950 Tower Lane, Suite 1050, Foster City, California

(Address of principal executive offices)

83-1281555

(I.R.S. Employer
Identification No.)

94404

(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 July 28, 2023 was 38,297,975.

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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 I, Item 1A, “Risk Factors” in this Quarterly Report on Form 10-Q.

- LIVMARLI® (maralixibat) oral solution (“Livmarli”) is currently our only U.S. Food and Drug Administration (“FDA”) and European Medicines Agency (“EMA”) approved product and the success of our business depends, in part, on our ability to market and sell Livmarli 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 Livmarli, we may not be able to generate viable Livmarli revenues.
- Livmarli 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 product 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 not complete the Asset Purchase (defined below) within the time frame we anticipated or at all.
- We may fail to realize all of the anticipated benefits of the Asset Purchase (defined below) 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 will need substantial additional financing to continue our commercialization efforts for Livmarli, develop our product candidates and implement our operating plans. If we fail to obtain additional financing, we may be forced to delay, reduce or eliminate our product development programs or commercialization efforts.
- If we are unable to obtain and maintain sufficient intellectual property protection for Livmarli 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 Livmarli 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
(In thousands, except share and per share data)

	June 30, 2023	December 31, 2022
	(Unaudited)	(Note 2)
Assets		
Current assets:		
Cash and cash equivalents	\$ 297,228	\$ 28,003
Short-term investments	32,726	123,716
Accounts receivable	35,326	23,994
Inventory	8,509	5,565
Prepaid expenses and other current assets	9,729	8,947
Total current assets	383,518	190,225
Restricted cash equivalents	—	100,000
Property and equipment, net	866	914
Operating lease right-of-use assets	1,614	1,431
Intangible assets, net	56,437	58,954
Other assets	1,153	1,382
Total assets	\$ 443,588	\$ 352,906
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 14,446	\$ 8,690
Accrued expenses	42,693	54,018
Operating lease liabilities	1,064	931
Derivative liability	—	1,090
Total current liabilities	58,203	64,729
Revenue interest liability, net	—	140,351
Operating lease liabilities, noncurrent	1,154	1,257
Convertible notes payable, net	305,611	—
Other liabilities	10	4,532
Total liabilities	364,978	210,869
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, \$0.0001 par value; 10,000,000 shares authorized, and no shares issued and outstanding as of June 30, 2023 and December 31, 2022	—	—
Common stock, \$0.0001 par value; 200,000,000 shares authorized; 38,222,058 shares issued and outstanding as of June 30, 2023; and 36,956,345 shares issued and outstanding as of December 31, 2022	4	4
Additional paid-in capital	575,522	535,074
Accumulated deficit	(496,992)	(392,824)
Accumulated other comprehensive income (loss)	76	(217)
Total stockholders' equity	78,610	142,037
Total liabilities and stockholders' equity	\$ 443,588	\$ 352,906

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 June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Revenue:				
Product sales, net	\$ 32,497	\$ 17,484	\$ 61,595	\$ 28,376
License revenue	5,000	—	7,500	2,000
Total revenue	37,497	17,484	69,095	30,376
Operating expenses:				
Cost of sales	6,812	2,524	11,791	4,948
Research and development	22,009	25,432	45,557	49,520
Selling, general and administrative	32,949	20,969	63,168	40,085
Total operating expenses	61,770	48,925	120,516	94,553
Loss from operations	(24,273)	(31,441)	(51,421)	(64,177)
Other income (expense):				
Interest income	3,627	293	5,899	362
Interest expense	(3,726)	(3,875)	(7,968)	(7,649)
Change in fair value of derivative liability	—	232	—	232
Loss from termination of revenue interest purchase agreement	(49,076)	—	(49,076)	—
Other (expense) income, net	(274)	1,299	(1,085)	1,145
Net loss before provision for (benefit from) income taxes	(73,722)	(33,492)	(103,651)	(70,087)
Provision for (benefit from) income taxes	316	(6,570)	517	(6,559)
Net loss	\$ (74,038)	\$ (26,922)	\$ (104,168)	\$ (63,528)
Net loss per share, basic and diluted	\$ (1.94)	\$ (0.84)	\$ (2.75)	\$ (2.00)
Weighted-average shares of common stock outstanding, basic	38,107,334	32,164,174	37,892,513	31,732,596
Weighted-average shares of common stock outstanding, diluted	38,107,334	32,179,171	37,892,513	31,740,136

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

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

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2023</u>	<u>2022</u>	<u>2023</u>	<u>2022</u>
Net loss	\$ (74,038)	\$ (26,922)	\$ (104,168)	\$ (63,528)
Other comprehensive income (loss):				
Unrealized gain (loss) on available-for-sale investments	54	(22)	219	(115)
Cumulative translation adjustments	(114)	(49)	74	(52)
Comprehensive loss	<u>\$ (74,098)</u>	<u>\$ (26,993)</u>	<u>\$ (103,875)</u>	<u>\$ (63,695)</u>

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, 2022	36,956,345	\$ 4	\$ 535,074	\$ (392,824)	\$ (217)	\$ 142,037
Issuance of common stock in connection with equity award plans	197,703	—	1,390	—	—	1,390
Issuance of common stock in at-the-market offerings, net of issuance costs of \$518	658,206	—	14,480	—	—	14,480
Issuance of common stock in connection with achievement of Contingent Milestone	199,993	—	4,292	—	—	4,292
Stock-based compensation	—	—	8,728	—	—	8,728
Net loss	—	—	—	(30,130)	—	(30,130)
Other comprehensive income	—	—	—	—	353	353
Balance as of March 31, 2023	38,012,247	4	563,964	(422,954)	136	141,150
Issuance of common stock in connection with equity award plans	101,699	—	803	—	—	803
Issuance of common stock in connection with employee stock purchase plan	76,481	—	1,294	—	—	1,294
Issuance of common stock in connection with settlement of Indemnification Holdback liability	31,631	—	896	—	—	896
Stock-based compensation	—	—	8,565	—	—	8,565
Net loss	—	—	—	(74,038)	—	(74,038)
Other comprehensive loss	—	—	—	—	(60)	(60)
Balance as of June 30, 2023	<u>38,222,058</u>	<u>\$ 4</u>	<u>\$ 575,522</u>	<u>\$ (496,992)</u>	<u>\$ 76</u>	<u>\$ 78,610</u>

	Common Stock		Additional Paid-In Capital	Accumulated Deficit	Accumulated Other Comprehensive Loss	Total Stockholders' Equity
	Shares	Amount				
Balance as of December 31, 2021	30,582,596	\$ 3	\$ 377,403	\$ (257,159)	\$ (35)	\$ 120,212
Issuance of common stock in connection with equity award plans	100,951	—	1,477	—	—	1,477
Issuance of common stock in at-the-market offerings, net of issuance costs of \$601	995,897	—	17,384	—	—	17,384
Restricted common stock vested in the period	33,398	—	—	—	—	—
Stock-based compensation	—	—	6,561	—	—	6,561
Net loss	—	—	—	(36,606)	—	(36,606)
Other comprehensive loss	—	—	—	—	(96)	(96)
Balance as of March 31, 2022	31,712,842	3	402,825	(293,765)	(131)	108,932
Issuance of common stock in connection with asset acquisition	609,305	—	15,585	—	—	15,585
Issuance of common stock in connection with equity award plans	92,593	—	1,408	—	—	1,408
Issuance of common stock in at-the-market offerings, net of issuance costs of \$184	165,018	—	3,905	—	—	3,905
Restricted common stock vested in the period	33,398	—	—	—	—	—
Issuance of common stock in connection with employee stock purchase plan	76,099	—	1,032	—	—	1,032
Stock-based compensation	—	—	6,818	—	—	6,818
Net loss	—	—	—	(26,922)	—	(26,922)
Other comprehensive loss	—	—	—	—	(71)	(71)
Balance as of June 30, 2022	<u>32,689,255</u>	<u>\$ 3</u>	<u>\$ 431,573</u>	<u>\$ (320,687)</u>	<u>\$ (202)</u>	<u>\$ 110,687</u>

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)

	Six Months Ended June 30,	
	2023	2022
Operating activities		
Net loss	\$ (104,168)	\$ (63,528)
Reconciliation of net loss to net cash used in operating activities:		
Stock-based compensation	16,918	13,379
Depreciation and amortization	2,673	941
Non-cash lease expense	290	207
Net accretion of discounts on investments	(1,462)	(103)
Non-cash interest expense related to the revenue interest liability	5,060	7,649
Amortization of debt discount and offering costs	307	—
Change in fair value of derivative liability	—	(232)
Change in fair value of contingent liabilities associated with an acquisition	667	(1,299)
Inventory reserves	665	—
Loss from termination of revenue interest purchase agreement	49,076	—
Deferred income taxes associated with an acquisition	—	(6,580)
Change in operating assets and liabilities:		
Accounts receivable	(11,332)	(10,723)
Prepaid and other current assets	(782)	1,493
Inventory	(1,767)	(1,247)
Other assets	229	(272)
Accounts payable, accrued expenses and other liabilities	7,964	819
Operating lease liabilities	(443)	(345)
Net cash used in operating activities	(36,105)	(59,841)
Investing activities		
Purchase of investments	(27,329)	(14,812)
Proceeds from maturities of investments	120,000	75,300
Purchase of property and equipment	(109)	(17)
Payments made for additions to intangible assets	(15,000)	—
Net cash provided by investing activities	77,562	60,471
Financing activities		
Proceeds from issuance of common stock in at-the-market offerings, net of issuance costs	14,480	21,289
Proceeds from the issuance of convertible notes, net	305,304	—
Proceeds from issuance of common stock pursuant to equity plans	3,487	3,917
Payments on revenue interest liability	(195,577)	(1,856)
Net cash provided by financing activities	127,694	23,350
Effect of exchange rate on cash, cash equivalents and restricted cash equivalents	74	(52)
Net increase in cash, cash equivalents and restricted cash equivalents	169,225	23,928
Cash, cash equivalents and restricted cash equivalents at beginning of period	128,003	131,340
Cash, cash equivalents and restricted cash equivalents at end of period	\$ 297,228	\$ 155,268
Supplemental disclosure of cash flow information:		
Operating cash flows paid for operating lease	\$ 527	\$ 438
Non-cash operating, investing and financing activities:		
Inventory purchases included in accrued liabilities	\$ 1,467	\$ 1,764
Right-of-use assets obtained in exchange for lease liability	\$ 473	\$ —
Stock-based compensation capitalized to inventory	\$ 375	\$ 177
Reclassification of Contingent Milestone liability to equity in connection with issuance of common stock upon achievement of Contingent Milestone	\$ 4,292	\$ —
Issuance of common stock in connection with settlement of Indemnification Holdback liability	\$ 896	\$ —
Issuance of common stock in exchange for acquired intangible assets	\$ —	\$ 15,585
Contingent milestone liability for common stock issuance for acquired intangible assets	\$ —	\$ 4,600
Indemnification Holdback liability in exchange for acquired intangible assets	\$ —	\$ 831
Transaction costs from acquisition in accrued liabilities	\$ —	\$ 176

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 focused on the identification, acquisition, development and commercialization of novel therapies for debilitating rare and orphan diseases.

The Company’s product LIVMARLI® (maralixibat) oral solution (“Livmarli”), is approved for the treatment of cholestatic pruritus in patients with Alagille syndrome (“ALGS”) three months of age and older in the United States and for the treatment of cholestatic pruritus in patients with ALGS two months of age and older in Europe.

The Company’s development pipeline consists of two clinical-stage product candidates, Livmarli and volixibat. The Company commenced significant operations in November 2018.

The Company views its operations and manages its business as one operating segment. The Company 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 June 30, 2023, the Company had an accumulated deficit of \$497.0 million and cash, cash equivalents and investments of \$330.0 million. The Company believes that its cash, cash equivalents and investments of \$330.0 million as of June 30, 2023, 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.

In April 2023, the Company completed a convertible notes offering, as further described in Note 10, with net proceeds of \$305.3 million, after deducting the initial purchasers’ discounts and commissions and offering expenses. From the net proceeds, \$192.7 million was used to repurchase all future revenue interests in connection with the Company’s Revenue Interest Purchase Agreement (“RIPA”) (Note 6). Upon repurchase and the termination of the RIPA, in accordance with its terms, the previously restricted cash equivalents of \$100.0 million were no longer restricted from use.

The unaudited condensed consolidated financial statements have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business. The unaudited condensed consolidated financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of this uncertainty. Management expects to continue to incur additional substantial losses in the foreseeable future as a result of the Company’s research and development activities.

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, 2022 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 significant 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, 2022, as filed with the SEC on March 8, 2023.

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 of assets, liabilities and expenses and the disclosure of contingent assets and liabilities

in the financial statements and accompanying notes. 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 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 six months ended June 30, 2023 reflect the Company's estimates of the impact of the geopolitical and macroeconomic environment, including the impact of inflation, bank failures, higher 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 accounting policies during the six months ended June 30, 2023, 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, unless indicated below.

Cash, Cash Equivalents and Restricted Cash Equivalents

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.

Restricted cash equivalents as of December 31, 2022 consisted of deposits placed in a segregated bank account as required under the terms of the Company's RIPA, as amended September 2021, with Mulholland SA LLC, an affiliate of Oberland Capital LLC, as agent for the purchasers party thereto (the "Purchasers"), and the Purchasers in connection with the sale of the Priority Review Voucher in December 2021. Upon repurchase and the termination of the RIPA in April 2023, in accordance with its terms, the previously restricted cash equivalents of \$100.0 million were no longer restricted from use.

The following table provides a reconciliation of cash, cash equivalents and restricted cash equivalents 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):

	<u>As of June 30,</u> <u>2023</u>	<u>As of December 31,</u> <u>2022</u>
Cash and cash equivalents	\$ 297,228	\$ 28,003
Restricted cash equivalents	—	100,000
Total cash, cash equivalents, and restricted cash equivalents	<u>\$ 297,228</u>	<u>\$ 128,003</u>

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 interest income (loss). 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 and/or commercial paper with short maturities. 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 this credit risk and continues to believe that this exposure is not significant.

The Company relies on a single distributor and a specialty pharmacy for all of the Company's sales of Livmarli in the United States as well as a single distributor for sales outside the United States.

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 June 30, 2023, the Company had two customers that individually accounted for approximately 19% and 11% of accounts receivable. As of December 31, 2022, the Company had one customer that accounted for approximately 23% of accounts receivable. For the three and six months ended June 30, 2023 and 2022, 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 also has 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 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 June 30, 2023. There was no bad debt expense for the three and six months ended June 30, 2023 and 2022.

Intangible Assets, Net

The Company accounts for asset acquisitions that do not meet the definition of a business using the cost accumulation method, whereby the cost of the acquisition, including certain transaction costs, is allocated to the asset (or assets) acquired on the basis of its (or their) relative fair value(s) on the measurement date. No goodwill is recognized in an asset acquisition.

Intangible assets are measured at their fair values as of the acquisition date or, in the case of commercial milestone payments, the date they become due. The evaluation of intangible assets includes assessing the amortization period for which the asset is expected to contribute to the future cash flows of the Company. Intangible assets with finite useful lives are amortized over their estimated useful lives, primarily on a straight-line basis when the Company is unable to reliably estimate the pattern of cash flow. The Company tests its finite lived intangible assets for impairment annually or if events or changes in circumstances indicate that it is more likely than not that the asset is impaired. If it is determined that the asset is impaired, the carrying value is written down to its estimated fair value, with the related impairment charge recognized in the unaudited condensed consolidated statements of operations in the period in which the impairment occurs. The Company has not recorded any impairments to its intangible assets for any of the periods presented.

The following table provides detail of the carrying amount of the Company's intangible assets (in thousands):

	June 30, 2023		
	Gross Carrying Value	Accumulated Amortization	Net Carrying Amount
Intangible asset - commercial milestone payments	\$ 34,000	\$ (2,288)	\$ 31,712
Intangible assets - Satiogen acquisition	28,107	(3,382)	24,725
Total intangible assets	<u>\$ 62,107</u>	<u>\$ (5,670)</u>	<u>\$ 56,437</u>

	December 31, 2022		
	Gross Carrying Value	Accumulated Amortization	Net Carrying Amount
Intangible asset - commercial milestone payments	\$ 34,000	\$ (1,333)	\$ 32,667
Intangible assets - Satiogen acquisition	28,107	(1,820)	26,287
Total intangible assets	<u>\$ 62,107</u>	<u>\$ (3,153)</u>	<u>\$ 58,954</u>

As of June 30, 2023, the remaining weighted-average amortization period of the Company's intangible assets was 12.8 years. The remaining weighted-average amortization periods of the commercial milestone payments and the Satiogen Pharmaceutical, Inc. ("Satiogen") acquisition intangible assets were 16.7 years and 7.9 years, respectively.

Amortization expense was \$1.3 million and \$2.5 million for the three and six months ended June 30, 2023, respectively, and \$0.5 million and \$0.8 million for the three and six months ended June 30, 2022, 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 June 30, 2023 (in thousands):

	Amount
2023 (remaining six months)	2,518
2024	5,035
2025	5,035
2026	5,035
2027	5,035
Thereafter	33,779
	<u>\$ 56,437</u>

Convertible Notes

The Company evaluates all conversion, repurchase and redemption features contained in a debt instrument to determine if there are any embedded features that require bifurcation as a derivative. The Company accounts for its convertible notes (see Note 10) as a long-term liability equal to the proceeds received from issuance, including any embedded conversion features, net of the unamortized debt discount and offering costs in the accompanying unaudited consolidated balance sheets. The debt issuance and offering costs are amortized over the contractual term of the convertible notes, using the effective interest method, as interest expense in the accompanying unaudited consolidated statements of operations.

Product Sales, Net

The Company recognizes product sales, net when the customer obtains control of our product, which occurs at a point in time, typically upon delivery of the Company's product to the customer.

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 Livmarli. 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 in the future 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 discounts and allowances 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 mix, 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. The liability for unpaid rebates is included in accrued expenses on the accompanying unaudited condensed consolidated balance sheets. To date, actual government rebates have not differed materially from the Company's estimates.

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 Livmarli

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. To date, actual other incentives have not differed materially from the Company's estimates.

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 refund liability in the period the related product revenue is recognized. To date, actual returns have not differed materially from the Company's estimates.

Net Loss Per Share

Basic net loss per share is computed by dividing net loss by the weighted-average shares of common stock outstanding for the period, without consideration for potentially dilutive securities. Diluted net loss per share is computed by dividing the net 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 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 because their effect would be anti-dilutive due to the Company's net loss. Basic and diluted net loss per share were the same for the three and six months ended June 30, 2023.

The following table sets for the computation of basic and diluted earnings per share for the three and six months ended June 30, 2022 (in thousands, except share and per share data):

	Three Months Ended June 30, 2022	Six Months Ended June 30, 2022
Numerator:		
Net loss, basic	\$ (26,922)	\$ (63,528)
Less: Change in fair value of Holdback Indemnification liability	199	199
Net loss, diluted	<u>\$ (27,121)</u>	<u>\$ (63,727)</u>
Denominator:		
Weighted-average shares of common stock outstanding, basic	32,164,174	31,732,596
Effect of dilutive securities:		
Weighted-average Holdback Indemnification shares issuable	14,997	7,540
Weighted-average shares of common stock outstanding, diluted	<u>32,179,171</u>	<u>31,740,136</u>
Net loss per share, basic	\$ (0.84)	\$ (2.00)
Net loss per share, diluted	\$ (0.84)	\$ (2.00)

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

	As of June 30, 2023	As of June 30, 2022
Options to purchase common stock and restricted stock units	10,647,891	8,897,778
Common stock issuable upon conversion of convertible notes	9,964,247	—
Employee stock purchase plan contingently issuable	22,207	25,562
Common stock subject to repurchase	—	55,668
Shares issuable as contingent consideration in connection with asset acquisition	—	199,993
Total	<u>20,634,345</u>	<u>9,179,001</u>

Recently Adopted Accounting Pronouncements

On January 1, 2023, the Company adopted Accounting Standards Update ("ASU") No. 2016-13, *Financial Instruments-Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments* ("ASU 2016-13"). ASU 2016-13 requires an entity to utilize a new impairment model that requires measurement and recognition of expected credit losses for most financial assets and certain other instruments, including but not limited to available-for-sale debt securities. Credit losses relating to available-for-sale debt

securities will be recorded through an allowance for credit losses rather than as a direct write-down to the security. The new guidance also modifies the impairment models for available-for-sale debt securities and for purchased financial assets with credit deterioration since their origination. In March 2020, the Financial Accounting Standards Board (“FASB”) issued ASU No. 2020-3, *Codification Improvements to Financial Instruments* which makes narrow-scope improvements to various financial instruments topics, including the new credit losses standard and clarifies the following areas (i) the contractual term of a net investment in a lease should be the contractual term used to measure expected credit losses; (ii) when an entity regains control of financial assets sold, an allowance for credit losses should be recorded. There was no impact on the accompanying unaudited condensed consolidated financial statements as of the adoption date, January 1, 2023.

Recent Accounting Pronouncements Not Yet Adopted

From time to time, new accounting pronouncements are issued by the 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 condensed consolidated financial statements and disclosures.

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):

	June 30, 2023			
	Level 1	Level 2	Level 3	Total
Financial assets:				
Money market funds	\$ 285,002	\$ —	\$ —	\$ 285,002
U.S. treasury bills	24,804	—	—	24,804
Commercial paper	—	2,930	—	2,930
U.S. government bonds	—	4,992	—	4,992
Total financial assets	<u>\$ 309,806</u>	<u>\$ 7,922</u>	<u>\$ —</u>	<u>\$ 317,728</u>
	December 31, 2022			
	Level 1	Level 2	Level 3	Total
Financial assets:				
Money market funds	\$ 124,227	\$ —	\$ —	\$ 124,227
U.S. treasury bills	4,975	—	—	4,975
Commercial paper	—	74,386	—	74,386
U.S. government bonds	—	44,354	—	44,354
Total financial assets	<u>\$ 129,202</u>	<u>\$ 118,740</u>	<u>\$ —</u>	<u>\$ 247,942</u>
Financial liabilities:				
Contingent milestone liability	\$ 3,900	\$ —	\$ —	\$ 3,900
Derivative liability	—	—	1,090	1,090
Indemnification holdback	—	—	617	617
Total financial liabilities	<u>\$ 3,900</u>	<u>\$ —</u>	<u>\$ 1,707</u>	<u>\$ 5,607</u>

The carrying amounts of certain financial instruments such as cash and cash equivalents, restricted cash equivalents, accounts receivable, prepaid expenses, other current assets, accounts payable and accrued expenses as of June 30, 2023 and December 31, 2022 approximate their related fair values due to the short-term maturities of these instruments.

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.

U.S. government bonds and commercial paper, are measured at fair value using Level 2 inputs. The Company reviews trading activity and pricing for these investments as of each measurement date.

The carrying amount of the revenue interest liability as of December 31, 2022 approximates its fair value and is based on the Company’s contractual repayment obligation to the Purchasers, based on the current estimates of future revenues, over the life of the RIPA.

The Contingent Milestone liability as of December 31, 2022 is considered a Level 1 input as the significant inputs were known and observable. The derivative liability and Indemnification Holdback liability (each as defined below) as of December 31, 2022 are each considered a Level 3 input based on the three-level hierarchy.

Derivative Liability

The debt pursuant to the RIPA (refer to Note 6 “Revenue Interest Purchase Agreement” for further information) contained an embedded derivative requiring bifurcation as a single compound derivative instrument. The Company estimated the fair value of the derivative liability using a “with-and-without” method. The “with-and-without” methodology involves valuing the whole instrument on an as-is basis and then valuing the instrument without the individual embedded derivative. The difference between the entire instrument with the embedded derivative compared to the instrument without the embedded derivative was the fair value of the derivative liability at December 31, 2022. The estimated probability and timing of underlying events triggering the exercisability of the put option contained within the RIPA, forecasted cash flows and the discount rate were significant unobservable inputs used to determine the estimated fair value of the entire instrument with the embedded derivative. As of December 31, 2022, the discount rate used for valuation of the derivative liability was 15.7%. There was no change in the fair value of the derivative liability during the three and six months ended June 30, 2023 prior to its extinguishment in connection with termination of the RIPA.

Indemnification Holdback

In May 2022, in connection with the acquisition of Satiogen (refer to Note 7 “Asset Acquisitions” for further information), the Company recorded at fair value liabilities related to the Company’s common stock issuable upon satisfaction of certain purchase price adjustments and indemnification obligations that may arise during the 12-month period following the asset acquisition date (“Indemnification Holdback”). The fair value of the Indemnification Holdback was classified within Level 3 of the fair value hierarchy and was estimated based upon the value of the Company’s common stock price. The fair value of the Indemnification Holdback was additionally determined based on management’s estimate of the probability of indemnification obligations being incurred during the one year following the acquisition date. The fair value of the Indemnification Holdback was initially measured on May 20, 2022, the date on which the Company completed the acquisition of Satiogen. The Company assessed the fair value of the Indemnification Holdback each reporting period until resolution of the related contingency and changes in fair value were recorded in other income (expense), net in the accompanying unaudited condensed consolidated statements of operations. During the three months ended June 30, 2023, the contingencies related to the Indemnification Holdback were resolved and the Company issued 31,631 shares of common stock.

The following table provides a summary of the changes in the estimated fair value of the Indemnification Holdback liability (in thousands):

	Indemnification Holdback Liability
Balance at December 31, 2022	\$ 617
Change in fair value	143
Balance at March 31, 2023	760
Change in fair value	136
Settlement of Indemnification Holdback liability	(896)
Balance at June 30, 2023	\$ —

The Indemnification Holdback is included in other liabilities on the accompanying unaudited condensed consolidated balance sheets as of December 31, 2022.

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):

June 30, 2023				
	Amortized Cost	Unrealized Gain	Unrealized Loss	Estimated Fair Value
Cash equivalents and investments:				
Money market funds	\$ 285,002	\$ —	\$ —	\$ 285,002
U.S. treasury bills	24,814	—	(10)	24,804
Commercial paper	2,930	—	—	2,930
U.S. government bonds	4,994	—	(2)	4,992
Total cash equivalents and investments	<u>\$ 317,740</u>	<u>\$ —</u>	<u>\$ (12)</u>	<u>\$ 317,728</u>
Classified as:				
Cash equivalents				\$ 285,002
Short-term investments				32,726
Total cash equivalents and investments				<u>\$ 317,728</u>

December 31, 2022				
	Amortized Cost	Unrealized Gain	Unrealized Loss	Estimated Fair Value
Cash equivalents and investments:				
Money market funds	\$ 124,227	\$ —	\$ —	\$ 124,227
U.S. treasury bills	4,980	—	(5)	4,975
Commercial paper	74,386	—	—	74,386
U.S. government bonds	44,579	—	(225)	44,354
Total cash equivalents and investments	<u>\$ 248,172</u>	<u>\$ —</u>	<u>\$ (230)</u>	<u>\$ 247,942</u>
Classified as:				
Cash equivalents				\$ 24,226
Cash equivalents - restricted				100,000
Short-term investments				123,716
Total cash equivalents, restricted cash equivalents and investments				<u>\$ 247,942</u>

As of June 30, 2023, the remaining contractual maturities of available-for-sale debt securities were less than 12 months. During the three and six months ended June 30, 2023 and 2022, 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):

	June 30, 2023	December 31, 2022
Work in progress	6,965	5,351
Finished goods	1,544	214
Total inventory	<u>\$ 8,509</u>	<u>\$ 5,565</u>

Accrued Expenses

Accrued expenses consist of the following (in thousands):

	June 30, 2023	December 31, 2022
Accrued compensation and related benefits	\$ 10,957	\$ 14,660
Accrued rebates payable	7,385	4,284
Accrued clinical trials	6,599	8,319
Accrued professional service fees	6,584	5,372
Accrued contract manufacturing and non-clinical costs	5,665	3,927
Accrued royalties payable	2,902	2,456
Accrued interest	2,601	—
Accrued milestone payments	—	15,000
Total accrued expenses	\$ 42,693	\$ 54,018

6. Revenue Interest Purchase Agreement

In December 2020, the Company entered into the RIPA, as amended in September 2021, with Mulholland SA LLC, an affiliate of Oberland Capital LLC, as agent for the Purchasers, and the Purchasers to obtain financing for the commercialization and further development of Livmarli and other working capital needs. Pursuant to the RIPA, the Company has received \$115.0 million consisting of an upfront payment of \$50.0 million in December 2020 and \$65.0 million in April 2021 associated with the acceptance for filing by the FDA of a New Drug Application for Livmarli for the treatment of cholestatic pruritus in patients with ALGS, less certain transaction expenses.

Under the RIPA, the Company was entitled to receive an additional \$35.0 million upon FDA approval of Livmarli, which it elected to forgo. The Company was also entitled to receive up to approximately \$50.0 million at the option of the Purchasers to finance in-licenses or other acquisitions on or prior to December 31, 2022, which the Company did not request.

As consideration for such payments, the Purchasers had the right to receive certain revenue interests (the “Revenue Interests”) from the Company based on annual product sales, net of Livmarli, in tiered payments (the “Revenue Interest Payments”) based on whether annual product sales, net are (i) less than or equal to \$350.0 million (“Tier 1”), (ii) exceeding \$350.0 million and less than or equal to \$1.1 billion (“Tier 2”), or (iii) exceeding \$1.1 billion (“Tier 3”). The Revenue Interest Payments were initially 9.75% (at Tier 1) and 2.0% (at Tier 2 and Tier 3) of such annual net sales.

Under the RIPA, the Company had an option (the “Call Option”) to terminate the RIPA and repurchase future Revenue Interests at any time upon advance written notice. Additionally, the Purchasers had an option (the “Put Option”) to terminate the RIPA and to require the Company to repurchase future Revenue Interests upon enumerated events such as a bankruptcy event, an uncured material breach, a material adverse effect or a change of control, or upon the 12th anniversary of the first payment made by Purchasers. If the Put Option is exercised prior to the first anniversary of the closing date by the Purchasers (except pursuant to a change of control), the required repurchase price is 120.0% of the Cumulative Purchaser Payments (minus all payments Company has made to the Purchasers in connection with the Revenue Interests). In all other cases, if the Put Option or the Call Option are exercised, the required repurchase price is 175.0% of the Cumulative Purchaser Payments (minus all payments the Company has made to the Purchasers in connection with the Revenue Interests), if such option is exercised prior to the third anniversary of the closing date, and 195.0% of the Cumulative Purchaser Payments (minus all payments Company has made to the Purchasers in connection with the Revenue Interests) if such option is exercised thereafter.

In addition, the RIPA contained various representations and warranties, information rights, non-financial and financial covenants, indemnification obligations and other provisions that are customary for a transaction of this nature. The Purchasers’ obligations to fund the scheduled installments were subject to certain customary conditions as set forth in the RIPA.

Concurrently with the RIPA, the Company entered into a Common Stock Purchase Agreement (“CSPA”) with certain affiliates of Oberland, pursuant to which the Company sold an aggregate of 509,164 shares of its common stock for an aggregate purchase price of \$10.0 million. The \$50.0 million upfront payment received pursuant to the RIPA and \$10.0 million received pursuant to the CSPA was allocated between the resulting financial instruments on a relative fair value basis, with \$49.2 million allocated to the debt under the RIPA and \$10.8 million allocated to the common stock issued under the CSPA.

The Put Option under the RIPA that was exercisable by Purchasers upon certain contingent events was determined to be an embedded derivative requiring bifurcation and separately accounted for as a single compound derivative instrument. The Company

recorded the initial fair value of the derivative liability of \$1.3 million as a debt discount, which was amortized to interest expense over the expected term of the debt using the effective interest method.

The Company imputed interest expense associated with the revenue interest liability using the effective interest rate method. The effective interest rate was calculated based on the rate that would enable the debt to be repaid in full over the anticipated life of the arrangement. The interest rate on this liability was variable during the term of the agreement depending on a number of factors, including the level of forecasted product sales, net. The Company evaluated the interest rate quarterly based on each period's product sales, net forecasts utilizing the prospective method. The Company recorded interest expense related to this arrangement of \$0.8 million and \$5.1 million for the three and six months ended June 30, 2023, respectively, and \$3.9 million and \$7.6 million for the three and six months ended June 30, 2022, respectively.

The Company incurred \$0.9 million of issuance costs in connection with the RIPA, which were amortized to interest expense over the estimated term of the debt.

Revenue Interest Payments made as a result of the Company's product sales, net reduce the revenue interest liability. During the six months ended June 30, 2023, the Company made payments of \$2.9 million in connection with the RIPA.

In April 2023, the Company exercised the Call Option and repurchased all future Revenue Interests. In connection with such repurchase, the Company made a payment of \$192.7 million, or 175% of the Cumulative Purchaser Payments (minus all payments that were made to the Purchasers in connection with the Revenue Interests.) As a result, the RIPA terminated in accordance with its terms.

The following table summarizes the revenue interest liability activity and the related derivative liability activity during the three and six months ended June 30, 2023 (in thousands):

	Revenue Interest Liability	Derivative Liability
Balance at December 31, 2022	\$ 140,351	\$ 1,090
Interest expense recognized	4,242	—
Revenue interest payments	(2,883)	—
Balance at March 31, 2023	\$ 141,710	\$ 1,090
Interest expense recognized	818	—
Revenue interest liability settlement	(192,694)	—
Loss (gain) from termination of revenue interest purchase agreement	50,166	(1,090)
Balance at June 30, 2023	\$ —	\$ —

The net loss from termination of RIPA of \$49.1 million, comprised of the \$50.2 million loss related to the settlement of the revenue interest liability and the \$1.1 million gain on the derecognition of the related derivative liability, was recorded in the accompanying unaudited condensed consolidated statements of operations.

7. Asset Acquisitions

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, 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. 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. In January

2023, the Company paid the accrued regulatory milestone of \$15.0 million associated with approval of Livmarli by the European Commission for the treatment of cholestatic pruritus in patients with ALGS two months of age and older. No additional milestones have been accrued as of June 30, 2023 as there were no potential milestones yet considered probable. There were no development or regulatory milestones achieved for Livmarli or volixibat during the six months ended June 30, 2023 and 2022.

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. The Company has not paid milestone payments pursuant to this agreement for the periods presented.

In May 2022, the Company completed the merger and acquisition of Satiogen for total consideration of approximately \$24.2 million. At acquisition, Satiogen's assets consisted of cash and intangible assets related to developed technology. The purchase consideration consisted of 609,305 shares of the Company's common stock issued upon the closing of the acquisition and cash consideration of \$2.6 million, excluding \$0.2 million of stock option exercise prices deemed to have been paid immediately prior to the acquisition, in respect of an equivalent amount of cash on the books of Satiogen, with up to an additional 32,494 shares of common stock that would have been issued upon the closing of the acquisition except the parties agreed to such shares being held back by the Company for 12 months from the acquisition date to satisfy certain purchase price adjustments and indemnification obligations that may arise during this period. Specifically, purchase price adjustments and indemnification obligations that arise will reduce the number of shares issuable by the Company at settlement in accordance with the terms of the definitive acquisition agreement. The purchase consideration also included issuance of up to an additional 199,993 shares of the Company's common stock, contingent upon the achievement of a certain milestone by June 30, 2025, subject to adjustment to satisfy certain purchase price adjustments and indemnification obligations that may arise. In December 2022, with the approval of Livmarli by the European Commission for the treatment of cholestatic pruritus in patients with ALGS two months of age and older, the milestone was achieved and the Company issued 199,993 shares of common stock in January 2023. Additionally, in June 2023, the contingencies related to the held back shares were resolved and the Company issued 31,631 shares of common stock. 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.

The Company accounted for the transaction as an asset acquisition as the set of acquired assets did not constitute a business and substantially all the fair value of the gross assets acquired was concentrated in a group of similar identifiable assets, namely, the Satiogen intangible assets consisted of intellectual property. The Company evaluated that the intellectual property assets acquired were deemed to be commercially viable and the cost of the acquisition was recorded as an intangible asset.

There was no gain or loss recognized from settlement of the preexisting contractual relationship with Satiogen as the pre-existing contract was determined to be at fair value on the date of acquisition. For the three and six months ended June 30, 2023, there were no significant expenses incurred that were approved for settlement against the Indemnification Holdback.

As the number of shares potentially issuable upon the resolution of the Indemnification Holdback and the Contingent Milestone was variable, they were recorded as liabilities at their respective fair values on the date of acquisition using the Company's common stock price. The fair value of the Indemnification Holdback was additionally determined based on management's estimate of the probability of indemnification obligations being incurred during the one year following the acquisition date, while the fair value of the Contingent Milestone was additionally determined based upon management's estimate of the probability of the milestone being met until the contingency was resolved in December 2022. The fair value of the Indemnification Holdback liability and the Contingent Milestone liability were remeasured at each reporting period until settled, with resulting changes in the fair value recorded in other income (expense) in the accompanying unaudited condensed consolidated statements of operations.

The following represents the consideration paid and allocation of purchase price for the acquisition of Satiogen (in thousands, except per share data):

Issued common stock	\$	15,585
Cash consideration		2,600
Indemnification Holdback		831
Contingent consideration settled in common stock		4,600
Transaction costs		545
Total purchase consideration		24,161
Assets acquired:		
Intangible assets - developed technology		21,561
Cash consideration		2,600
Total assets acquired	\$	24,161

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 will be required to pay 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. As of June 30, 2023, no milestones had been accrued as there were no potential milestones considered probable.

8. Collaboration and License Agreements

License and Collaboration Agreement with CANbridge

In April 2021, the Company entered into an exclusive license and collaboration agreement with CANbridge Pharmaceuticals, Inc. ("CANbridge"). Under the terms of the agreement, CANbridge has obtained the exclusive right to develop and commercialize Livmarli within the Greater China regions (China, Hong Kong, Macau and Taiwan). In connection with the agreement, the Company received an upfront payment of \$11.0 million, which, upon satisfaction of the performance obligation and receipt by CANbridge of the right to use and benefit from the license in May 2021, was recorded as license revenue. Additionally, the Company is eligible to receive up to \$5.0 million in research and development funding, and up to \$109.0 million for the achievement of future regulatory and commercial milestones, with double-digit tiered royalties based on product net sales. The Company concluded at inception of the agreement that the transaction price should not include the variable consideration related to unachieved developmental and regulatory milestones as this consideration was considered to be constrained as it is probable that the inclusion of such variable consideration could result in a significant reversal in cumulative revenue. The Company will recognize any consideration related to sales-based payments when the related sales occur, as the Company has determined that these amounts relate predominantly to the license granted and therefore will be recognized at the later of (i) when or as the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). The Company re-evaluates the transaction price at each reporting period as uncertain events are resolved and other changes in circumstances occur. In January 2022, CANbridge achieved a regulatory milestone, triggering a milestone payment to the Company of \$2.0 million which was recorded as license revenue in the accompanying unaudited condensed consolidated statements of operations for the six months ended June 30, 2022. In June 2023, CANbridge achieved another regulatory milestone, triggering a milestone payment to the Company of \$5.0 million, which, upon the release of the constraint, was included in the transaction price and recorded as license revenue in the accompanying unaudited condensed consolidated statements of operations for the three and six months ended June 30, 2023. For the three and six months ended June 30, 2023, the Company recorded research and development funding of \$1.1 million and \$1.4 million, respectively,

and for the three and six months ended June 30, 2022, the Company recorded research and development funding of \$0.3 million and \$0.7 million, respectively, payable by CANbridge to the Company which is reflected as a reduction of research and development expense in the accompanying unaudited condensed consolidated statements of operations. As of June 30, 2023 and December 31, 2022, such research and development funding of \$1.6 million and \$0.2 million, respectively, was recorded as a receivable which was included in accounts receivable on the accompanying unaudited condensed consolidated balance sheets.

License and Collaboration Agreement with GC Biopharma

In July 2021, the Company entered into an exclusive license and collaboration agreement with GC Biopharma. Under the terms of the agreement, GC Biopharma has obtained the exclusive right to develop and commercialize Livmarli within South Korea for ALGS, progressive familial intrahepatic cholestasis (“PFIC”), and biliary atresia (“BA”). In connection with the agreement, the Company received a \$5.0 million upfront payment, which, upon satisfaction of the performance obligation and receipt by GC Biopharma of the right to use and benefit from the license, was recorded as license revenue. Additionally, the Company is entitled to certain research and development funding and up to \$23.0 million for the achievement of future regulatory and commercial milestones, with double-digit tiered royalties based on product net sales. At inception of the agreement, the Company concluded that the transaction price should not include the variable consideration related to unachieved developmental and regulatory milestones as this consideration was considered to be constrained as it is probable that the inclusion of such variable consideration could result in a significant reversal in cumulative revenue for this contract when the uncertainty is resolved in the future. The Company will recognize any consideration related to sales-based payments (including milestones and royalties) when the related sales occur, as the Company has determined that these amounts relate predominantly to the license granted and therefore will be recognized on the later to occur of satisfaction of the performance obligation or the occurrence of the related sales. The Company re-evaluates the transaction price at each reporting period as uncertain events are resolved and other changes in circumstances occur. In February 2023, GC Biopharma achieved a regulatory milestone under this agreement triggering a milestone payment to the Company of \$2.5 million, which upon the release of the constraint was included in the transaction price and recognized as license revenue in the accompanying unaudited condensed consolidated statements of operations for the six months ended June 30, 2023. For the three and six months ended June 30, 2023 and 2022, research and development funding reflected as a reduction of research and development expense in the accompanying unaudited condensed consolidated statements of operations was insignificant.

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. Under the terms of the agreement, Takeda will be responsible for regulatory approval and commercialization of Livmarli in Japan. Takeda will also be responsible for development, including conducting clinical studies in cholestatic indications. The Company is responsible for commercial supply to Takeda. In exchange, the Company is eligible to receive a percentage of Takeda’s annualized 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 fully constrained all revenues upon transfer of control of the license to Takeda, which occurred when Takeda could use and benefit from the license, and will recognize any consideration related to sales-based payments when the related sales occur, as the Company has determined that these amounts relate predominantly to the license granted and therefore will be recognized on the later to occur of satisfaction of the performance obligation or the occurrence of the related sales.

9. Leases

In January 2019, the Company entered into an operating lease agreement for office space which consisted of approximately 5,600 square feet (the “Initial Lease”). The lease term is approximately four years with an option to extend the term for one five-year term, which at the time was not reasonably assured of exercise and therefore, not included in the lease term. The lease contained a tenant improvement allowance of \$0.4 million, which has been recorded as leasehold improvements on the accompanying unaudited condensed consolidated balance sheets with a corresponding reduction of the right-of-use (“ROU”) asset at inception of the lease. Rent payments commenced in August 2019.

In November 2019, the Company amended the operating lease agreement (the “Amended Agreement”) to extend the term of the Initial Lease through March 2025. This extension was accounted for as a lease modification and the Company recorded an increase to the ROU asset and lease liability of \$0.6 million at the time of the amendment.

Additionally, pursuant to the Amended Agreement, the Company expanded the office space by 5,555 square feet for a five-year term expiring in March 2025 (the “Expanded Space”). The Company accounted for the Expanded Space as a separate contract as there were material additional rights of use that were not included in the Initial Lease. The Amended Agreement contained a tenant improvement allowance of \$0.8 million in connection with the expanded space, which has been recorded as leasehold improvements within property and equipment, net on the accompanying unaudited condensed consolidated balance sheets with a corresponding reduction of the ROU asset at inception of the lease for the expanded space.

The ROU and corresponding lease liabilities were estimated using a weighted-average incremental borrowing rate of 8.0%.

As of June 30, 2023, the Company recorded an aggregate ROU asset of \$1.6 million and an aggregate lease liability of \$2.2 million on the accompanying unaudited condensed consolidated balance sheets. The weighted-average remaining lease term is 2.4 years.

As of June 30, 2023, undiscounted future minimum payments under the Company's operating leases are as follows (in thousands):

	Undiscounted Rent Payments
2023 (remaining six months)	599
2024	1,191
2025	357
2026	114
2027	114
Thereafter	57
Total undiscounted lease payments	2,432
Less: imputed interest	(214)
Total lease liability	\$ 2,218

Rent expense was \$0.2 million for the three months ended June 30, 2023 and 2022, and \$0.4 million for the six months ended June 30, 2023 and 2022. Variable lease payments for operating expenses for the three and six months ended June 30, 2023 and 2022 were immaterial.

10. Convertible Notes

In April 2023, the Company issued \$316.3 million aggregate principal amount of its 4.00% Convertible Senior Notes due 2029 (the "Notes") in a private placement to qualified institutional buyers pursuant to Rule 144A under the Securities Act of 1933, as amended. The Notes are subject to the terms and conditions of an Indenture (the "Indenture") between the Company and U.S. Bank Trust Company, National Association, as trustee.

The net proceeds from the issuance of the Notes were \$305.3 million, after deducting the initial purchasers' discounts and commissions and offering expenses.

The Notes are the Company's senior, unsecured obligations and are (i) senior in right of payment to any of the Company's indebtedness that is expressly subordinated to the Notes in right of payment; (ii) equal in right of payment to any of the Company's indebtedness that is not so subordinated; (iii) effectively subordinated to any of the Company's secured indebtedness, to the extent of the value of the collateral securing that indebtedness; and (iv) structurally subordinated to all indebtedness and other liabilities, including trade payables, and (to the extent the Company is not a holder thereof) preferred equity, if any, of the Company's subsidiaries.

The Notes accrue interest at a rate of 4.00% per annum, payable semi-annually in arrears on May 1 and November 1 of each year, beginning on November 1, 2023. The Notes will mature on May 1, 2029, unless earlier converted, redeemed or repurchased by the Company. Before January 2, 2029, noteholders will have the right to convert their Notes only in the following circumstances:

- (i) during any calendar quarter (and only during such calendar quarter) commencing after the calendar quarter ending on June 30, 2023, if the last reported sale price per share of the Company's common stock exceeds 130% of the conversion price for the Notes for each of at least 20 trading days, whether or not consecutive, during the 30 consecutive trading days ending on, and including, the last trading day of the immediately preceding calendar quarter;
- (ii) during the five consecutive business days immediately after any 10 consecutive trading day period (such 10 consecutive trading day period, the "measurement period") if the trading price per \$1,000 principal amount of Notes for each trading day of the measurement period was less than 98% of the product of the last reported sale price per share of the common stock on such trading day and the conversion rate on such trading day;
- (iii) upon the occurrence of certain corporate events or distributions on the common stock, as described in the Indenture; and
- (iv) if the Company calls such Notes for redemption.

Additionally, noteholders will have the right to convert their Notes at any time from January 2, 2029 until the close of business on the scheduled trading day immediately before the maturity date. The Company may settle conversions by paying or delivering, as applicable, cash, shares of its common stock or a combination of cash and shares of its common stock, at the Company's election. The initial conversion rate for the Notes is 31.5075 shares of common stock per \$1,000 principal amount of Notes, which represents an

initial conversion price of approximately \$31.74 per share of common stock. The conversion rate and conversion price will be subject to customary adjustments upon the occurrence of certain events. In addition, if certain corporate events that constitute a “Make-Whole Fundamental Change” (as defined in the Indenture) occur, then the conversion rate will, in certain circumstances, be increased for a specified period of time.

The Company may not redeem the Notes at its option at any time before May 5, 2026. The Notes are redeemable, in whole or in part (subject to the partial redemption limitation described below), at the Company’s option at any time, and from time to time, on or after May 5, 2026 and, in the case of a partial redemption, on or before the 50th scheduled trading day immediately before the maturity date, at a cash redemption price equal to the principal amount of the Notes to be redeemed, plus accrued and unpaid interest, if any, to, but excluding, the redemption date, but only if the last reported sale price per share of the Company’s common stock exceeds 130% of the conversion price on (i) each of at least 20 trading days, whether or not consecutive, during the 30 consecutive trading days ending on, and including, the trading day immediately before the date the Company sends the related redemption notice; and (ii) the trading day immediately before the date the Company sends such notice. In addition, calling any Note for redemption will constitute a Make-Whole Fundamental Change with respect to that Note, in which case the conversion rate applicable to the conversion of that Note will be increased in certain circumstances if it is converted after it is called for redemption. Pursuant to the partial redemption limitation, the Company may not elect to redeem less than all of the outstanding Notes unless at least \$75.0 million aggregate principal amount of Notes are outstanding and not called for redemption as of the time the Company sends the related redemption notice.

If a “Fundamental Change” (as defined in the Indenture) occurs, then, subject to a limited exception for certain cash mergers, noteholders may require the Company to repurchase their Notes at a cash repurchase price equal to the principal amount of the Notes to be repurchased, plus accrued and unpaid interest, if any, to, but excluding, the fundamental change repurchase date. The definition of Fundamental Change includes certain business combination transactions involving the Company and certain de-listing events with respect to the Company’s common stock.

The Indenture contains customary events of default with respect to the Notes and provides that upon certain events of default occurring and continuing, the Trustee may, and the Trustee at the request of holders of at least 25% in principal amount of the Notes shall, declare all principal and accrued and unpaid interest, if any, of the Notes to be due and payable. In case of certain events of bankruptcy, insolvency or reorganization, involving the Company or a significant subsidiary, all of the principal of and accrued and unpaid interest on the Notes will automatically become due and payable.

As of June 30, 2023, the Notes consisted of the following (in thousands):

	Convertible Notes
Principal amount	\$ 316,250
Unamortized debt discount and issuance costs	(10,639)
Net carrying amount	<u>\$ 305,611</u>

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 June 30, 2023, the remaining amortization period of the debt discount was approximately 5.8 years and the effective interest on the Notes was 4.6%. The following table sets forth interest expense recognized related to the Notes in the three and six months ended June 30, 2023 (in thousands):

	Convertible Notes
Coupon interest expense	\$ 2,601
Amortization of debt discount and issuance costs	307
Total interest expense on convertible notes	<u>\$ 2,908</u>

As of June 30, 2023, the estimated fair value of the Notes was \$355.0 million. 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 has been classified as Level 2 in the fair value hierarchy.

11. Stockholders’ Equity

Common Stock

In August 2020, the SEC declared effective a registration statement on Form S-3 (“Shelf Registration”) covering the sale of up to \$300.0 million of the Company’s securities. Also, in August 2020, the Company entered into a sales agreement (“Sales Agreement”) with SVB Securities LLC (“SVB Securities”) pursuant to which the Company may elect to issue and sell, from time to time, shares of common stock having an aggregate offering price of up to \$75.0 million under the Shelf Registration through SVB

Securities acting as the sales agent and/or principal. During the six months ended June 30, 2023, the Company issued and sold 658,206 shares of common stock pursuant to the Sales Agreement resulting in gross proceeds to the Company of \$15.0 million. The net proceeds to the Company for the six months ended June 30, 2023, after deducting sales commissions to SVB Securities and other issuance expenses were approximately \$14.5 million. As of June 30, 2023, the Company has issued and sold an aggregate of 2,125,090 shares of common stock pursuant to the Sales Agreement resulting in aggregate gross proceeds to the Company of \$43.7 million. The remaining capacity under the Sales Agreement is approximately \$31.3 million as of June 30, 2023.

In August 2022, the Company completed an underwritten public offering of its common stock pursuant to the Shelf Registration. The Company issued and sold 3,478,261 shares of common stock at a public offering price of \$23.00 per share. In addition, the Company granted the underwriters an option, exercisable for 30 days, to purchase up to 521,739 additional shares of its common stock at the public offering price, less the underwriting discounts, commissions and offering expenses, which the underwriters exercised in full. The underwritten public offering, including the underwriters' exercise of their option, resulted in net proceeds to the Company of \$86.1 million after deducting underwriting discounts, commissions and offering expenses.

On September 9, 2022, the Company filed an automatic shelf registration statement on Form S-3 with the SEC, 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 June 30, 2023, the Company had not issued any securities pursuant to the automatic shelf registration statement.

Common Stock Reserved for Issuance

Common stock reserved for issuance is as follows:

	As of June 30, 2023	As of December 31, 2022
Stock options, restricted stock units and performance stock units issued and outstanding	10,647,891	8,955,557
Reserved for future stock awards or option grants	1,453,028	1,596,947
Reserved for employee stock purchase plan	1,081,089	1,157,570
Common stock issuable upon conversion of convertible notes	9,964,247	—
Common stock held back in connection with asset acquisition	—	31,638
Common stock issuable as contingent consideration in connection with asset acquisition	—	199,993
	23,146,255	11,941,705

12. Stock-Based Compensation

Equity Incentive Plans

In November 2018, the Company adopted the 2018 Equity Incentive Plan (the "2018 Plan"), which permits the granting of stock awards and incentive and nonstatutory stock options to employees, directors and consultants of the Company.

In July 2019, the Company's board of directors and stockholders approved and adopted the 2019 Equity Incentive Plan (the "2019 Plan"). The 2019 Plan became effective on July 17, 2019. Under the 2019 Plan, the Company may grant stock options, stock appreciation rights, restricted stock, restricted stock units and other stock or cash-based awards to individuals who are then employees, officers, directors or consultants of the Company. Shares subject to outstanding awards under the 2018 Plan as of the effective date of the 2019 Plan that are subsequently canceled, forfeited or repurchased by the Company will be added to the shares reserved under the 2019 Plan. In addition, the number of shares of common stock available for issuance under the 2019 Plan will be automatically increased on the first day of each calendar year during the ten-year term of the 2019 Plan, beginning with January 1, 2020 and ending with January 1, 2029, by an amount equal to 5% of the outstanding number of shares of the Company's common stock on December 31st of the preceding calendar year or such lesser amount as determined by the Company's board of directors. As of June 30, 2023, 1,114,199 shares of common stock were available for issuance under the 2019 Plan.

In March 2020, the compensation committee of the Company's board of directors approved and adopted the 2020 Inducement Plan (the "2020 Inducement Plan"). Under the 2020 Inducement Plan, the Company may grant nonstatutory stock options, stock appreciation rights, restricted stock and restricted stock units to new employees entering into employment with the Company in accordance with Nasdaq Listing Rule 5635(c)(4). At adoption, the 2020 Inducement Plan authorized 750,000 shares of the Company's common stock for future issuance. In 2021 and 2020, the Company's board of directors authorized an additional 1,000,000 and 750,000 shares of the Company's common stock for future issuance, respectively. As of June 30, 2023, 338,829 shares of common stock were available for issuance under the 2020 Inducement Plan.

Stock Options

The following table summarizes stock option activity during the six months ended June 30, 2023 (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, 2022	8,340,083	\$ 13.63	7.5	\$ 51,645
Granted	1,531,789	\$ 23.98		
Exercised	(153,678)	\$ 14.27		
Canceled and forfeited	(198,295)	\$ 18.18		
Outstanding as of June 30, 2023	9,519,899	\$ 15.19	7.2	\$ 102,171
Vested and exercisable as of June 30, 2023	5,651,269	\$ 11.69	6.2	\$ 80,121

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. The weighted-average grant date fair value per share of stock options granted during the six months ended June 30, 2023 and 2022 was \$17.24 and \$12.37 per share, respectively. The total intrinsic value of options exercised during the six months ended June 30, 2023 and 2022 was \$1.7 million. As of June 30, 2023, the total unrecognized stock-based compensation related to unvested stock option awards granted was \$51.4 million, which the Company expects to recognize over a weighted-average period of approximately 2.6 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 June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Expected term (in years)	5.50-6.08	5.50-6.08	5.31-6.08	5.50-6.08
Expected volatility	81.59%-83.13%	80.86%-83.20%	81.59%-85.24%	80.86%-83.20%
Risk-free interest rate	3.35%-3.89%	2.78%-3.25%	3.35%-3.91%	1.46%-3.25%
Expected dividend yield	—	—	—	—

Restricted Stock Units

The following table summarizes the activity under the Company's restricted stock units for the six months ended June 30, 2023:

	Number of Awards	Weighted-Average Grant Date Fair Value per Award
Unvested and outstanding as of December 31, 2022	615,474	\$ 18.36
Granted	553,419	\$ 23.63
Vested	(145,724)	\$ 17.51
Cancelled/Forfeited	(43,012)	\$ 20.15
Unvested and outstanding as of June 30, 2023	980,157	\$ 21.38

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 June 30, 2023, the total unrecognized stock-based compensation related to restricted stock unit awards granted was \$16.5 million, which the Company expects to recognize over a weighted-average period of approximately 2.4 years.

Performance Stock Units

In January 2023, the Company granted an aggregate of 135,835 performance stock units to certain executive participants ("2023 Executive PSUs"). The 2023 Executive PSUs are subject to a performance condition of achieving certain net product sales levels related to Livmarli during the year ended December 31, 2024. If the performance condition is met, the first tranche of the award will vest on March 15, 2025 and the second tranche will vest on March 15, 2026, subject to the executive employees' continuous service through each vesting date. The number of units to be vested in the first tranche of the 2023 Executive PSUs is calculated by multiplying two-thirds of the 2023 Executive PSUs granted by a percentage calculated based on attained Livmarli sales metrics, as certified by the Company's Compensation Committee. The number of units to be vested in the second tranche of the 2023 Executive PSUs equals 50% of the units vested in the first tranche.

In June 2023, the Company granted an aggregate of 12,000 PSUs to certain employees ("2023 Employee PSUs"). The 2023 Employee PSUs are subject to a performance condition of achieving certain net product sales related to Livmarli in the US for the year ended December 31, 2023.

The following table summarizes the activity under the Company's performance stock units for the six months ended June 30, 2023:

	Number of Awards	Weighted-Average Grant Date Fair Value per Award
Unvested and outstanding as of December 31, 2022	—	\$ —
Granted	147,835	\$ 23.64
Unvested and outstanding as of June 30, 2023	147,835	\$ 23.64

As of June 30, 2023, the total unrecognized stock compensation related to performance stock units granted was \$1.5 million, which the Company expects to recognize over a weighted-average period of approximately 2.0 years.

2019 Employee Stock Purchase Plan

In July 2019, the Company's board of directors and stockholders approved and adopted the 2019 Employee Stock Purchase Plan ("ESPP"). A total of 500,000 shares of common stock were approved to be initially reserved for issuance under the ESPP. In addition, the number of shares of common stock available for issuance under the ESPP will be automatically increased on the first day of each calendar year during the first ten years of the term of the ESPP, beginning with January 1, 2020 and ending with January 1, 2029, by an amount equal to the lesser of (i) 1% of the outstanding number of shares of common stock on December 31st of the preceding calendar year, (ii) 1,500,000 shares of common stock or (iii) such lesser amount as determined by the Company's board of directors. The ESPP became effective on July 17, 2019 and generally provides for six-month consecutive offering periods beginning on May 11th and November 11th of each year. During the three and six months ended June 30, 2023, 76,481 shares were issued under the ESPP. As of June 30, 2023, the Company had 1,081,089 shares available for future issuance under the ESPP. The stock-based compensation related to the ESPP was \$0.2 million for the three months ended June 30, 2023 and 2022, and \$0.5 million and \$0.3 million for the six months ended June 30, 2023 and 2022, respectively.

Restricted Common Stock

In November 2018, in connection with the issuance of Series A Preferred Stock, the Company's founders agreed to modify their outstanding shares of common stock to include vesting provisions that require continued service to the Company in order to vest in those shares. As such, the 562,500 modified shares of common stock became compensatory upon such modification. All restricted common stock was fully vested as of December 31, 2022. During the three and six months ended June 30, 2022, 33,398 and 66,796 shares vested, respectively.

Stock-Based Compensation

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Selling, general and administrative	\$ 5,685	\$ 4,225	\$ 11,531	\$ 8,201
Research and development	2,672	2,416	5,387	5,001
Total	<u>\$ 8,357</u>	<u>\$ 6,641</u>	<u>\$ 16,918</u>	<u>\$ 13,202</u>

Stock-based compensation capitalized into inventory was \$0.2 million for the three months ended June 30, 2023 and 2022, and \$0.4 million and \$0.2 million for the six months ended June 30, 2023, and 2022, respectively.

13. Contingencies

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 financial statements.

An estimated loss contingency is accrued in the Company's 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 such 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.

14. Subsequent Events

Asset Purchase Agreement

On July 16, 2023, the Company executed an Asset Purchase Agreement (the "Purchase Agreement") with Traveře Therapeutics, Inc., a Delaware corporation ("Traveře").

Subject to the terms and conditions of the Purchase Agreement, at the closing (the "Closing") of the transactions contemplated by the Purchase Agreement (the "Asset Purchase"), the Company will purchase from Traveře substantially all of the assets that are primarily related to its business of development, manufacturing (including synthesis, formulation, finishing or packaging) and commercialization of Chenodal and Cholbam (also known as Kolbam, and together with Chenodal, the "Products") for an aggregate purchase price, subject to certain adjustments pursuant to the terms of the Purchase Agreement, of up to \$445.0 million in cash, with \$210.0 million due at the Closing and up to \$235.0 million due after the Closing, upon the achievement of certain milestones based on specified amounts of annual net sales (tiered from \$125.0 million to \$500.0 million) of the Products (the "Milestone Events").

The parties entered into a Transitional Services Agreement pursuant to which Traveře has agreed to perform certain services for a period of time following the consummation of the Asset Purchase with respect to the Company's use and operation of the assets purchased in the Asset Purchase.

Consummation of the Asset Purchase is subject to customary closing conditions, including, among others, (i) obtaining certain material consents, (ii) the expiration or termination of the applicable waiting period (and any extension thereof) under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, (iii) the absence of any law, order or legal proceeding prohibiting the Asset Purchase, (iv) the accuracy of representations and warranties set forth in the Agreement (subject to customary materiality qualifiers) and material compliance with covenants set forth in the Agreement and (v) the execution and delivery of certain related ancillary documents. The Company expects the Closing to occur in the third quarter of 2023.

The Company and Traveře are permitted under certain circumstances to terminate the Agreement, including in the event that, subject to an automatic extension through April 16, 2024 to satisfy antitrust conditions, the Asset Purchase is not consummated by October 16, 2023.

Private Placement

Concurrently with the execution and delivery of the Purchase Agreement, certain parties have entered into subscription agreements (the “Subscription Agreements”) with the Company, pursuant to which they have agreed, subject to the terms and conditions of the Subscription Agreements, to purchase immediately prior to, but subject to, the consummation of the Asset Purchase, shares of the Company's common stock with a par value of \$0.0001 per share for an aggregate purchase price of approximately \$210.0 million at a price of \$26.25 per share, the closing price of the Company's common stock on July 14, 2023.

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, 2022, which was filed with the Securities and Exchange Commission ("SEC") on March 8, 2023. 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 focused on the identification, acquisition, development and commercialization of novel therapies for debilitating rare and orphan diseases. We focus on diseases for which the unmet medical need is high and the biology for treatment is clear.

Our product LIVMARLI (maralixibat) oral solution ("Livmarli"), a novel, orally administered, minimally-absorbed ileal bile acid transporter ("IBAT") inhibitor, is approved for the treatment of cholestatic pruritus in patients with Alagille syndrome ("ALGS") three months of age and older in the United States and for the treatment of cholestatic pruritus in patients with ALGS two months of age and older in Europe. We believe the prevalent patient population in the United States is approximately 2,000 to 2,500 pediatric ALGS patients, which, based on our current expectations and beliefs, represents a greater than \$500.0 million market opportunity. ALGS is estimated to impact one out of every 30,000 births globally. We market and commercialize Livmarli in the United States 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. We are also developing Livmarli for progressive familial intrahepatic cholestasis ("PFIC") and biliary atresia ("BA"). We have submitted a Supplemental New Drug Application ("sNDA") to the U.S. Food and Drug Administration ("FDA") and a marketing authorization to the European Marketing Agency ("EMA") based on positive data from our Phase 3 study of Livmarli in patients with PFIC. We also expect to report topline data from our EMBARK Phase 2b clinical trial in BA in the second half of 2023.

We are advancing our second 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"). Volixibat has been studied in over 400 adults for up to 48 weeks. Clinical trials of volixibat have shown significant activity on IBAT and bile acid markers such as 7 α C4, fecal bile acids and cholesterol, demonstrating potent biological activity. We expect to conduct an interim analysis of our VISTAS Phase 2b clinical trial in PSC and report interim data from our VANTAGE Phase 2b clinical trial in PBC, both in the second half of 2023.

We were incorporated in May 2018 and commenced operations in November 2018. 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 Livmarli, and conducting business development activities relating to, among other things, portfolio expansion through collaborations and acquisitions.

We have only one product approved for commercial sale and have not generated significant revenues from product sales, net as of June 30, 2023. We expect to incur significant operating losses for the foreseeable future. Since inception, we have funded our operations to date primarily through debt, equity, revenue interest financings and cash from our product sales, net and collaboration revenue.

Recent Developments

Asset Purchase Agreement

On July 16, 2023, we executed an Asset Purchase Agreement (the “Purchase Agreement”) with Travers Therapeutics, Inc., a Delaware corporation (“Travers”).

Subject to the terms and conditions of the Purchase Agreement, at the closing (the “Closing”) of the transactions contemplated by the Purchase Agreement (the “Asset Purchase”), we will purchase from Travers substantially all of the assets that are primarily related to its business of development, manufacturing (including synthesis, formulation, finishing or packaging) and commercialization of Chenodal and Cholbam (also known as Kolbam, and together with Chenodal, the “Products”) for an aggregate purchase price, subject to certain adjustments pursuant to the terms of the Purchase Agreement, of up to \$445.0 million in cash, with \$210.0 million due at the Closing and up to \$235.0 million due after the Closing, upon the achievement of certain milestones based on specified amounts of annual net sales (tiered from \$125.0 million to \$500.0 million) of the Products (the “Milestone Events”).

The parties entered into a Transitional Services Agreement pursuant to which Travers has agreed to perform certain services for a period of time following the consummation of the Asset Purchase with respect to our use and operation of the assets purchased in the Asset Purchase.

Consummation of the Asset Purchase is subject to customary closing conditions, including, among others, (i) obtaining certain material consents, (ii) the expiration or termination of the applicable waiting period (and any extension thereof) under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, (iii) the absence of any law, order or legal proceeding prohibiting the Asset Purchase, (iv) the accuracy of representations and warranties set forth in the Agreement (subject to customary materiality qualifiers) and material compliance with covenants set forth in the Agreement and (v) the execution and delivery of certain related ancillary documents. We expect the Closing to occur in the third quarter of 2023.

We and Travers are permitted under certain circumstances to terminate the Agreement, including in the event that, subject to an automatic extension through April 16, 2024 to satisfy antitrust conditions, the Asset Purchase is not consummated by October 16, 2023.

Private Placement

Concurrently with the execution and delivery of the Purchase Agreement, certain parties have entered into subscription agreements (the “Subscription Agreements”) with us, pursuant to which they have agreed, subject to the terms and conditions of the Subscription Agreements, to purchase immediately prior to, but subject to, the consummation of the Asset Purchase, shares of our common stock with a par value of \$0.0001 per share for an aggregate purchase price of approximately \$210.0 million at a price of \$26.25 per share, the closing price of our common stock on July 14, 2023 (such transaction, the “Private Placement”).

Financing Transactions

On April 17, 2023, we completed an offering of \$316.3 million aggregate principal of 4.00% Convertible Senior Notes due 2029 (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. We used a portion of the net proceeds to repurchase the revenue interests pursuant to the Revenue Interest Purchase Agreement (“RIPA”), entered into in December 2020, as amended September 2021, by and among us and Mulholland SA LLC, an affiliate of Oberland Capital LLC, as agent for the purchasers party thereto (the “Purchasers”), and the Purchasers. We repurchased the revenue interests from the Purchasers at a call price of \$192.7 million. Upon repurchase of the revenue interests from the Purchasers, the RIPA, in accordance with its terms, was terminated with no further payments due to the Purchasers and the \$100.0 million of previously restricted cash equivalents was no longer restricted from use. The terms of these Notes are further described in Note 10 to our unaudited condensed consolidated financial statements.

In August 2020, the Securities and Exchange Commission (“SEC”) declared effective a registration statement on Form S-3 (“Shelf Registration”) covering the sale of up to \$300.0 million of our securities. Also, in August 2020, we entered into a sales agreement (“Sales Agreement”) with SVB Securities LLC (“SVB Securities”) pursuant to which we may elect to issue and sell, in an at the market offering, from time to time, shares of common stock having an aggregate offering price of up to \$75.0 million under the Shelf Registration through SVB Securities acting as the sales agent and/or principal.

In August 2022, we completed an underwritten public offering of our common stock pursuant to the Shelf Registration. We sold 3,478,261 shares of common stock at a price to the public of \$23.00 per share. In addition, we granted the underwriters an option, exercisable for 30 days, to purchase up to 521,739 additional shares of our common stock at the public offering price, less the underwriting discounts, commissions and offering expenses, which the underwriters exercised in full. The underwritten public offering, including the underwriters’ exercise of their option, resulted in net proceeds to us of \$86.1 million after deducting underwriting discounts, commissions and offering expenses.

During the six months ended June 30, 2023, we issued and sold 658,206 shares of common stock pursuant to the Sales Agreement at a weighted-average price of \$22.79 per share, respectively, resulting in aggregate gross proceeds to us of \$15.0 million. The net proceeds after deducting sales commissions to SVB Securities and other issuance expenses were approximately \$14.5 million. As of June 30, 2023, we have issued and sold an aggregate of 2,125,090 shares of common stock pursuant to the Sales Agreement, resulting in aggregate gross proceeds to us of \$43.7 million. The remaining capacity under the Sales Agreement is approximately \$31.3 million as of June 30, 2023.

Financial Overview

Our net loss was \$74.0 million and \$26.9 million for the three months ended June 30, 2023 and 2022, respectively, and \$104.2 million and \$63.5 million for the six months ended June 30, 2023 and 2022, respectively. Our net loss for the three and six months ended June 30, 2023 includes a \$49.1 million loss related to the extinguishment of the liabilities related to the RIPA. As of June 30, 2023, we had an accumulated deficit of \$497.0 million, compared to \$392.8 million as of December 31, 2022. As of June 30, 2023, we had cash, cash equivalents and investments of \$330.0 million, compared to cash, cash equivalents, restricted cash equivalents and investments of \$251.7 million as of December 31, 2022.

We anticipate we will continue to generate losses for the foreseeable future as we continue commercial activities for Livmarli, 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 our product sales of Livmarli will continue to increase on an annual basis, though seasonality in demand and international product ordering may result in quarterly fluctuations. We have also entered into collaboration arrangements with other companies whereby we are entitled to receive upfront and license fees, research and development funding, development and sales-based milestones, and tiered royalties based on sales of commercialized products. In select countries, we have also entered distribution agreements for the sale of Livmarli and such distributors may have fluctuating purchase levels of product. 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, product revenue from Livmarli and through a combination of equity offerings, including the private placement described above, 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.

Prior to the regulatory approval of Livmarli, the manufacturing and related costs were expensed as research and development as any future economic benefit was not considered probable; accordingly, these costs were not capitalized and as a result gross margins resulting from product sales will initially be higher until we deplete inventories that we had expensed prior to receiving approval. We expect we will substantially deplete such inventories within the next 12 months from the filing of this Quarterly Report on Form 10-Q.

Geopolitical and Macroeconomic Developments

Due to the ongoing military conflict between Ukraine and Russia, the U.S. and global financial markets experienced volatility, which has led to disruptions to trade, commerce, pricing stability, credit availability and supply chain continuity globally. In connection with the military conflict, the United States, United Kingdom and European Union (“EU”), along with others, imposed significant new sanctions and export controls against Russia, Russian banks and certain Russian individuals and may implement additional sanctions or take further punitive actions in the future. The full economic and social impact of the sanctions imposed on Russia (as well as possible future punitive measures that may be implemented), as well as the counter measures imposed by Russia, in addition to the ongoing military conflict between Ukraine and Russia and related sanctions, which could conceivably expand into the surrounding region, remains uncertain; however, both the conflict and related sanctions have resulted and could continue to result in disruptions to trade, commerce, pricing stability, credit availability and supply chain continuity in both Europe and globally, and has introduced significant uncertainty into global markets. As a result, our business and results of operations may be adversely affected by the ongoing military conflict between Ukraine and Russia and related sanctions, particularly to the extent it escalates to involve additional countries, further economic sanctions or wider military conflict.

Although we did not see a significant financial impact to our business operations as a result of recent geopolitical and macroeconomic developments, such as recent and potential future disruptions in access to bank deposits or lending commitments due to bank failures, the COVID-19 pandemic and the ongoing Ukraine-Russia conflict for the six months ended June 30, 2023, there may be potential impacts to our business in the future that are highly uncertain and difficult to predict such as disruptions or restrictions in our supply chain, disruption or restrictions on our employees’ ability to travel, disruptions to or delays in ongoing non-clinical trials, clinical trials, third-party manufacturing supply and other operations, inability for patients to see their healthcare providers and access our product and product candidates, the potential diversion of healthcare resources away from the conduct of clinical trials to focus on pandemic or adverse geopolitical and macroeconomic concerns, interruptions or delays in the operations of the FDA or other

regulatory authorities, continued increases in inflation and interest rates, bank failures, changes in availability and cost of credit and our ability to raise capital and conduct business development activities. The ultimate impact of these geopolitical and macroeconomic developments, as well as any lasting effects on our revenue and the way we conduct our business, is highly uncertain and subject to continued change, and we recognize that they may continue to present unique challenges for us.

We continue to believe that our existing cash, cash equivalents and investments and existing sources of and access to financing are adequate to satisfy our needs for working capital, capital expenditures, debt service requirements and other business development initiatives that we plan to strategically pursue in the 12 months from the issuance of the unaudited condensed consolidated financial statements included in this Quarterly Report on Form 10-Q. However, should adverse geopolitical and macroeconomic events and any associated recession or depression continue for a prolonged period, our results of operations, financial condition, liquidity and cash flows could be materially impacted as a result of a lower likelihood of effectively and efficiently developing new medicines and successfully commercializing our products.

License Agreements

Assignment and License Agreement with Shire (Takeda)

In November 2018, we entered into the Shire License Agreement with Shire, which was subsequently acquired by Takeda, in which we were granted an exclusive, royalty bearing worldwide license to develop and commercialize our two product candidates, Livmarli and volixibat. As part of the Shire License Agreement, we were assigned license agreements held by Shire with Satiogen, Pfizer, and Sanofi (collectively, “Assigned License Agreements”).

Under the Shire License Agreement and Assigned License Agreements, to date, we have paid aggregate development regulatory and sales milestones of \$66.0 million related to our Livmarli and volixibat programs.

Satiogen Acquisition

In May 2022, we completed the acquisition of Satiogen for total consideration of \$24.2 million. At the closing of the acquisition, Satiogen’s assets consisted of cash and intangible assets related to developed technology. Through the transaction, we obtained all Satiogen-owned intellectual property relating to Livmarli and volixibat. The transaction resulted in a reduction of total licensing royalty obligations for Livmarli and volixibat.

The total potential consideration for the acquisition consisted of 841,792 shares of common stock and approximately \$2.6 million in cash, excluding \$0.2 million of stock option exercise prices deemed to have been paid immediately prior to the acquisition, in respect of an equivalent amount of cash on the books of Satiogen acquired by us at closing.

Components of Results of Operations

Revenue

Product Sales, Net

Our approved product, Livmarli, has been approved for the treatment of cholestatic pruritus in patients with ALGS three months of age and older in the United States and for the treatment of cholestatic pruritus in patients with ALGS two months of age and older in Europe. We expect our product sales of Livmarli will continue to increase on an annual basis, though seasonality in demand and international product ordering may result in quarterly fluctuations.

Our U.S. revenue from product sales, net further depends on our prescription mix of commercial payors, Medicaid and free drugs under our patient assistance program. We expect our prescription mix and resulting gross to net adjustment in the U.S. to remain consistent; however, we cannot reliably estimate it given our limited experience in the market. Our rest of world revenue from product sales, net primarily depends on our contractual obligations with our distributors and results of pricing negotiations with governmental authorities in certain European countries where we launch Livmarli and expect to launch our other product candidates, if approved. In addition, in certain countries, governments place large periodic orders. The timing of these orders can be inconsistent and can create quarter-to-quarter variation in revenue.

License Revenue

Under the exclusive licensing agreements with CANbridge and GC Biopharma, we have recognized as revenue the upfront nonrefundable payments related to the licenses granted upon satisfaction of certain performance obligations. Pursuant to the agreements, we are eligible to receive future milestone payments. These milestone payments are fully constrained and will be recognized in revenue in the period when it is probable that a significant reversal of cumulative revenue recognized for the contract would not occur. We are also eligible to receive royalty payments related to the agreements, which will be recognized as the underlying product sales occur.

Our license revenue is dependent upon our licensees’ achievement of future milestones, which are not within our control, and we are unable to reliably estimate the timing of such revenues.

Cost of Sales

Cost of sales consist of third party manufacturing costs, personnel, facility and other costs of manufacturing commercial products, transportation and freight, amortization of finite-lived intangible assets, amortization of capitalized intangibles associated with contractual milestone payments paid to licensors upon certain regulatory approval and sales-based events and royalty payments payable on net sales of Livmarli under licensing agreements. Cost of sales may also include period costs related to certain manufacturing services and inventory adjustment charges. Prior to receiving approval from the FDA in September 2021 to market and sell Livmarli in the United States, we expensed all costs incurred related to the manufacture of Livmarli as research and development expense because of the inherent risks associated with the development of a drug candidate, the uncertainty about the regulatory approval process and the lack of history as a company of regulatory approval of drug candidates. Our inventory of Livmarli produced prior to FDA approval is available for commercial or clinical use.

Operating Expenses

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 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;
- research and development funding for collaboration arrangements;
- 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.

Our research and development expense may increase in the future as we continue to develop our current product candidates and look to acquire and develop additional product candidates.

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 consisted 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 Livmarli in the United States 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 and accounting activities.

Loss from termination of revenue interest purchase agreement

Loss from termination of RIPA is related to the repurchase and termination of the RIPA in the second quarter of 2023. The repurchase payment included a premium over the remaining revenue interest liability and resulted in the recognition of a loss from termination of RIPA in our condensed consolidated statements of operations and comprehensive loss.

Interest Income

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

Interest Expense

We incur interest expense on our convertible notes and on the RIPA. Interest on our convertible notes consists of a 4% per annum fixed rate of interest and amortization of debt discount and amortization costs. Interest on the RIPA consisted primarily of costs associated with our liability and non-cash interest costs associated with the amortization of the related debt discount and deferred issuance costs. Prior to extinguishment of the RIPA in the second quarter of 2023, we imputed interest expense associated with this liability using the effective interest rate method which is calculated based on the rate that would enable the debt to be repaid in full over the anticipated life of the arrangement.

Other Expense, Net

Other expense, net consists of gain or loss from remeasurement of the liabilities associated with the common stock to be issued in connection with the acquisition of Satiogen after the satisfaction of certain purchase price adjustments and indemnification obligations that may arise during the 12 month period following the asset acquisition date (“Indemnification Holdback”) and the liability associated with the common stock to be issued in connection with the acquisition of Satiogen as contingent consideration upon achievement of a certain milestone (“Contingent Milestone”) both of which were recorded in connection with our acquisition of Satiogen and transactional currency exchange gain or loss.

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, including the amount of assets, liabilities, expenses and the disclosure of contingent assets and liabilities. 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 six months ended June 30, 2023 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.

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 June 30, 2023 and 2022

The following table summarizes our results of operations for the three months ended June 30, 2023 and 2022 (in thousands):

	Three Months Ended June 30,		
	2023	2022	Change
Revenue:			
Product sales, net	\$ 32,497	\$ 17,484	\$ 15,013
License revenue	5,000	—	5,000
Total revenue	37,497	17,484	20,013
Operating expenses:			
Cost of sales	\$ 6,812	\$ 2,524	\$ 4,288
Research and development	22,009	25,432	(3,423)
Selling, general and administrative	32,949	20,969	11,980
Total operating expenses	61,770	48,925	12,845
Loss from operations	(24,273)	(31,441)	7,168
Other income (expense):			
Interest income	3,627	293	3,334
Interest expense	(3,726)	(3,875)	149
Change in fair value of derivative liability	—	232	(232)
Loss from termination of revenue interest purchase agreement	(49,076)	—	(49,076)
Other (expense) income, net	(274)	1,299	(1,573)
Net loss before provision for (benefit from) for income taxes	(73,722)	(33,492)	(40,230)
Provision for (benefit from) income taxes	316	(6,570)	6,886
Net loss	\$ (74,038)	\$ (26,922)	\$ (47,116)

Product Sales, Net

Product sales, net was \$32.5 million for the three months ended June 30, 2023, compared to \$17.5 million for the three months ended June 30, 2022. The increase in product sales, net was a result of our continued commercialization of Livmarli in the United States, and, to a lesser extent, in certain countries in Europe and other international sales through partner market supply orders.

License Revenue

License revenue was \$5.0 million for the three months ended June 30, 2023 due to the achievement of a regulatory milestone by CANbridge associated with our license agreement, compared to zero for the three months ended June 30, 2022.

Cost of Sales

For the three months ended June 30, 2023, cost of sales was \$6.8 million, compared to \$2.5 million for the three months ended June 30, 2022. The increase in cost of sales was primarily a result of increased royalties payable on net sales of Livmarli under licensing agreements, amortization of acquired intangibles associated with our acquisition of Satiogen and commercial milestone payments, increased commercial product development costs and costs associated with excess and obsolete inventory.

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 June 30,		
	2023	2022	Change
<i>Product-specific costs:</i>			
Livmarli	\$ 5,639	\$ 9,746	\$ (4,107)
Volixibat	4,633	4,840	(207)
<i>Non product-specific costs:</i>			
Stock-based compensation	2,671	2,416	255
Personnel	6,894	5,908	986
Other	2,172	2,522	(350)
Total research and development expenses	\$ 22,009	\$ 25,432	\$ (3,423)

Research and development expenses were \$22.0 million for the three months ended June 30, 2023, a decrease of \$3.4 million compared to the three months ended June 30, 2022. The decrease was primarily due to:

- for Livmarli programs, a decrease of \$4.1 million, due to decreases of \$2.7 million in clinical trial expenses primarily for MARCH Phase 3 clinical trial, which was completed during 2022 and \$1.1 million in manufacturing development expenses;
- for volixibat programs, a decrease of \$0.2 million, primarily due to discontinuation of the intrahepatic cholestasis of pregnancy clinical trial; and
- for other expense, a decrease of \$0.4 million, primarily due to other general research and development activities; partially offset by
- an increase of \$1.0 million for personnel related expenses, related to an increase in employee headcount to support our development pipeline.

Selling, General and Administrative Expenses

Selling, general and administrative expenses were \$32.9 million for the three months ended June 30, 2023, an increase of \$12.0 million compared to the three months ended June 30, 2022. The increase was primarily due to an increase of \$5.3 million in personnel and other compensation related expenses, including an increase of \$1.5 million in stock-based compensation, reflecting an increase in the number of our selling, marketing and administrative employees to support commercial activities for Livmarli and increased requirements of operating as a public company, an increase of \$2.9 million in expenses primarily related to legal, public relations and international expansion activities, an increase of \$1.8 million in advertising and promotion expenses associated with commercial activities for Livmarli, and an increase of \$1.7 million in other general administrative expenses.

Loss from termination of revenue interest purchase agreement

Loss from termination of RIPA was \$49.1 million for the three months ended June 30, 2023, compared to zero for the three months ended June 30, 2022. The loss was related to the premium paid to repurchase the revenue interests pursuant to the RIPA.

Interest Income

Interest income was \$3.6 million for the three months ended June 30, 2023, an increase of \$3.3 million compared to the three months ended June 30, 2022. The increase was primarily due to higher interest earned on our cash equivalents and investment balances largely due to higher money market and treasury yields as well as increased invested balances.

Interest Expense

Interest expense was \$3.7 million for the three months ended June 30, 2023, a decrease of \$0.1 million compared to the three months ended June 30, 2022. For the three months ended June 30, 2023, interest expense consisted of \$2.9 million related to our convertible notes issued during the period and \$0.8 million related to the RIPA prior to extinguishment. For the three months ended June 30, 2022, interest expense of \$3.9 million was related to the RIPA.

Other Expense, Net

Other expense, net was \$0.3 million expense for the three months ended June 30, 2023, compared to other income, net of \$1.3 million for the three months ended June 30, 2022. The change was primarily related to the remeasurement of the Indemnification Holdback liability upon issuance of the underlying common stock and reflects an increase in the fair value of our common stock price during the period.

Change in Provision For (Benefit From) Income Taxes

Provision for income taxes was \$0.3 million for the three months ended June 30, 2023, compared to benefit from income taxes of \$6.6 million for the three months ended June 30, 2022. The change was related to the deferred tax liability recognized as a result of intangible assets acquired as part of the Satiogen acquisition and corresponding reduction in our valuation allowance against our net deferred tax assets during the three months ended June 30, 2022.

Results of Operations for the Six Months Ended June 30, 2023 and 2022

The following table summarizes our results of operations for the six months ended June 30, 2023 and 2022 (in thousands):

	Six Months Ended June 30,		
	2023	2022	Change
Revenue:			
Product sales, net	\$ 61,595	\$ 28,376	\$ 33,219
License revenue	7,500	2,000	5,500
Total revenue	69,095	30,376	38,719
Operating expenses:			
Cost of sales	11,791	\$ 4,948	\$ 6,843
Research and development	45,557	49,520	(3,963)
Selling, general and administrative	63,168	40,085	23,083
Total operating expenses	120,516	94,553	25,963
Loss from operations	(51,421)	(64,177)	12,756
Other income (expense):			
Interest income	5,899	362	5,537
Interest expense	(7,968)	(7,649)	(319)
Change in fair value of derivative liability	—	232	(232)
Loss from termination of revenue interest purchase agreement	(49,076)	—	(49,076)
Other income (expense), net	(1,085)	1,145	(2,230)
Net loss before provision for (benefit from) for income taxes	(103,651)	(70,087)	(33,564)
Provision for (benefit from) income taxes	517	(6,559)	7,076
Net loss	\$ (104,168)	\$ (63,528)	\$ (40,640)

Product Sales, Net

Product sales, net was \$61.6 million for the six months ended June 30, 2023, compared to \$28.4 million for the six months ended June 30, 2022. The increase in product sales, net was a result of our continued commercialization of Livmarli in the United States, and, to a lesser extent, in certain countries in Europe and other international sales through partner market supply orders.

License Revenue

License revenue was \$7.5 million for the six months ended June 30, 2023 due to the achievement of regulatory milestones by CANbridge and GC Biopharma associated with our license agreements, compared to \$2.0 million for the six months ended June 30, 2022 due to the achievement of a regulatory milestone by CANbridge associated with our license agreement.

Cost of Sales

For the six months ended June 30, 2023, cost of sales was \$11.8 million, compared to \$4.9 million for the six months ended June 30, 2022. The increase in cost of sales was primarily a result of increased royalties payable on net sales of Livmarli under licensing agreements, amortization of acquired intangibles associated with our acquisition of Satiogen and commercial milestone payments, increased commercial product development costs and costs associated with excess and obsolete inventory.

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):

	Six Months Ended June 30,		
	2023	2022	Change
Product-specific costs:			
Livmarli	\$ 12,878	\$ 19,219	\$ (6,341)
Volixibat	9,480	8,991	489
Non product-specific costs:			
Stock-based compensation	5,387	5,001	386
Personnel	13,198	11,725	1,473
Other	4,614	4,584	30

Total research and development expenses	\$ 45,557	\$ 49,520	\$ (3,963)
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Research and development expenses were \$45.6 million for the six months ended June 30, 2023, a decrease of \$4.0 million compared to the six months ended June 30, 2022. The decrease was primarily due to:

- for Livmarli programs, a decrease of \$6.3 million, primarily due to decreases of \$3.4 million in clinical trial expenses primarily for MARCH Phase 3 clinical trial, which was completed during 2022, \$1.6 million in manufacturing development expenses, \$0.6 million in general development costs and \$0.5 million in non-clinical expenses; partially offset by
- for volixibat programs, an increase of \$0.5 million, primarily due to an increase of clinical trial expenses for PSC and PBC; and
- for personnel related expenses, an increase of \$1.5 million, related to an increase in employee headcount to support our development pipeline.

Selling, General and Administrative Expenses

Selling, general and administrative expenses were \$63.2 million for the six months ended June 30, 2023, an increase of \$23.1 million compared to the six months ended June 30, 2022. The increase was primarily due to an increase of \$12.0 million in personnel and other compensation related expenses, including an increase of \$3.3 million in stock-based compensation, reflecting an increase in the number of our selling, marketing and administrative employees to support commercial activities for Livmarli and increased requirements of operating as a public company, an increase of \$5.2 million in expenses primarily related to legal, public relations, accounting and international expansion activities, an increase of \$2.8 million in advertising and promotion associated with commercial activities for Livmarli, and an increase of \$2.7 million in other general administrative expenses.

Loss from termination of revenue interest purchase agreement

Loss from termination of RIPA was \$49.1 million for the six months ended June 30, 2023, compared to zero for the six months ended June 30, 2022. The loss was related to the premium paid to repurchase the revenue interests pursuant to the RIPA.

Interest Income

Interest income was \$5.9 million for the six months ended June 30, 2023, an increase of \$5.5 million compared to the six months ended June 30, 2022. The increase was primarily due to higher interest earned on our cash equivalents and investment balances largely due to higher money market and treasury yields as well as an increase in invested balances.

Interest Expense

Interest expense was \$8.0 million for the six months ended June 30, 2023, an increase of \$0.3 million compared to the six months ended June 30, 2022. For the six months ended June 30, 2023, interest expense consisted of \$2.9 million related to our convertible notes issued during the period and \$5.1 million related to the RIPA prior to extinguishment. For the six months ended June 30, 2022, interest expense of \$7.7 million was related to the RIPA.

Other Expense, Net

Other expense, net was \$1.1 million expense for the six months ended June 30, 2023, compared to other income, net of \$1.1 million for the six months ended June 30, 2022. For the six months ended June 30, 2023, the expense was primarily related to the remeasurement of the Contingent Milestone liability and the Indemnification Holdback liability prior to issuance of the underlying common stock during the six-month period ended June 30, 2023 and reflects an increase in the fair value of our common stock price during the period. For the six months ended June 30, 2022, the income was primarily related to the remeasurement of the Contingent Milestone liability and the Indemnification Holdback liability as of June 30, 2022 and reflects a decrease in the fair value of our common stock price as of June 30, 2022.

Change in Provision For (Benefit From) Income Taxes

Provision for income taxes was \$0.5 million for the six months ended June 30, 2023 compared to benefit from income taxes of \$6.6 million for the six months ended June 30, 2022. The change was related to the deferred tax liability recognized as a result of intangible assets acquired as part of the Satiogen acquisition and corresponding reduction in our valuation allowance against our net deferred tax assets during the six months ended June 30, 2022.

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 \$330.0 million of cash, cash equivalents and investments as of June 30, 2023, compared to cash, cash equivalents, restricted cash equivalents and investments of \$251.7 million as

of December 31, 2022. Since inception, we have incurred operating losses and negative cash flows from operations. As of June 30, 2023, we had an accumulated deficit of \$497.0 million, compared to \$392.8 million as of December 31, 2022.

On July 16, 2023, we executed the Purchase Agreement with Travers. Subject to the terms and conditions of the Purchase Agreement, at the Closing of the Asset Purchase, we will purchase from Travers substantially all of the assets of Travers that are primarily related to its business of development, manufacture (including synthesis, formulation, finishing or packaging) and commercialization of the Products for an aggregate purchase price, subject to certain adjustments pursuant to the terms of the Purchase Agreement, of up to \$445.0 million in cash, with \$210.0 million due at the Closing and up to \$235.0 million after the Closing, upon the achievement of certain milestones based on specified amounts of annual net sales (tiered from \$125.0 million to \$500.0 million) of the Products. Concurrently with the execution and delivery of the Purchase Agreement, certain parties have entered into Subscription Agreements with us, pursuant to which they have agreed, subject to the terms and conditions of the Subscription Agreements, to purchase immediately prior to, but subject to, the consummation of the Asset Purchase, shares of our common stock for an aggregate purchase price of approximately \$210.0 million at a price per share equal to \$26.25, which we will use to finance the upfront payment at the Closing of the Asset Purchase.

On April 17, 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 terms of the convertible Notes are further described in Note 10 to our unaudited condensed consolidated financial statements. We used a portion of the net proceeds to repurchase the revenue interests from the Purchasers at a call price of \$192.7 million. Upon repurchase of the revenue interests from the Purchasers, the RIPA, in accordance with its terms, was terminated with no further payments due to the Purchasers and the \$100.0 million of our previously restricted cash equivalents was no longer restricted from use.

In September 2022, we filed an automatic shelf registration statement on Form S-3 with the SEC, which became effective upon filing, pursuant to which we registered 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 June 30, 2023, we have not issued any securities pursuant to the automatic shelf registration statement. We may lose well-known seasoned issuer status in the future upon the occurrence of certain events prescribed by the Securities Act or the rules and regulations of the SEC occurring after we file this Quarterly Report if, at the time of those certain events, the worldwide market value of our voting and non-voting common equity held by our non-affiliates does not equal \$700.0 million or more, calculated as of a date within 60 days prior to such events. If that were to occur and we were no longer considered a well-known seasoned issuer, we anticipate needing to amend our automatic shelf registration statement on Form S-3 prior to the occurrence of such events in order to sell securities under that Form S-3 on an ongoing basis.

In August 2022, we completed an underwritten public offering of our common stock pursuant to the Shelf Registration. We sold 3,478,261 shares of common stock at a price to the public of \$23.00 per share. In addition, we granted the underwriters an option, exercisable for 30 days, to purchase up to 521,739 additional shares of our common stock at the public offering price, less the underwriting discounts, commissions and offering expenses, which the underwriters exercised in full. The underwritten public offering, including the underwriters' exercise of their option, resulted in net proceeds to us of \$86.1 million after deducting underwriting discounts, commissions and offering expenses.

In August 2020, the SEC declared effective the Shelf Registration covering the sale of up to \$300.0 million of our securities. Also, in August 2020, we entered into the Sales Agreement with SVB Securities pursuant to which we may elect to issue and sell, in an at the market offering, from time to time, shares of common stock having an aggregate offering price of up to \$75.0 million under the Shelf Registration through SVB Securities acting as the sales agent and/or principal. During the six months ended June 30, 2023, we issued and sold 658,206 shares of common stock pursuant to the Sales Agreement resulting in aggregate gross proceeds to us of \$15.0 million. The net proceeds for the six months ended June 30, 2023, to us after deducting sales commissions to SVB Securities and other issuance expenses were approximately \$14.5 million. As of June 30, 2023, we have issued and sold an aggregate of 2,125,090 shares of common stock pursuant to the Sales Agreement, resulting in aggregate gross proceeds to us of \$43.7 million. The remaining capacity under the Sales Agreement is approximately \$31.3 million as of June 30, 2023.

Based on our current and anticipated level of operations and cash generated from sales of Livmarli, including the expected net proceeds from the Private Placement (with respect to the upfront payment due at the Closing of the Asset Purchase), we believe our existing 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. Our cash, cash equivalents and investments include money market funds, government agency securities and commercial paper. We maintain established guidelines relating to diversification and maturities of our investments to preserve principal and maintain liquidity.

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 Livmarli and potentially expand into additional

markets, hire additional staff, including clinical, scientific, operational, financial and management personnel and pay potential development and commercial milestones in connection with our license agreements.

Our primary use of cash is to fund operating expenses. Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses.

Although Livmarli has been approved for the treatment of cholestatic pruritus in patients with ALGS in patients three months of age and older in the U.S. and for the treatment of cholestatic pruritus in patients two months of age and older in Europe, and we expect product revenues to increase as we continue commercial activities, Livmarli may not achieve commercial success. Our principal sources of liquidity are cash from the sale of our investments, product revenue from sales of Livmarli, and, to a lesser extent, from our collaboration agreements. Until such time, if ever, as we can generate sufficient product revenue from sales of Livmarli, our current product candidates or any future product candidates, if approved, to fund operations, we 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. As a result of adverse geopolitical and macroeconomic developments, such as the COVID-19 pandemic, actions taken to slow its spread, the ongoing military conflict between Ukraine and Russia and related sanctions, actual and anticipated changes in interest rates, economic inflation and the responses by central banking authorities to control such inflation, bank failures, the global credit and financial markets have experienced 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 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.

Contractual Obligations

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

On April 17, 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. Our first interest payment is due on November 1, 2023. 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 10 to our unaudited condensed consolidated financial statements.

We used a portion of the net proceeds from the Notes to repurchase the revenue interests from the Purchasers at a call price of \$192.7 million. Upon repurchase of the revenue interests from the Purchasers, the RIPA, in accordance with its terms, was terminated with no further payments due to the Purchasers.

Under the Shire License Agreement, 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 7 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 9 to our unaudited condensed consolidated financial statements.

We are party to certain license and collaboration agreements, which contain a number of contractual obligations. Those contractual obligations may entitle us to receive, or may obligate us to make, certain payments. The amount and timing of those payments are unknown or uncertain as we are unable to estimate the timing or likelihood of the events that will obligate those payments.

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 cancelable contracts.

Cash Flows

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

	Six Months Ended June 30,	
	2023	2022
Net cash used in operating activities	\$ (36,105)	\$ (59,841)
Net cash provided by investing activities	77,562	60,471
Net cash provided by financing activities	127,694	23,350
Effect of exchange rate on cash, cash equivalents and restricted cash equivalents	74	(52)
Net increase in cash, cash equivalents and restricted cash equivalents	\$ 169,225	\$ 23,928

Net Cash Used in Operating Activities

Net cash used in operating activities was \$36.1 million for the six months ended June 30, 2023, reflecting our net loss of \$104.2 million partially offset by non-cash adjustments of \$74.2 million. Non-cash adjustments consisted primarily of loss from termination of RIPA of \$49.1 million, \$16.9 million of stock-based compensation, \$5.1 million of effective interest expense in connection with the RIPA, \$3.0 million of depreciation and amortization of our intangible assets and fixed assets and non-cash lease expense, \$0.7 million expense related to the change in fair value of contingent liabilities associated with an acquisition and a \$0.7 million charge associated with excess and obsolete inventory, offset by \$1.5 million net accretion of discounts on our investments. Additionally, cash used in operating activities reflected changes in net operating assets of \$6.1 million, consisting primarily of a \$11.3 million increase in accounts receivable related to sales of Livmarli, a \$8.0 million increase in accounts payable, accrued expenses and other liabilities resulting primarily from an increase in accounts payable for clinical and sales and marketing expenses, a \$0.8 increase in prepaid and other current assets, a \$1.8 million increase in inventory, a \$0.4 million decrease in operating lease liability and a \$0.2 million decrease in other assets.

Net cash used in operating activities was \$59.8 million for the six months ended June 30, 2022, reflecting our net loss of \$63.5 million partially offset by non-cash adjustments of \$14.0 million. Non-cash adjustments consisted primarily of \$13.4 million of stock-based compensation, \$7.7 million of effective interest expense in connection with the RIPA, \$6.6 million related to deferred tax liability recognized as a result of intangible assets acquired as part of the Satiogen acquisition, \$1.3 million gain related to the change in fair value of Indemnification Holdback and Contingent Milestone liabilities, \$0.9 million of depreciation and amortization of our fixed assets and operating lease right-of use assets and \$0.2 million related to the change in fair value of the derivative liability. Additionally, cash used in operating activities reflected changes in net operating assets of \$10.3 million, consisting primarily of a \$10.7 million increase in accounts receivable related to sales of Livmarli, a \$1.2 million increase in inventory, and a \$0.3 million decrease in operating lease liability, partially offset by a \$1.5 million decrease in prepaid expenses and other current assets and a \$0.8 million decrease in accounts payable.

Net Cash Provided by Investing Activities

Net cash provided by investing activities was \$77.6 million for the six months ended June 30, 2023, primarily due to proceeds of \$120.0 million from maturities of investments, partially offset by \$27.3 million used in purchases of investments and \$15.0 million used in the acquisition of intangible assets associated with our license payment related to approval of Livmarli by the European Commission for the treatment of cholestatic pruritus in patients with ALGS two months of age and older.

Net cash provided by investing activities was \$60.5 million for the six months ended June 30, 2022, due to \$75.3 million from maturities of investments offset by purchased investments of \$14.8 million.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was \$127.7 million for the six months ended June 30, 2023, due to net proceeds of \$305.3 million from the issuance of convertible notes, \$14.5 million from the issuance and sale of common stock under the Sales Agreement with SVB Securities, pursuant to which we issued and sold an aggregate of 658,206 shares of common stock at a weighted-average price of \$22.79 per share, and proceeds of \$3.5 million from employee equity award exercises, partially offset by \$195.6 million in revenue interest payments and payments to repurchase the revenue interests under the RIPA.

Net cash provided by financing activities was \$23.4 million for the six months ended June 30, 2022, due to net proceeds of \$21.3 million from the issuance and sale of common stock under the Sales Agreement with SVB Securities, pursuant to which we issued and sold an aggregate of 1,160,915 shares of common stock at a weighted-average price of \$19.01 per share, and proceeds of

\$3.9 million from employee equity award exercises, partially offset by \$1.9 million of revenue interest payments made under the RIPA.

JOBS Act

As an emerging growth company under the Jumpstart Our Business Startups Act of 2012 (“JOBS Act”), we can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies. We intend to rely on other exemptions provided by the JOBS Act, including without limitation, not being required to comply with the auditor attestation requirements of Section 404(b) of the Sarbanes-Oxley Act of 2002, as amended (“Sarbanes-Oxley Act”).

As of June 30, 2023, the market value of our common stock held by non-affiliates exceeded \$700.0 million. As a result, we will be a large accelerated filer and thus will cease to be an emerging growth company effective December 31, 2023. Additionally, we will no longer qualify as a smaller reporting company beginning with our first Quarterly Report on Form 10-Q for the quarterly period ending March 31, 2024.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

Interest Rate Risk

Our cash, cash equivalents and investments as of June 30, 2023 consist of readily available checking, money market funds, and investments. The primary objective of our investment activities is to preserve our capital to fund operations. We 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. Although we are seeing, and expect to continue to see, increased interest rates, due to the strategies we employ, 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 due to the short-term nature of the instruments in our portfolio and the low risk profile of our investments. For example, a hypothetical change in interest rates of 100 basis points would not have a material impact on the fair market value of our cash equivalents and investments as of June 30, 2023. In addition, we maintain significant amounts of cash and cash equivalents at one financial institution that is in excess of federally insured limits.

Foreign Currency Rate Risk

Our operations include activities in the U.S., Switzerland and certain other countries in Europe. While our operating results are exposed to changes in foreign currency exchange rates between the U.S. dollar and various foreign currencies, the most significant of which are the Swiss Franc and the Euro, as of June 30, 2023 and December 31, 2022, we had minimal assets and liabilities denominated in foreign currencies and, as a result, changes in foreign currency exchange rates did not have a material impact on our business, financial condition or results of operations for the six months ended June 30, 2023.

Effects of Inflation

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation and changing prices had a significant impact on our results of operations for any periods presented herein.

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 June 30, 2023 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 Products and Development of our Product Candidates

Livmarli is currently our only FDA and EMA-approved product and the success of our business depends, in part, on our ability to market and sell Livmarli profitably.

Livmarli is approved by the FDA for the treatment of cholestatic pruritus in patients with ALGS three months of age and older and by the EMA for the treatment of cholestatic pruritus in patients with ALGS two months of age and older. The success of our business depends, in part, on our ability to market and sell Livmarli profitably. Our successful commercialization of Livmarli depends on a number of factors, including, among others, the following:

- our ability to grow and maintain our sales team and scale our distribution capabilities in the United States (“U.S.”) and certain countries in Europe and Canada;
- the availability of adequate reimbursement for Livmarli and a commercially viable sales price of Livmarli;
- acceptance by physicians, payors and patients of the benefits, safety and efficacy of Livmarli, including relative to alternative and competing treatments;
- a continued acceptable safety profile of Livmarli;
- the effect of recent or potential health care legislation and regulatory changes in the U.S. and the European Union (“EU”);
- our ability to successfully obtain the substances and materials used in manufacturing Livmarli 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 Livmarli and avoid third-party patent interference or intellectual property infringement claims; and
- sufficient patient population that would benefit from Livmarli as ALGS is a rare disease and 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 Livmarli 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 Livmarli, we may not be able to generate viable Livmarli revenues.

To successfully commercialize Livmarli 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. The majority of pediatric cholestatic patients are treated at tertiary care centers and transplant centers and therefore can be addressed with a targeted sales force. We have established our own commercial capabilities in the U.S. to target these centers. We are also in the process of further establishing our capabilities 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 Livmarli and our other 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 of our own sales force to market Livmarli 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 Livmarli, 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 Livmarli 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 Livmarli and our other product candidates, if approved, 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 Livmarli 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, is critical to the commercial success of Livmarli in the U.S. and in international markets. Coverage for Livmarli 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 or limit changes in list price;
- changes in federal, state or foreign government regulations or private third-party payors' reimbursement policies;
- 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 Livmarli 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 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 Livmarli 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 Member States of the EU, 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. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels. For instance, governmental authorities in the EU and third-party payors could base pricing and reimbursement terms on what they perceive to be comparable products, even if approved for different indications. These pricing and reimbursement decisions may impact the pricing and reimbursement of Livmarli in such jurisdictions. 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.

Historically, products launched in the EU and other foreign countries do not follow price structures of the U.S. and generally prices tend to 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 Livmarli 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 products or any future product candidates, if approved, in those countries would be negatively affected.

Livmarli 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 Livmarli 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 Livmarli or our then-approved products, 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 Livmarli or our then-approved product candidates are safe and effective treatments;
- physician and patient willingness to adopt a new therapy over other available therapies, including, for Livmarli, to treat cholestatic pruritus in patients with ALGS;
- limitations, warnings or adverse drug reactions contained in the labeling or product inserts approved by the FDA, EMA or comparable 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 Livmarli or our then-approved product candidates are approved for;
- patients and caregivers properly using Livmarli or our then-approved product candidates as instructed;
- the prevalence and severity of side effects from the use or potential misuse of Livmarli or our then-approved product candidates;
- 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 Livmarli or our then-approved product candidates and the processes required to place orders with those pharmacies;
- the ability of our patient services hub to provide adequate support for patients and physicians to prescribe and access Livmarli and our then-approved product candidates;
- the timing of market introduction of Livmarli and any then-approved product 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 Livmarli or our then-approved product candidates 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
- for Livmarli, patient satisfaction leading to a high percentage of patients deriving clinical benefit and staying on Livmarli chronically in the real world setting, as has been seen in clinical trials.

If Livmarli or any of our then-approved product candidates fails 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.

Livmarli and our product candidates may cause undesirable side effects or have other properties that could limit Livmarli's 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 our product candidates' use. 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 by the FDA, EMA or comparable 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, 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, gastrointestinal bleeding and bone fracture. The frequency of observed AEs has not increased over time. In clinical trials of volixibat, the most common AEs reported were mild to moderate GI events observed in the volixibat groups.

Additionally, in respect of Livmarli or if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by Livmarli 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 approvals of such product, including the FDA or EMA withdrawing approval for Livmarli;
- 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 institute a REMS;
- 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 Livmarli or the affected product;
- our ability to promote Livmarli and any then-approved product 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 Livmarli and any then-approved product may suffer.

Such events could prevent us from achieving or maintaining market acceptance of Livmarli or the particular product candidate, if approved, 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, President Biden signed the American Rescue Plan Act of 2021 into law, which eliminates 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, beginning 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 Livmarli and our product candidates. Livmarli 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 Livmarli 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 Livmarli 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 product 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 Livmarli or our product candidates, if approved;
- decreased demand for Livmarli or our 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 Livmarli, or unapproved uses of our other product candidates, if approved, or if physicians misuse or use off-label Livmarli or our other 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, EMA and comparable 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, EMA or comparable 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 suffer from other forms of cholestatic pruritus, we 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 in the EU for the treatment of cholestatic pruritus in patients with ALGS two months of age and older. If we are found to have promoted off-label uses of Livmarli or our other product candidates, if approved, we may receive warning or untitled letters and become subject to significant criminal and civil liability, which would materially harm our business. Further, 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.

Livmarli 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. In addition, if the FDA, EMA or comparable 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 EMA 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 cGMP requirements and with the FDA's good clinical practice ("GCP").

In addition, Livmarli was the subject of a marketing authorization granted by the European Commission in accordance with Article 14.8 of the EMA Regulations under exceptional circumstances. This type of authorization is reviewed annually to reassess the risk-benefit balance of the medicinal product. 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 for a renewable period of one year and will normally not lead to the completion of a full dossier/approval. In addition, we are subject to FDA and EMA post-marketing requirements including the conduct and submission of non-clinical, clinical and registry studies and the FDA's and EMA's prohibition against marketing Livmarli for uses that are not approved by the FDA and EMA, respectively, as reflected in Livmarli's approved labeling. These and similar requirements could be imposed by the FDA, EMA or comparable 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, EMA or comparable regulatory authorities for compliance with cGMP regulations. If we or a regulatory agency 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 EMA 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 Livmarli 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, EMA's and comparable 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 Livmarli and 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.

We may pursue approval in the U.S. or certain countries in Europe using accelerated approval, 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, EMA or comparable 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 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 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, EMA or comparable 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, EMA or comparable 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 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. For example, the FDA typically requires results from two well controlled Phase 3 clinical trials to support an NDA submission seeking approval to market a new drug. We believe that the positive results from our single Phase 3 MARCH clinical trial, will be sufficient to support our NDA supplement submitted February 2023 seeking approval for Livmarli for the treatment of cholestatic pruritus associated with PFIC. In addition, in April 2023, we submitted a variation application and data from our MARCH Phase 3 clinical trial for PFIC to the EMA seeking to extend the label for Livmarli to include the treatment of PFIC in patients two months of age or older. However, the FDA or the EMA may require additional Phase 3 trials in order to expand the label or may reject expansion altogether.

To the extent that the results of our clinical trials are not satisfactory to the FDA, EMA or comparable 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.

Further, 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 are also conducting clinical trials in countries that have not yet had Livmarli or volixibat trials conducted and we have not yet worked with such foreign regulatory authorities. As a result, we could face patient recruitment issues in certain countries where such foreign regulatory authorities are not familiar with Livmarli or volixibat. Additionally, other pharmaceutical companies targeting these same cholestatic liver diseases are recruiting clinical trial patients from these patient populations, and have expanded access programs available, which may delay or make it more difficult to fully enroll 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. Clinical trials often fail to demonstrate safety and efficacy of the product candidate studied for the target indication. Most product candidates that commence clinical trials are never approved by regulatory authorities for commercialization. In the case of Livmarli and volixibat, we are seeking to develop treatments for rare cholestatic liver 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. Importantly, because the measure of pruritus relies on subjective patient feedback, it is inherently difficult to evaluate, and is subject to placebo effect. It can be influenced by factors outside of our control and can vary widely from day to day for a particular patient, and from patient to patient and site to site within a clinical trial. The placebo effect may also have a significant impact on pruritus trials.

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. For example, volixibat has been evaluated primarily for the treatment of non-alcoholic steatohepatitis and has not been evaluated in PSC or PBC, and our clinical development strategy is predicated on observations of IBAT inhibition in cholestatic settings. Similarly, Livmarli has not yet been evaluated in BA. As such, our hypothesis of efficacy in these diseases will be evaluated in these target patient populations and may not show the desired clinical results. 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 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 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 regulatory authorities disagreeing as to the design or implementation of our clinical trials or agreement to commence our clinical trials;
- the FDA's, EMA's or comparable 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");
- IRBs refusing to approve, 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, EMA or comparable 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 or comparable 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, EMA or comparable regulatory authorities. The FDA, EMA or comparable 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, EMA or comparable 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, EMA or comparable 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 EMA in the EU and by comparable regulatory authorities in other markets. In the U.S., we are not permitted to market our product candidates until we receive regulatory approval from the FDA. 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 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, EMA or comparable regulatory authorities. The FDA, EMA or comparable 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. For example, we recently announced positive results from our Phase 3 MARCH clinical trial of Livmarli in PFIC, and we have submitted an sNDA for Livmarli to the FDA in February 2023 for the treatment of cholestatic pruritus associated with PFIC and a marketing authorization variation to the EMA in April 2023 for Livmarli in the treatment of PFIC. However, the FDA or the EMA may not agree with our interpretation of the results and may not grant approval.

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

- the FDA, EMA or comparable 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 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.;
- we may be unable to demonstrate to the satisfaction of the FDA, EMA or comparable 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 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 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 regulatory authorities to support the submission of an NDA or other comparable submissions in 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 EMA;
- the FDA, EMA or comparable 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, EMA or comparable 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, EMA or comparable 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 foreign marketing application for our product candidates, the FDA, EMA or comparable 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, which may be required to ensure safe use of the drug after approval. For example, for Livmarli we have submitted an sNDA to the FDA in February 2023 for the treatment of cholestatic pruritus associated with PFIC and a marketing authorization variation to the EMA in April 2023 for the treatment of PFIC, which may not result in approval or may result in approval contingent on additional clinical trials or implementation of a REMS. The FDA, EMA or comparable regulatory authorities also may approve a product candidate for a more limited indication or patient population than we originally requested, and the FDA, EMA or comparable regulatory authorities may not approve the labeling that we believe is necessary or desirable for the successful commercialization of a product. The FDA has set the PDUFA date for the PFIC sNDA as December 13, 2023. If approval is granted by the FDA, the approval may or may not happen on the PDUFA date. 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 Livmarli and volixibat. 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, EMA or comparable 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 new product candidates require substantial technical, financial and human resources whether or not we ultimately identify any candidates. If we are unable to develop or commercialize any other product candidates, our business and prospects will suffer.

If we fail to develop our current and any future product candidates for additional indications, our commercial opportunity will be limited.

One of our strategies is to pursue clinical development of Livmarli and volixibat in additional cholestatic disease conditions such as PFIC, BA, PSC and PBC.

Developing, obtaining regulatory approval and commercializing Livmarli and volixibat for additional indications will require substantial additional funding and is prone to the risks of failure inherent in drug development. We may not be able to successfully advance any of these indications through the development process. Even if we receive regulatory approval to market Livmarli and volixibat for the treatment of any of these additional indications, any such additional indications may not be successfully commercialized, widely accepted in the marketplace or more effective than other commercially available alternatives. If we are unable to successfully develop and commercialize Livmarli and volixibat for these additional indications, our commercial opportunity will be limited.

Risks Related to Our Business and Industry

We have incurred 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. We have only one product approved for commercial sale, and 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 net losses since our inception in May 2018. For the six months ended June 30, 2023 and 2022, we reported a net loss of \$104.2 million and \$63.5 million, respectively. As of June 30, 2023, we had an accumulated deficit of \$497.0 million.

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 Livmarli in the U.S. and in the 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. For example, we have generated limited product revenues from Livmarli and Livmarli may not be successfully commercialized. 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.

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 recent and potential future disruptions in access to bank deposits or lending commitments due to bank failures, the ongoing COVID-19 pandemic and the military conflict between Ukraine and Russia and related sanctions. General business and economic conditions that could affect our business, financial condition or results of operations include fluctuations in economic growth, 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 and our suppliers operate. For example, inflation rates, particularly in the U.S., have increased recently to levels not seen in years, and increased inflation may result in increases in our operating costs (including our labor costs), reduced liquidity and limits on our ability to access credit or otherwise raise capital on acceptable terms, if at all. In response to rising inflation, the U.S. Federal Reserve has raised, and may again raise, interest rates, which, coupled with reduced government spending and volatility in financial markets may have the effect of further increasing economic uncertainty and heightening these risks. Risks of a prolonged global economic downturn are particularly true in Europe, which is undergoing a continued severe economic crisis. A weak or declining 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.

Additionally, financial markets around the world experienced volatility due to recent bank failures and the ongoing military conflict between Ukraine and Russia. In connection with the military conflict, the U.S., United Kingdom and EU, along with others, imposed significant sanctions and export controls against Russia, Russian banks and certain Russian individuals and may implement additional sanctions or take further punitive actions in the future. The full economic and social impact of the sanctions imposed on Russia (as well as possible future punitive measures that may be implemented), as well as the counter measures imposed by Russia, in addition to the ongoing military conflict between Ukraine and Russia and related sanctions, which could conceivably expand into the

surrounding region, remains uncertain; however, both the conflict and related sanctions have resulted and could continue to result in disruptions to trade, commerce, pricing stability, credit availability, supply chain continuity and reduced access to liquidity in both Europe and globally, and has introduced significant uncertainty into global markets. In particular, the Russia-Ukraine conflict and related sanctions has contributed to rapidly rising costs of living (driven largely by higher energy prices) in Europe and other advanced economies. Further, a weak or declining economy could strain our suppliers and manufacturers, possibly resulting in additional supply disruption for the production of Livmarli. As a result, our business and results of operations may be adversely affected by the ongoing conflict between Ukraine and Russia and related sanctions, particularly to the extent it escalates to involve additional countries, further economic sanctions or wider military conflict. We have operations, as well as current and potential new suppliers and manufacturers, throughout Europe. If economic conditions in Europe and other key markets for our business remain uncertain or deteriorate further, we could experience adverse effects on our business, financial condition or results of operations.

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, is smaller than we anticipate, we may not be able to achieve profitability and growth. We focus our clinical development of Livmarli on treatments for rare pediatric cholestatic liver diseases with relatively small patient populations. Given the small number of patients who have the diseases that we are targeting with Livmarli, it is critical to our ability to grow and become profitable that we continue to successfully identify patients with these rare pediatric cholestatic liver diseases. We are focusing our clinical development of volixibat as a treatment for PSC and PBC, diseases with relatively small patient populations. In addition, our estimates of the patient populations for our target indications, including ALGS, have been derived from a variety of sources, including the 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. In the U.S., Livmarli's FDA-approved indication is for the treatment of cholestatic pruritus in patients with ALGS three months of age and older, and in the EU, Livmarli's EMA-approved indication is for the treatment of cholestatic pruritus in patients with ALGS two months of age and older. The actual number of ALGS patients three months of age and older in the U.S. or two months of age and older in the EU with active cholestatic pruritus is difficult to estimate and may turn out to be lower than expected. Further, even if we obtain significant market share for our product candidates, if approved, because the potential target populations are very small, we may never achieve profitability despite obtaining such significant market share. Lastly, the potentially addressable patient population for PFIC and ALGS, or any of our potential indications, may even be further reduced as 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 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 EMA approval. Consequently, approval by the FDA does not ensure approval by the EMA, approval by the EMA does not assure approval by the FDA, and approval of either or both of the FDA and EMA does not assure approval by regulatory authorities in other countries or jurisdictions. Further, marketing approvals in countries outside the U.S. and 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 product candidates will be harmed, which would adversely affect our business, prospects, financial condition and results of operations.

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

The ability of the FDA, EMA and other government agencies 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, which is inherently fluid and unpredictable.

Disruptions at the FDA, EMA and other government agencies may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities. If a prolonged government shutdown occurs, including as a result of the U.S. Congress failing to timely raise the U.S. debt ceiling, or if global health concerns continue to prevent the FDA, EMA or comparable regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA, EMA or comparable regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on 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., Europe, 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 medicinal products that impact, among others, orphan designation medicinal products. 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 medicinal product remained under the threshold of € 50 million in 12 months. Above this threshold, an orphan designation medicinal product would be subject to a full assessment by the G-BA regarding its benefits compared to an appropriate comparator therapy, just like any other medicinal product. 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 products. 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 medicinal products 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 executive, judicial and Congressional challenges to certain aspects of the Affordable Care Act. For example, U.S. federal tax legislation enacted in 2017, informally titled The Tax Cuts and Jobs Act ("Tax Act"), included a provision which repealed, effective January 1, 2019, the tax-based shared responsibility payment imposed by the Affordable Care Act on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." On June 17, 2021 the U.S. Supreme Court dismissed a challenge on procedural grounds that argued the Affordable Care Act is unconstitutional in its entirety because the "individual mandate" was repealed by Congress. However, it is possible that the Affordable Care Act will be subject to additional judicial or Congressional challenges in the future.

In addition, the Affordable Care Act has been subject to various health reform measures. For example, prior to the U.S. Supreme Court ruling, on January 28, 2021, the Biden administration issued an executive order that initiated a special enrollment period for purposes of obtaining health insurance coverage through the Affordable Care Act marketplace. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the Affordable Care Act. On August 16, 2022, President Biden signed the Inflation Reduction Act of 2022 ("IRA") 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 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 drugs and biologics 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 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 will take effect progressively starting in fiscal year 2023, although the Medicare drug price negotiation program is currently subject to legal challenges. It is currently unclear how the IRA will be effectuated but is likely to have a significant impact on the pharmaceutical industry.

Other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. On August 2, 2011, the Budget Control Act of 2011 was signed into law, which, among other things, included reductions to Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 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. On January 2, 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers, including hospitals, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Additionally, on March 11, 2021, President Biden signed the American Rescue Plan Act of 2021 into law, which eliminates the statutory Medicaid drug rebate cap, currently set at 100% of a drug’s average manufacturer price, for single source and innovator multiple source drugs, beginning 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.

In July 2021, the Biden administration released an executive order, “Promoting Competition in the American Economy,” with multiple provisions aimed at prescription drugs. In response to Biden’s executive order, on September 9, 2021, HHS released a Comprehensive Plan for Addressing High Drug Prices that outlines principles for drug pricing reform. The plan sets out a variety of potential legislative policies that Congress could pursue as well as potential administrative actions HHS can take to advance these principles. In addition, in response to the Biden administration’s October 2022 executive order, on February 14, 2023, HHS released a report outlining three new models for testing by the Center for Medicare and Medicaid Innovation which will be evaluated on their ability to lower the cost of drugs, promote accessibility, and improve quality of care. It is unclear whether the models will be utilized in any health reform measures in the future.

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 Livmarli 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 Livmarli and our other product candidates, if approved.

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;
- unexpected changes in tariffs, 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 the Russia-Ukraine conflict and rising interest rates;
- 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 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 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. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. For instance, we are aware that Albireo was purchased by Ipsen in March 2023. 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.

Outside of surgery and Livmarli, there are no other approved therapies for use in patients three months of age and older with ALGS in the U.S. Ursodeoxycholic acid (“UDCA”), cholestyramine and other bile salt resins, rifampin, naltrexone and other agents, such as selective serotonin reuptake inhibitors are used off label to treat ALGS patients suffering from cholestatic pruritus. Despite the lack of FDA approval for such indication, these older, generic agents are perceived as the standard of care for treating ALGS patients suffering from cholestatic pruritus.

We are aware of two other companies pursuing clinical development of therapies that reduce sBA levels via the IBAT pathway. GlaxoSmithKline plc and Ipsen have IBATis in clinical development for cholestatic liver diseases. We are aware that Ipsen has received approval for odeixibat for the treatment of pruritus in patients with PFIC, and pruritus in patients with ALGS 12 months and older, in the U.S. and for the treatment of PFIC in Europe. Ipsen has been granted orphan designation for PFIC in Europe and if

Livmarli is deemed similar, it could prevent the approval of Livmarli in PFIC or result in the loss of orphan designation for Livmarli for PFIC in Europe. Ipsen is also conducting a study of odeixibat in BA and plans to pursue other cholestatic liver diseases. We are aware that Ipsen has submitted a variation application to the EMA seeking approval for odeixibat for use in patients with ALGS. We are aware that GlaxoSmithKline plc has completed a Phase 2 trial of its IBATi in PBC patients and is conducting a Phase 3 trial in PBC. We are also aware that Intercept Pharmaceuticals, Inc. is exploring BA as an indication for obeticholic acid. Further, we may compete with companies that are developing gene therapy for the treatment of PFIC. In adult settings of cholestasis, similar to pediatric settings, cholestyramine, UDCA, rifampin and naltrexone are commonly used agents. We are not aware of FDA approved therapeutics for the treatment of PSC. We are aware of several agents in clinical development for the treatment of PSC, including Cymabay's seladelpar, DURECT Corporation's DUR928, HighTide Biopharmaceutical Inc.'s HTD1801, Immunic Therapeutics' IMU-838, Intercept's Ocaliva, or obeticholic acid, Ipsen's elafibranor and IPN60250, NGM Biopharmaceuticals Inc.'s NGM282, Escient Pharmaceuticals's EP547, Chemomab Therapeutics Ltd.'s CM-101, and Pliant Therapeutics' PLN-74809. Intercept Pharmaceuticals, Inc.'s Ocaliva is approved as a second-line treatment for PBC. We are aware of several agents in clinical development for the treatment of PBC including Cymabay's seladelpar, Ipsen's elafibranor, Zydus Therapeutics Inc.'s saroglitazar magnesium, Escient Pharmaceuticals Inc.'s EP547, NGM Biopharmaceuticals, Inc.'s NGM282, Calliditas Therapeutics AB's setanaxib, COUR Pharmaceuticals' CNP-104 and Umeocrine Cognition's golexanolone.

Even though we have obtained orphan drug designation for Livmarli in PFIC, ALGS and BA, 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, 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, ALGS and BA in the U.S. We also received orphan drug designation for Livmarli for PFIC, ALGS and BA in the EU. 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 EMA from approving another marketing application for the same drug (or, in the case of the EMA, 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 agency's agreed upon pediatric investigation plan. 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 EMA 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 EMA 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. Specifically, Ipsen has been granted orphan designation for PFIC in the EU and if Livmarli is deemed similar, it could prevent the approval of, or result in the loss of orphan designation for, Livmarli for PFIC in the EU. 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, ALGS and BA, if we receive approval for Livmarli for a modified or different indication, our current orphan designations may not provide us with exclusivity.

Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process in the U.S. 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.

Although we have received a breakthrough therapy designation for Livmarli, this may not lead to a faster development, regulatory review or approval process, and it does not increase the likelihood that Livmarli will receive marketing approval in the U.S. for other indications.

We have received a breakthrough therapy designation for Livmarli for the treatment of PFIC2. A breakthrough therapy is defined as a therapy that is intended, alone or in combination with one or more other therapies, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the therapy may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For therapies that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Therapies designated as breakthrough therapies by the FDA may also be eligible for priority review and accelerated approval. The breakthrough therapy designation for Livmarli may not result in a faster development process, review or approval compared to therapies considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, the FDA may later decide that Livmarli no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

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 Livmarli, our product candidates and any future product candidates that we may develop. We also intend to establish commercial partnerships outside of North America and 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 products 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 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 liver disease. 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 products. 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 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 EMA 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 products that we acquire may not have the market potential we believe, be manufactured or sold profitably, or achieve market acceptance.

We may not complete the Asset Purchase within the time frame we anticipated or at all.

The completion of the Asset Purchase is subject to a number of conditions, including (i) obtaining certain material consents, (ii) the expiration or termination of the applicable waiting period (and any extension thereof) under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, (iii) the absence of any law, order or legal proceeding prohibiting the Asset Purchase, (iv) the accuracy of representations and warranties set forth in the Purchase Agreement (subject to customary materiality qualifiers) and material compliance with covenants set forth in the Purchase Agreement and (v) the execution and delivery of certain related ancillary documents. The failure to satisfy all of the required conditions could delay the completion of the Asset Purchase for a significant period of time or prevent it from occurring at all. A delay in completing the Asset Purchase could cause us to realize some or all of the benefits later than we otherwise would expect to realize them if the Asset Purchase were successfully completed within the anticipated timeframe, and could result in additional transaction costs or in other negative effects associated with uncertainty about the completion of the Asset Purchase.

We may fail to realize all of the anticipated benefits of the Asset Purchase or those benefits may take longer to realize than expected.

We believe that there are significant benefits that may be realized from the product candidates acquired from Travele. The full benefits of the transactions contemplated by the Purchase Agreement, 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. For example, the Phase 3 RESTORE study may not reach its primary endpoint or may not support an approval. Failure to achieve the anticipated benefits of the Asset Purchase could adversely affect our results of operations or cash flows, divert needed resources away from our current products, decrease or delay any accretive effect of the Asset Purchase and negatively impact the price of our common stock.

In addition, we will be required to devote significant attention and resources prior to closing to prepare for the post-closing integration of the acquired assets. This process may disrupt the businesses and, if ineffective, would limit the anticipated benefits of the acquisition. In addition, even if the Asset Purchase is completed, we may not be able to integrate the acquired assets successfully. If we fail to successfully integrate the acquired assets, our results of operations could be adversely affected. Any integration process will require significant time and resources, require significant attention from management and 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. This competition has become exacerbated by the increase in employee resignations taking place throughout the U.S. in 2022, which is commonly referred to as the “great resignation.” We may also experience employee turnover as a result of the ongoing “great resignation.”

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, rising 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 June 30, 2023, we had 213 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 Livmarli and volixibat, 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 Livmarli, 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 also 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. However, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for our product candidates or otherwise advance our business. 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. 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 Livmarli, 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 manmade 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 Livmarli and volixibat. Our ability to obtain clinical supplies of Livmarli and volixibat 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, EMA or comparable regulatory authorities, including those laws that require the reporting of true, complete and accurate information to the FDA, EMA or comparable 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. and in certain countries in Europe, 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 Medicare, Medicaid and other federal healthcare 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 and state healthcare fraud and abuse laws, false claims laws, transparency laws, health information privacy and security 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 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; 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 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 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 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 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, 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 and evolving U.S. and foreign laws, regulations, rules, contractual obligations, 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), 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 Telephone Consumer Protection Act (“TCPA”) imposes specific requirements 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. Class action suits are the most common method for private enforcement.

At the state level, the California Consumer Privacy Act (“CCPA”), which took effect on January 1, 2020, imposes obligations on covered businesses. These obligations include, but are not limited to, providing specific disclosures in privacy notices and affording California residents, including consumers, business representatives and employees, certain rights related to their personal data. The CCPA provides for administrative fines for violations (up to \$7,500 per violation) and a private right of action for certain data breaches. Although the CCPA exempts some data processed in the context of clinical trials, the CCPA may increase compliance costs and potential liability with respect to other personal data we maintain about California residents. The CCPA has expanded substantially as a result of the California Privacy Rights Act of 2020 (“CPRA”), which took effect January 1, 2023. The CPRA established a new California Privacy Protection Agency to implement and enforce the CPRA, which could increase the risk of enforcement. Other states have also enacted data privacy laws, several of which take effect in 2023 including Virginia, Utah,

Colorado, and Connecticut. While these states, like the CCPA, also exempt some data in the context of clinical trials, these developments further complicate compliance efforts and increase legal risks and compliance costs for us and our third party partners. In addition, data privacy and security laws have been proposed at the federal, state, and local levels in recent years, which could further complicate compliance efforts. Privacy advocates and industry groups have also proposed, and may propose, standards with which we are legally or contractually bound to comply.

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, 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 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 regimes because of our data processing activities that involve the personal data of individuals residing in the European Economic Area ("EEA") and/or UK, such as in connection with our clinical trials in Europe, and early access program in multiple EU countries, and because of any potential 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. 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 offences or convictions. Under the EU 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.

In the ordinary course of business, we may 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 similarly stringent interpretations of their 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 and UK's standard data protection clauses, the UK's International Data Transfer Agreement / Addendum, and the EU-U.S. Data Privacy Framework (which allows for transfers for 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 United States was insufficient and levied a 1.2 billion Euro fine against the company and prohibited the company from transferring personal data to the United States. 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.

In addition, we may also be 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 may also publish privacy policies, marketing materials and other statements, such as compliance with certain certifications or self-regulatory principles, regarding data privacy and security. If these policies, materials or statements are found to be deficient, lacking in transparency, deceptive, unfair or misrepresentative of our practices, we may be subject to adverse consequences.

Obligations related to data privacy and security are quickly changing, becoming increasingly stringent, and creating regulatory 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 all applicable 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 upon whom we rely may fail to comply with such obligations, which could negatively impact our business operations and compliance posture.

Our, or the third parties' upon which we rely, 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 information, 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 information, 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 products; 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 Livmarli 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 Livmarli 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 Livmarli 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. 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 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 for Livmarli and volixibat. 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, EMA or comparable 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 or comparable 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 or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

Our CROs are not our employees and, except for remedies available to us under our agreements with such CROs, we cannot 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, 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 product 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 our clinical and commercial drug supplies, or to supply, manufacture or distribute Livmarli. 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, active pharmaceutical ingredients (“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 Livmarli, 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 Livmarli. As Livmarli and our product candidates are intended to treat rare liver diseases, we will only require a low-volume of raw materials and APIs, and in the case of Livmarli and volixibat, 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 Livmarli and any product candidates that are approved.

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

- fail to supply us with our product 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, EMA or comparable regulatory authorities. Any need to find and qualify new suppliers or manufacturers could adversely impact our ability to commercialize Livmarli 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 current good manufacturing practices (“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. A number of our contract suppliers and manufacturers must comply with cGMP requirements enforced by the FDA through its 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 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 Livmarli or our product candidates will be negatively affected.

In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the safety of Livmarli or our product candidates 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 Livmarli and volixibat.

We and our third-party suppliers and manufacturers are vulnerable to geopolitical and macroeconomic developments, such as recent and potential future bank failures, the ongoing conflict between Ukraine and Russia, related sanctions, future pandemics,

increasing 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 certain 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 or defective packaging. If such events result in any interruption in the supply of a drug substance or other material or in the manufacture of Livmarli, such interruption could have a material adverse effect on our business, financial condition, operating results and prospects.

We rely on a single specialty pharmacy and a single distributor for all of our sales of Livmarli in the U.S. and use a single distributor in certain countries in Europe. 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, a decline in our revenue, results of operations and cash flows. Further, we are in the process of switching or adding contract manufacturing organizations for both Livmarli and volixibat, which is cost-intensive and time-consuming. The success of these transfers is necessary for continuous supply to clinical trials and potential future commercial demand.

Risks Related to Our Financial Position and Capital Requirements

We will need substantial additional financing to continue our commercialization efforts for Livmarli, develop our product candidates and implement our operating plans. If we fail to obtain additional financing, 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 Livmarli, 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 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 will 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 recent and potential future disruptions in access to bank deposits or lending commitments due to bank failures, the ongoing conflict between Ukraine and Russia and related sanctions, the COVID-19 pandemic, actions taken to slow its spread, 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 Livmarli or volixibat 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 indebtedness under the Notes. Further, in July 2023, in connection with and subject to the consummation of, the Asset Purchase, we entered into the Subscription Agreements, pursuant to which we expect to issue approximately 8 million shares of our common stock. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a stockholder. For example, if we elect to settle our conversion obligation under the Notes in shares of our common stock or a combination of cash and shares of our common stock, the issuance of such common stock may 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 Livmarli, 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 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 that 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, 2022, we had federal and California and other state net operating loss (“NOL”) carryforwards of approximately \$153.1 million, \$19.1 million and 17.7 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, 2022, we also had federal and state research and development credit carryforwards totaling \$32.5 million and \$4.2 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 Tax Act, as modified by the Coronavirus Aid, Relief, and Economic Security Act (“CARES Act”), federal NOLs generated in taxable years beginning after December 31, 2017 may be carried forward indefinitely, but the deductibility of such federal NOLs in taxable years beginning after December 31, 2020 is limited to 80% of taxable income. It is uncertain if and to what extent various states will conform to the Tax Act or the CARES Act. 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. In addition, at the state level, there may be periods during which the use of net operating losses is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. 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.

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 June 30, 2023, we had \$316.3 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, if triggered, 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 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 and shares of our common stock (subject to our right or obligation to pay cash in lieu of all or a portion of such shares). If shares of our common stock are issued to the holders of the Notes upon conversion, 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.

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

The accounting method for reflecting the Notes on our balance sheet, accruing interest expense for the Notes and reflecting the underlying shares of our common stock in our reported diluted earnings per share may adversely affect our reported earnings and financial condition.

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, we expect that the Notes we are offering will be 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, and accounting standards may change in the future in a manner that may adversely affect our 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.

Furthermore, if any of the conditions to the convertibility of the Notes is satisfied, then we may be required under applicable accounting standards to reclassify the liability carrying value of the Notes as a current, rather than a long-term, liability. This reclassification could be required even if no noteholders convert their Notes and could materially reduce our reported working capital.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain sufficient intellectual property protection for Livmarli 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 Livmarli and other our 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. 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 Livmarli or our 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 may 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 Livmarli 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 Livmarli 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 Livmarli, 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 Livmarli 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 Livmarli and any then-approved product;
- we cannot ensure that we will be able to successfully commercialize Livmarli 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 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 Livmarli 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 Livmarli or our product candidates in the U.S. or in foreign jurisdictions. Even if such patents do successfully issue, third parties may 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 Livmarli or our product candidates. Furthermore, even if they are unchallenged, our patents may not adequately

protect our intellectual property, provide exclusivity for Livmarli 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 Livmarli or our product candidates is threatened, it could dissuade companies from collaborating with us to develop, or threaten our ability to commercialize, Livmarli 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 Livmarli 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 Livmarli 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 Livmarli 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 IBATs, including maralixibat (the active pharmaceutical ingredient 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 active pharmaceutical ingredients 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, the U.S. and foreign government actions related to Russia's conflict in Ukraine may limit or prevent filing, prosecution, and 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 planned Unified Patent Court may in particular present uncertainties for our ability to protect and enforce our patent rights against competitors in Europe. In 2012, the European Patent Package ("EU Patent Package") regulations were passed with the goal of providing a single pan-European Unitary Patent and a new European Unified Patent Court ("UPC"), for litigation involving European patents. Implementation of the EU Patent Package will likely occur in the first half of 2023. 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, 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 Patent Package as currently proposed, 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 maralixibat and volixibat 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 Livmarli 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 Livmarli 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 including Shire, Pfizer, our wholly-owned subsidiary Satiogen, and Sanofi, related to Livmarli and our product candidates. For example, we have our 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 Livmarli 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 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 Livmarli 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 Livmarli 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 Livmarli 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 Livmarli 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 Livmarli or one of our current or future product candidates. Nevertheless, we are not aware of any issued patents that will prevent us from marketing Livmarli 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 Livmarli 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 Livmarli, 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 Livmarli 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 Livmarli 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 Livmarli 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 Livmarli 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, Livmarli, 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 Livmarli and any then-approved product. Further, we may incorrectly determine that our technologies, Livmarli 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 Livmarli 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 Livmarli, our product candidates and future approved products 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 Livmarli 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 Livmarli 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 Livmarli 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 Livmarli 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 Livmarli 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 Livmarli 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 occurs 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, 2023 to August 2, 2023 has ranged from a low of \$19.19 to a high of \$30.09. In addition to the factors discussed in this “Risk Factors” section, these factors include, among others:

- the degree of physician and patient adoption of Livmarli and use of Livmarli necessary for commercial success;
- our failure to grow and maintain our own sales force to market Livmarli;
- our ability to market and sell Livmarli where approved;

- our ability to commercialize Livmarli in certain countries in Europe, and our ability to grow and maintain an international sales force;
- any delay in our regulatory filings for Livmarli 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 Livmarli 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 Livmarli 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 products;
- management transitions and additions or departures of key scientific or management personnel;
- unanticipated serious safety concerns related to the use of Livmarli 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 ALGS, PFIC and other cholestatic liver diseases that we may target;
- 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 Ukraine-Russia conflict, the COVID-19 pandemic, economic slowdowns, recessions, inflation, bank failures, rising interest rates and tightening of credit markets; and
- other events or factors, many of which are beyond our control.

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, including pursuant to the Shelf Registration, Sales Agreement, Subscription Agreements and our equity incentive plans, 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. 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 the Shelf Registration. For example, in August 2020, we entered into the Sales Agreement with SVB Securities, 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 \$75.0 million under the Shelf Registration through SVB Securities acting as the sales agent and/or principal. The remaining capacity under the Sales Agreement is approximately \$31.3 million as of June 30, 2023. Further, in July 2023, in connection with and subject to the consummation of, the Asset Purchase, we entered into the Subscription Agreements, pursuant to which we expect to issue approximately 8 million shares of our common stock. 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, in March 2020, we

adopted a 2020 Inducement Plan, pursuant to which 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. 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 of 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 a delisting, we can provide no assurance that any action taken by us to restore compliance with listing requirements would 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, the president 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 to amend any bylaws by stockholder action or to amend specific provisions of our 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 Delaware General Corporate Law, 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 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 Delaware General Corporation Law, 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 Delaware General Corporation Law 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 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 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. 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

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

In the course of our business, we and the third parties upon which we rely, may 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 on which we depend to operate our business, 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 cyber-attacks, 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 upon which we rely may be vulnerable to a heightened risk of these attacks, including cyber-attacks 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 upon which we rely 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 (such as credential stuffing), social engineering (including through deep fakes, which may be increasingly more difficult to identify as fake, and phishing), ransomware, supply-chain attacks, server malfunctions, software or hardware failure, loss of data or other information technology assets, adware, telecommunications failures and other similar threats. 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 remains common and has increased risks to our information technology systems and data, as more of our employees utilize network connections, computers and devices outside our premises or network, including working at home, while in transit and in public locations.

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 rely upon third-party service providers and technologies to 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 rely on third-party research institution collaborators, CROs, other contractors and consultants for many aspects of our business, including research and development activities and manufacturing of Livmarli 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 our third-party service providers experience a security incident or other interruption, we could experience adverse consequences. While we may be entitled to damages if our third-party service providers fail to satisfy their privacy or security-related obligations to us, any award may be insufficient to cover our damages, or we may be unable to recover such award. Similarly, supply-chain attacks have increased in frequency and severity, and we cannot guarantee that third parties and infrastructure in our supply chain or our third-party partners' supply chains have not been compromised or that they do not contain exploitable defects or bugs that could result in a breach of or disruption to our information technology systems or the third-party information technology systems that support us and our services.

Any of the previously identified or similar threats could 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 upon whom we rely. A security incident or other interruption could disrupt our ability (and that of third parties upon whom we rely) to provide our products 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 may require us to implement and maintain specific security measures, industry-standard or reasonable security measures to protect our information technology systems and sensitive information. While we have developed systems and processes designed to protect the integrity, confidentiality and security of the sensitive information under our control, we cannot assure you that our security measures or those of the third parties we depend on will be effective in preventing security incidents. We may be unable to anticipate attempted security breaches, identify them before our sensitive information is exploited, or react in a timely manner. For example, we take steps to detect and remediate vulnerabilities, but we may not be able to detect and remediate all vulnerabilities because the threats and techniques used to exploit the vulnerability change frequently and are often sophisticated in nature. Therefore, such vulnerabilities could be exploited but may not be detected until after a security incident has occurred. These vulnerabilities pose material risks to our business. Further, we may experience delays in developing and deploying remedial measures designed to address any such identified vulnerabilities.

Applicable data privacy and security obligations may require us to notify relevant stakeholders of security incidents. Such disclosures are costly, and the disclosure or the failure to comply with such requirements could lead to adverse consequences. Although, to our knowledge, we have not experienced a material system failure or security incident to date, if such an event were to occur, or we (or a third party upon whom we rely) are perceived to have experienced a security incident, 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.

Our insurance coverage may not be adequate for cybersecurity liabilities, may not continue to be available to us on economically reasonable terms, or at all, and any insurer may deny coverage as to any future claim. 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 Livmarli 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 are an emerging growth company and a smaller reporting company, and the reduced reporting requirements applicable to emerging growth companies and smaller reporting companies may make our common stock less attractive to investors.

We are an emerging growth company, as defined in the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and exemptions from the requirements of holding nonbinding advisory votes on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company until December 31, 2024, although circumstances could cause us to lose that status earlier, including if we become a "large accelerated filer" as defined in Rule 12b-2 under the Exchange Act or if we have total annual gross revenue of \$1.235 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31 or, if we issue more than \$1.0 billion in non-convertible debt during any three year period before that time, we would cease to be an emerging growth company immediately. Even after we no longer qualify as an emerging growth company, we may still qualify as a "smaller reporting company" which would allow us to take advantage of many of the same exemptions from disclosure requirements including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. Investors may find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies. As a result, changes in rules of accounting principles generally accepted in the U.S. or their interpretation, the adoption of new guidance or the application of existing guidance to changes in our business could significantly affect our financial position and results of operations.

We are also a "smaller reporting company" as defined in the Exchange Act. We may continue to be a smaller reporting company even after we are no longer an emerging growth company. We may take advantage of certain of the scaled disclosures available to smaller reporting companies and will be able to take advantage of these scaled disclosures for so long as our voting and non-voting common stock held by non-affiliates is less than \$250.0 million measured on the last business day of our second fiscal quarter, or our annual revenue is less than \$100.0 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is less than \$700.0 million measured on the last business day of our second fiscal quarter.

As of June 30, 2023, the market value of our common stock held by non-affiliates exceeded \$700.0 million. As a result, we will be a large accelerated filer and thus will cease to be an emerging growth company effective December 31, 2023. Additionally, we will no longer qualify as a smaller reporting company beginning with our first Quarterly Report on Form 10-Q for the quarterly period ending March 31, 2024.

If we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired.

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 will require that we incur substantial additional professional fees and internal costs to expand our accounting and finance functions and that we expend significant management efforts. Prior to our initial public offering, we have never been required to test our internal controls within a specified period, and, as a result, we may experience difficulty in meeting these reporting requirements in a timely manner.

We may discover weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our consolidated financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. 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 regulatory authorities.

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

As a public company, we have incurred and will continue to incur significant legal, accounting and other expenses that we did not incur as a private company. 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, or 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. Recent legislation permits emerging growth companies to implement many of these requirements over a longer period. We have taken advantage of this new legislation, but we may be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. As of June 30, 2023, the market value of our common stock held by non-affiliates exceeded \$700.0 million. Consequently, we will be a large accelerated filer and will therefore cease to be an emerging growth company effective December 31, 2023. As a result of this transition, we will be 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 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 regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

We expect the rules and regulations applicable to 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 Livmarli and any then-approved product or services. For example, we expect these rules and regulations 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 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.

None.

Item 6. Exhibits.

Exhibit Number	Description
2.1+	<u>Asset Purchase Agreement, dated July 16, 2023, by and between Mirum Pharmaceuticals, Inc. and Travele 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).</u>
3.1	<u>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).</u>
3.2	<u>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).</u>
4.1	<u>Form of Common Stock Certificate (incorporated by reference to Exhibit 4.1 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-232251), filed with the SEC on July 8, 2019).</u>
4.2	<u>Investors' Rights Agreement, dated November 5, 2018 (incorporated by reference to Exhibit 4.2 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-232251), filed with the SEC on June 21, 2019).</u>
4.3	<u>Indenture, dated as of April 17, 2023, between Mirum Pharmaceuticals, Inc. and U.S. Bank Trust Company, National Association, as trustee (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on April 17, 2023).</u>
4.4	<u>Form of certificate representing the 4.00% Convertible Senior Notes due 2029 (included as Exhibit A to Exhibit 4.3 above) (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on April 17, 2023).</u>
10.1*	<u>Form of Subscription Agreement</u>
10.2	<u>Form of Registration Rights Agreement (incorporated by reference to Exhibit 10.2 to the Registrant's Current Report on Form 8-K, filed with the SEC on July 17, 2023).</u>
31.1*	<u>Certification of Principal Executive and Financial Officer pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</u>
32.1*#	<u>Certification of Principal Executive and Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</u>
101.INS*	Inline XBRL Instance Document
101.SCH*	Inline XBRL Taxonomy Extension Schema Document
101.CAL*	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB*	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE*	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104*	Cover Page Interactive Data File (formatted as inline XBRL and contained in Exhibit 101)

* Filed herewith.

+ Certain confidential information contained in this Exhibit, marked in brackets, has been omitted, because it is both not material and of the type of information that the registrant treats as private or confidential.

The information in Exhibit 32.1 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: August 7, 2023

By: /s/ Christopher Peetz

Christopher Peetz
President and Chief Executive Officer
(Principal Executive and Financial Officer)

SUBSCRIPTION AGREEMENT

This Subscription Agreement (this “**Agreement**”) is made and entered into as of July 16, 2023 (the “**Effective Date**”) by and among Mirum Pharmaceuticals, Inc., a Delaware corporation (the “**Company**”), and each of the purchasers listed on the signature pages hereto, severally and not jointly (each a “**Purchaser**” and together the “**Purchasers**”). Certain terms used and not otherwise defined in the text of this Agreement are defined in Section 8 hereof.

RECITALS

WHEREAS, the Company is party to that certain Asset Purchase Agreement, by and between the Company and Traverre Therapeutics, Inc., a Delaware corporation (“**Traverre**”), dated on or about the date hereof (the “**Purchase Agreement**”), pursuant to which, among other things, the Company will purchase certain assets (among them assets relating to Cholbam and Chenodal) and assume certain liabilities from Traverre (the “**Transaction**”);

WHEREAS, the Company desires to sell to the Purchasers, and the Purchasers, severally and not jointly, desire to purchase from the Company, an aggregate amount equal to \$210,000,000 of shares of the Company’s Common Stock, par value \$0.0001 per share (the “**Common Stock**”), at a per share purchase price equal to the Purchase Price, in accordance with the terms and provisions of this Agreement, immediately prior to, but subject to, the closing of the Transaction; and

WHEREAS, the Company and each Purchaser is executing and delivering this Agreement in reliance upon the exemption from securities registration afforded by Section 4(a)(2) of the 1933 Act;

NOW, THEREFORE, in consideration of the foregoing and the mutual representations, warranties and covenants herein contained, the parties hereto hereby agree as follows:

SECTION 1. Authorization of Shares.

1.01 The Company has authorized the sale and issuance of shares of Common Stock on the terms and subject to the conditions set forth in this Agreement. The shares of Common Stock sold hereunder at the Closing (as defined below) shall be referred to as the “**Shares**.”

SECTION 2. Sale and Purchase of the Shares.

2.01 Upon the terms and subject to the conditions herein contained, the Company agrees to sell and issue to each Purchaser, and each Purchaser agrees, severally and not jointly, to purchase from the Company, at a closing to take place remotely via exchange of executed documents (the “**Closing**” and the date of the Closing, the “**Closing Date**”) to occur immediately prior to, but subject to, the closing of the Transaction, that number of Shares (the “**Closing Shares**”) set forth opposite such Purchaser’s name on the Schedule of Purchasers for the aggregate Purchase Price set forth under the heading “Subscription Amount.”

2.02 At or prior to the Closing, each Purchaser will pay the subscription amount set forth opposite such Purchaser’s name on the Schedule of Purchasers (the “**Subscription Amount**”) by wire transfer of immediately available funds in accordance with wire instructions provided by the Company to the Purchasers at least two Business Days prior to the Closing (the “**Wire Instructions Notice**”). If so requested by the Company in the Wire Instructions Notice and agreed by the applicable Purchaser, the Subscription Amount of each Purchaser shall be paid into an escrow fund or trust account designated by the Company in writing (the “**Escrow Account**”) to be released to the Company only upon satisfaction of each of the closing conditions set forth in Section 6 below. In the event the Closing does not occur within three Business Days of the Closing Date specified in the Wire Instructions Notice, unless otherwise agreed by the Company and such Purchaser, the Company shall, or shall cause the escrow agent for the Escrow Account to, promptly (but not later than two Business Days thereafter) return the aggregate Purchase Price to each Purchaser by wire transfer of U.S. dollars in immediately available funds to the account specified by such Purchaser. On the Closing Date, the Company will deliver, against payment by each

Purchaser of its Subscription Amount, the Closing Shares in book-entry form, and shall provide evidence of such issuance from the Company's transfer agent as of the Closing Date to each Purchaser. Notwithstanding anything to the contrary in this Agreement, the Schedule of Purchasers may be amended by the Company and the affected Purchaser up to three (3) Business Days prior to the Closing, without the consent of the other parties hereto, to reflect the actual number of Shares purchased by each Purchaser at the Closing, provided that the Company shall provide to Purchasers such updated Schedule of Purchasers.

SECTION 3 Representations and Warranties of the Purchasers

. Each Purchaser, severally and not jointly, represents and warrants to the Company as of the date of this Agreement and as of the Closing Date that:

3.01 Validity. The execution, delivery and performance of this Agreement and the consummation by the Purchaser of the transactions contemplated hereby have been duly authorized by all necessary corporate, partnership, limited liability or similar actions, as applicable, on the part of such Purchaser. This Agreement has been duly executed and delivered by the Purchaser and, assuming that this Agreement constitutes the valid and binding obligation of the Company, constitutes a valid and binding obligation of the Purchaser, enforceable against it in accordance with its terms, except as limited by applicable bankruptcy, insolvency, reorganization, moratorium, fraudulent conveyance, and any other laws of general application affecting enforcement of creditors' rights generally, and as limited by laws relating to the availability of specific performance, injunctive relief, or other equitable remedies.

3.02 Brokers. There is no broker, investment banker, financial advisor, finder or other person which has been retained by the Purchaser who is entitled to any fee or commission for which the Company will be liable in connection with the execution of this Agreement and the consummation of the transactions contemplated hereby.

3.03 Investment Representations and Warranties. The Purchaser understands and agrees that the offering and sale of the Shares has not been registered under the 1933 Act or any applicable state securities laws and is being made in reliance upon federal and state exemptions for transactions not involving a public offering which depend upon, among other things, the bona fide nature of the investment intent and the accuracy of the Purchaser's representations as expressed herein.

3.04 Acquisition for Own Account. The Purchaser is acquiring the Shares for its own account for investment and not with a view towards distribution in a manner which would violate the 1933 Act or any applicable state or other securities laws. The Purchaser has not been formed for the specific purpose of acquiring the Shares.

3.05 No General Solicitation. The Purchaser became aware of this offering of the Shares solely by means of direct contact between the Purchaser, on the one hand, and (x) the Company; (y) Morgan Stanley & Co. LLC and/or Evercore Group L.L.C. (the "**Placement Agents**") and/or (z) their respective representatives, on the other hand. The Shares were offered to the Purchaser solely by direct contact between the Purchaser and the Company, the Placement Agents and/or their respective representatives. The Purchaser acknowledges that it is not relying upon, and has not relied upon, any statement, representation or warranty made by any person or entity (including the Company, the Placement Agents or their respective representatives), other than the representations and warranties by the Company contained in this Agreement, in making its investment or decision to invest in the Company. The Purchaser did not become aware of this offering of the Shares, nor were the Shares offered to the Purchaser, by any other means, and none of the Company, the Placement Agents or their respective representatives acted as an investment adviser, broker or dealer to the Purchaser. The Purchaser acknowledges that the Shares (i) were not offered by any form of general solicitation or general advertising and (ii) are not being offered in a manner involving a public offering under, or in a distribution in violation of, the Securities Act, or any state securities laws. The Purchaser has a substantive pre-existing relationship with the Company, one of the Placement Agents or their respective Affiliates.

3.06 Ability to Protect Its Own Interests and Bear Economic Risks. The Purchaser is a sophisticated institutional investor, has the capacity to protect its own interests in connection with the transactions

contemplated by this Agreement, and has sufficient knowledge and experience in investing in investments similar to the Shares to properly evaluate the merits and risks of the investment in the Shares. The Purchaser is able to bear the substantial risks of an investment in the Shares including but not limited to loss of the Purchaser's entire investment therein.

3.07 Accredited Investor; Qualified Institutional Buyer. The Purchaser is (i) a qualified institutional buyer (as defined in Rule 144A of the 1933 Act) or (ii) an "accredited investor" within the meaning of Rule 501(a) (1), (2), (3) or (7) under the 1933 Act.

3.08 Restricted Shares. The Purchaser understands that the Shares will be characterized as "restricted securities" under the federal securities laws inasmuch as they are being acquired from the Company in a private placement under Section 4(a)(2) of the 1933 Act and that, under such laws and applicable regulations, such Shares may be resold without registration under the 1933 Act only in certain limited circumstances.

3.09 Review and Advisors. In making its decision to subscribe for and purchase the Shares, the Purchaser has relied solely upon its own independent investigation. Without limiting the generality of the foregoing, the Purchaser has not relied on any statements or other information provided by the Company or the Placement Agents or any of their respective affiliates, or any of their respective control persons, officers, directors, employees, partners, agents or representatives, concerning the Company or the Shares or the offer and sale of the Shares or the Purchaser's decision to purchase the Shares. The Purchaser acknowledges and agrees that such Purchaser has received such information as the Purchaser deems necessary in order to make an investment decision with respect to the Shares, including with respect to the Company and the Transaction. The Purchaser and the Purchaser's professional advisor(s), if any, have had the full opportunity to ask such questions, receive such answers and obtain such financial and other information as the Purchaser and the Purchaser's professional advisor(s), if any, have deemed necessary to make an investment decision with respect to the Shares. The foregoing, however, does not limit or modify the representations and warranties of the Company in Section 4 of this Agreement or the right of the Purchasers to rely thereon or the right of the Purchasers to rely on the truth, accuracy and completeness of the SEC Reports.

3.10 Residency. Such Purchaser's residence (if an individual) or offices in which its investment decision with respect to the Shares was made (if an entity) are located at the address immediately below such Purchaser's name on the Schedule of Purchasers, or as otherwise noted on the Schedule of Purchasers.

3.11 Disclosure of Information. The Purchaser has had an opportunity to discuss the Company's business, management, financial affairs and the terms and conditions of the offering of the Shares and the terms of the Transaction with the Company's management. The foregoing, however, does not limit or modify the representations and warranties of the Company in Section 4 of this Agreement or the right of the Purchasers to rely thereon or the right of the Purchasers to rely on the truth, accuracy and completeness of the SEC Reports.

SECTION 4. Representations and Warranties by the Company.

. The Company represents and warrants to the Purchasers as of the date of this Agreement and as of the Closing Date that:

4.01 Absence of Changes. The Company has conducted its business only in the ordinary course of business (except for the execution and performance of this Agreement and the Purchase Agreement, and the discussions, negotiations, and transactions related thereto) and (i) there has not been any change, condition, event, circumstance, occurrence, result, state of facts or development that has or would reasonably be expected to have a materially adverse effect on the business, financial condition, assets, operations, results of operations, stockholders' equity or financial performance of the Company and its subsidiaries, taken as a whole (a "**Material Adverse Effect**"), (ii) there have been no transactions entered into by the Company or any of its subsidiaries, other than those in the ordinary course of business and except as contemplated in this Agreement and the Purchase Agreement, which are material with respect to the Company and its subsidiaries considered as one enterprise, (iii) there has been no dividend or distribution of any kind declared, paid or made by the Company on any class of its capital stock, (iv) neither the Company nor any of its subsidiaries has made any material change in or material amendment to,

modification of or waiver of any material right under, or termination of any material contract and (v) there has not been any other event or condition that has had or would reasonably be expected to have a Material Adverse Effect.

4.02 Organization and Good Standing. The Company is a corporation duly incorporated and is validly existing and in good standing under the laws of the State of Delaware, and has all necessary power and authority (i) to conduct its business in all material respects in the manner in which its business is currently being conducted, (ii) to own or lease and use its property and assets in the manner in which its property and assets are currently owned or leased and used in all material respects and (iii) to perform its obligations under all contracts by which it is bound in all material respects. The Company is duly qualified as a foreign corporation to transact business and is in good standing in each other jurisdiction in which such qualification is required, whether by ownership or leasing of property or the conduct of business, except where the failure so to qualify or to be in good standing would not result in a Material Adverse Effect. Each of the Company's subsidiaries has been duly incorporated or organized and is validly existing as a corporation or other organization in good standing under the laws of the jurisdiction in which it is incorporated or organized has all necessary power and authority (i) to conduct its business in all material respects in the manner in which its business is currently being conducted, (ii) to own or lease and use its property and assets in the manner in which its property and assets are currently owned or leased and used in all material respects and (iii) to perform its obligations under all contracts by which it is bound in all material respects. Each of the Company's subsidiaries is duly qualified as a foreign corporation to transact business and is in good standing in each other jurisdiction in which such qualification is required, whether by ownership or leasing of property or the conduct of business, except where the failure so to qualify or to be in good standing would not result in a Material Adverse Effect.

4.03 Validity; Valid Issuance of Shares. The Company has all requisite corporate power and authority to enter into this Agreement and the Registration Rights Agreement and to consummate the transactions contemplated by this Agreement, subject only to the terms of the Purchase Agreement. The execution and delivery of this Agreement and the consummation of the transactions contemplated by this Agreement by the Company have been duly authorized by all necessary corporate action on the part of the Company. Assuming the due authorization, execution and delivery by Purchaser, this Agreement constitutes a legal, valid and binding obligation of the Company, enforceable against the Company in accordance with its terms, except as limited by applicable bankruptcy, insolvency, reorganization, moratorium, fraudulent conveyance, and any other laws of general application affecting enforcement of creditors' rights generally, and as limited by laws relating to the availability of specific performance, injunctive relief, or other equitable remedies. The Shares are duly authorized and, when issued, sold and delivered in accordance with the terms and for the consideration set forth in this Agreement, will be validly issued, fully paid and nonassessable and free and clear of any liens or other restrictions, other than restrictions on transfer under applicable state and federal securities laws or such restrictions as the Purchaser has agreed to in writing with the Company, and will not have been issued in violation of or subject to any preemptive or similar rights created under the Company's certificate of incorporation or bylaws or the Delaware General Corporation Law.

4.04 Governmental Consents and Filings. Assuming the accuracy of the representations made by the Purchasers in Section 3 hereof and except as set forth in the Purchase Agreement, no material consent, approval, order or authorization of, or registration, qualification, designation, declaration or filing with, or giving of notice to, any Governmental Entity (as defined below) is required on the part of the Company in connection with the execution and delivery of, or the consummation of the transactions contemplated by this Agreement, except for filings pursuant to Regulation D of the 1933 Act and applicable state securities laws, which have been made or will be made in a timely manner.

4.05 Absence of Violations, Defaults and Conflicts. Neither the Company nor any of its subsidiaries is (i) in violation of its certificate or articles of incorporation, bylaws or similar organizational document, (ii) in default in the performance or observance of any obligation, agreement, covenant or condition contained in any contract, indenture, mortgage, deed of trust, loan or credit agreement, note, lease or other agreement or instrument to which the Company or any of its subsidiaries is a party or by which it or any of them may be bound or to which any of the properties or assets of the Company or any subsidiary is subject (collectively, "*Agreements and Instruments*"), except for such defaults that would not, singly or in the aggregate, result in a Material Adverse Effect, or (iii) in violation of any law, statute, rule, regulation, judgment, order, writ or decree of any arbitrator, court, governmental body, regulatory body, administrative agency or other authority, body or agency

having jurisdiction over the Company or any of its subsidiaries or any of their respective properties, assets or operations (each, a “**Governmental Entity**”), except for such violations that would not, singly or in the aggregate, result in a Material Adverse Effect. The execution, delivery and the performance of this Agreement and the consummation of the transactions contemplated herein (including the issuance and sale of the Shares) and compliance by the Company with its obligations hereunder do not and will not, whether with or without the giving of notice or passage of time or both, (1) conflict with or constitute a breach of, or default under, or result in the creation or imposition of any lien, charge or encumbrance upon any properties or assets of the Company or any subsidiary pursuant to, the Agreements and Instruments, (2) result in any violation of the provisions of the certificate or articles of incorporation, bylaws or similar organizational document of the Company or any of its subsidiaries or (3) result in any violation of any applicable law, statute, rule, regulation, judgment, order, writ or decree of any Governmental Entity, except in the case of clauses (1) and (3), for such violations as would not, singly or in the aggregate, have or reasonably be expected to have a Material Adverse Effect, or materially affect the validity of the Shares or the legal authority of the Company to perform its obligations hereunder and timely comply in all material respects with the terms of this Agreement or the Purchase Agreement.

4.06 Absence of Proceedings. There is no action, suit, proceeding or, to the knowledge of the Company, inquiry or investigation, before or brought by any Governmental Entity now pending or, to the knowledge of the Company, threatened, against or affecting the Company or any of its subsidiaries, which would have or reasonably be expected to have a Material Adverse Effect or materially affect the validity of the Shares or the legal authority of the Company to perform its obligations hereunder and timely comply in all material respects with the terms of this Agreement or the Purchase Agreement.

4.07 Possession of Licenses and Permits. The Company and its subsidiaries possess such permits, licenses, approvals, consents and other authorizations (collectively, “**Governmental Licenses**”) issued by the appropriate Governmental Entities necessary to conduct the business now operated by them, except where the failure so to possess would not, singly or in the aggregate, have or reasonably be expected to have a Material Adverse Effect. The Company and its subsidiaries are in compliance with the terms and conditions of all Governmental Licenses, except where the failure so to comply would not, singly or in the aggregate, have or reasonably be expected to have a Material Adverse Effect. All of the Governmental Licenses are valid and in full force and effect, except when the invalidity of such Governmental Licenses or the failure of such Governmental Licenses to be in full force and effect would not, singly or in the aggregate, have or reasonably be expected to have a Material Adverse Effect. Neither the Company nor any of its subsidiaries has received any notice of proceedings relating to the revocation or modification of any Governmental Licenses which, singly or in the aggregate, if the subject of an unfavorable decision, ruling or finding, would have or reasonably be expected to have a Material Adverse Effect.

4.08 Payment of Taxes. All United States federal income tax returns of the Company and its subsidiaries required by law to be filed have been filed and all taxes shown by such returns or otherwise assessed, which are due and payable, have been paid, except assessments against which appeals have been or will be promptly taken and as to which adequate reserves have been provided. No assessment in connection with United States federal tax returns has been made against the Company. The Company and its subsidiaries have filed all other tax returns that are required to have been filed by them through the date hereof or have timely requested extensions thereof pursuant to applicable foreign state, local or other law except insofar as the failure to file such returns would not have or reasonably be expected to have a Material Adverse Effect, and has paid all taxes due pursuant to such returns or all taxes due and payable pursuant to any assessment received by the Company and its subsidiaries, except for such taxes, if any, as are being contested in good faith and as to which adequate reserves have been established by the Company or its subsidiaries and except where the failure to pay such taxes would not have or reasonably be expected to have a Material Adverse Effect. The Company is classified as a Subchapter C corporation for U.S. federal tax purposes.

4.09 Insurance. The Company and its subsidiaries carry or are entitled to the benefits of insurance, with what the Company reasonably believes to be financially sound and reputable insurers, in such amounts and covering such risks as is adequate for the conduct of their respective businesses and the value of their respective properties and assets, and all such insurance is in full force and effect. The Company has no reason to believe that it or any of its subsidiaries will not be able (i) to renew its existing insurance coverage as and when such policies expire or (ii) to obtain comparable coverage from similar institutions as may be necessary or appropriate to

conduct its business as now conducted and at a cost that would not have or reasonably be expected to have a Material Adverse Effect.

4.10 Investment Company Act. The Company is not required, and upon the issuance and sale of the Shares will not be required, to register as an “investment company” under the Investment Company Act of 1940, as amended.

4.11 Regulatory Matters. Except as would not, singly or in the aggregate, have or reasonably be expected to have a Material Adverse Effect: (i) neither the Company nor any of its subsidiaries has received any FDA Form 483, notice of adverse finding, warning letter or other correspondence or written notice from the U.S. Food and Drug Administration (“**FDA**”) or any other Governmental Entity alleging or asserting noncompliance with any Applicable Laws (as defined in clause (ii) below) or Authorizations (as defined in clause (iii) below); (ii) the Company and each of its subsidiaries is and has been in compliance with statutes, laws, ordinances, rules and regulations applicable to the Company and its subsidiaries for the ownership, testing, development, manufacture, packaging, processing, use, distribution, marketing, labeling, promotion, sale, offer for sale, storage, import, export or disposal of any product manufactured or distributed by the Company, including without limitation, the Federal Food, Drug, and Cosmetic Act, 21 U.S.C. § 301, et seq., similar laws of other Governmental Entities and the regulations promulgated pursuant to such laws (collectively, “**Applicable Laws**”); (iii) the Company and each of its subsidiaries possesses all licenses, certificates, approvals, clearances, authorizations, permits and supplements or amendments thereto required by any such Applicable Laws and/or to carry on its businesses as now conducted (“**Authorizations**”) and such Authorizations are valid and in full force and effect and the Company is not in violation of any term of any such Authorizations; (iv) neither the Company nor any of its subsidiaries has received notice of any ongoing claim, action, suit, proceeding, hearing, enforcement, investigation, arbitration or other action from any Governmental Entity or third party alleging that any product, operation or activity is in violation of any Applicable Laws or Authorizations or has any knowledge that any such Governmental Entity or third party is considering any such claim, litigation, arbitration, action, suit, investigation or proceeding, nor, to the Company’s knowledge, has there been any noncompliance with or violation of any Applicable Laws by the Company or any of its subsidiaries that could reasonably be expected to require the issuance of any such communication or result in an investigation, corrective action, or enforcement action by FDA or similar Governmental Entity; (v) neither the Company nor any of its subsidiaries has received notice that any Governmental Entity has taken, is taking or intends to take action to limit, suspend, modify or revoke any Authorizations or has any knowledge that any such Governmental Entity is threatening or is considering such action; and (vi) the Company and each of its subsidiaries has filed, obtained, maintained or submitted all reports, documents, forms, notices, applications, records, claims, submissions and supplements or amendments as required by any Applicable Laws or Authorizations and that all such reports, documents, forms, notices, applications, records, claims, submissions and supplements or amendments were complete, correct and not misleading on the date filed (or were corrected or supplemented by a subsequent submission).

4.12 Compliance With Laws. The Company has complied in all material respects with, is not in material violation of, and has not received any written notice alleging any violation with respect to, any applicable provisions of any statute, law or regulation with respect to the conduct of its business, or the ownership or operation of its properties or assets.

4.13 SEC Reports. A copy of each form, report, statement, schedule, prospectus, proxy, registration statement and other document, if any, filed by the Company with the Securities and Exchange Commission (the “**Commission**”) since its initial registration of the class of Common Stock under the 1934 Act (the “**SEC Reports**”) is available to Purchasers via the Commission’s EDGAR system. None of the SEC Reports filed under the 1934 Act contained, when filed or, if amended prior to the date of this Agreement, as of the date of such amendment with respect to those disclosures that are amended, any untrue statement of a material fact or omitted to state a material fact required to be stated therein or necessary to make the statements therein, in the light of the circumstances under which they were made, not misleading. The Company has timely filed each report, statement, schedule, prospectus, and registration statement that the Company was required to file with the Commission since its initial registration of the class of shares of Common Stock under the Exchange Act. There are no material outstanding or unresolved comments in comment letters from the staff of the Division of Corporation Finance with respect to any of the SEC Reports.

4.14 Financial Statements. The Company has filed with the Securities and Exchange Commission its (i) audited balance sheets as of December 31, 2022, together with related audited statements of income, stockholders' equity and cash flows, and notes thereto for the fiscal year then ended, and (ii) unaudited balance sheets as of March 31, 2023, together with related unaudited statements of income, stockholders' equity and cash flows, and notes thereto, of the Company for the fiscal period then ended ((i) and (ii) collectively, the "**Financial Statements**"). The Financial Statements have been prepared in accordance with United States generally accepted accounting principles ("**GAAP**") applied on a consistent basis throughout the periods indicated, except that the unaudited Financial Statements may not contain all footnotes and other presentation items required by GAAP and are subject to normal and recurring year-end adjustments that are not reasonably expected to be material in amount. The Financial Statements fairly present in all material respects the financial condition and operating results of the Company as of the dates, and for the periods, indicated therein, subject in the case of the unaudited Financial Statements to normal year-end audit adjustments. Except as set forth in the Financial Statements, between March 31, 2023, and the date of this Agreement, the Company has not incurred any material liabilities or obligations, contingent or otherwise, other than (a) liabilities incurred in the ordinary course of business; (b) obligations under contracts and commitments incurred in the ordinary course of business; (c) liabilities for transaction expenses incurred in connection with the transactions contemplated by this Agreement and the Purchase Agreement; and (d) liabilities and obligations of a type or nature not required under GAAP to be reflected in the Financial Statements. The Company maintains and will continue to maintain a standard system of accounting established and administered to provide reasonable assurance that transactions are recorded as necessary to permit preparation of the financial statements of the Company in conformity with GAAP. The Company has not extended or maintained credit, arranged for the extension of credit, modified or renewed an extension of credit, in the form of a personal loan or otherwise, to or for any director or executive officer of the Company. The Company meets the requirements for use of Form S-3ASR under the 1933 Act.

4.15 Common Shares. The issued and outstanding shares of Common Stock are registered pursuant to Section 12(b) of the 1934 Act and are listed for trading on the Nasdaq Global Markets under the symbol "MIRM". There is no suit, action, proceeding or investigation pending or, to the knowledge of the Company, threatened against the Company by Nasdaq or the Commission to prohibit or terminate the listing of the shares of Common Stock on the Nasdaq Global Market or to deregister the class of shares of Common Stock under the 1934 Act. The Company has taken no action that is designed to terminate the registration of the class of shares of Common Stock under the 1934 Act.

4.16 Capitalization. As of the date of this Agreement, the authorized capital stock of the Company consists of (i) 10,000,000 shares of preferred stock, par value \$0.0001 per share ("**Preferred Shares**"), and (ii) 200,000,000 shares of Common Stock. As of the date of this Subscription Agreement, (i) no Preferred Shares are issued and outstanding, (ii) 38,294,091 shares of Common Stock are issued and outstanding, (iii) options to acquire 9,578,024 shares of Common Stock are issued and outstanding and (iv) 1,086,749 restricted stock units and/or performance stock units are issued and outstanding. Except as set forth above and pursuant to the other Subscription Agreements and arrangements referred to in the SEC Reports filed prior to the date of this Subscription Agreement, there are no outstanding other rights to subscribe for, purchase or acquire from the Company any shares of Common Stock or other equity interests in the Company, or securities convertible into or exchangeable or exercisable for such equity interests. Other than as disclosed in the SEC Reports, the Company does not have any subsidiaries and does not own, directly or indirectly, interests or investments (whether equity or debt) in any person, whether incorporated or unincorporated. Other than as disclosed in the SEC Reports, there are no stockholder agreements, voting trusts or other agreements or understandings to which the Company is a party or by which it is bound. There are no securities or instruments issued by or to which the Company is a party containing anti-dilution or similar provisions that will be triggered by the issuance of the Shares hereunder or under any other Subscription Agreement, in each case, that have not been or will not be waived on or prior to the Closing Date.

4.17 No Additional Agreements. Other than as specified in this Agreement or as set forth on Schedule 4.16 hereto, the Company has not entered into any side letter or similar agreement with any investor, including, without limitation, any other Purchaser, in connection with such investor's direct or indirect investment in the Company's securities. No Subscription Agreement with any other Purchaser includes terms and conditions that are more favorable to such other Purchaser thereunder than the Purchaser hereunder, other than (i) terms particular to the regulatory requirements of such other Purchaser or its affiliates or related funds that are mutual funds or (ii) the closing mechanics set forth in Section 2.

4.18 Private Placement. None of the Company, its subsidiaries or any person acting on its or their behalf, has, directly or indirectly, made any offers or sales of any security or solicited any offers to buy any security under any circumstances that would require registration under the 1933 Act of the Shares being sold pursuant to this Agreement. Assuming the accuracy of the representations and warranties of the Purchasers contained in Section 3 hereof, the issuance and sale of the Shares is exempt from registration under the 1933 Act.

4.19 No Disqualification Events. No “bad actor” disqualifying event described in Rule 506(d)(1)(i)-(viii) of the 1933 Act (a “**Disqualification Event**”) is applicable to the Company or, to the Company’s knowledge, any Company Covered Person (as defined below), except for a Disqualification Event as to which Rule 506(d)(2)(ii-iv) or (d)(3) is applicable. “**Company Covered Person**” means, with respect to the Company as an “issuer” for purposes of Rule 506 promulgated under the 1933 Act, any person listed in the first paragraph of Rule 506(d)(1). The Company is not aware of any Person (other than any Company Covered Person) that has been or will be paid (directly or indirectly) remuneration for solicitation of purchasers in connection with the sale of the Shares pursuant to this Agreement. The Company has complied, to the extent applicable, with its disclosure obligations under Rule 506(e).

4.20 No General Solicitation. Neither the Company nor, to the Company’s knowledge, any Person acting on behalf of the Company has, directly or indirectly, offered or sold any of the Shares or Closing Shares or solicited any offers to buy any Shares or Closing Shares, under any circumstances that would require registration under the 1933 Act of the offer and sale of the Shares by any form of general solicitation or general advertising.

4.21 No Integrated Offering. Assuming the accuracy of the Purchasers’ representations and warranties set forth in Section 3 hereof, none of the Company, its subsidiaries or, to the Company’s knowledge, any of its or their Affiliates or any Person acting on its or their behalf has, directly or indirectly, at any time within the past six months, made any offers or sales of any Company security or solicited any offers to buy any security under circumstances that would (i) eliminate the availability of the exemption from registration under Regulation D under the 1933 Act in connection with the offer and sale by the Company of the Shares as contemplated hereby or (ii) cause the offering of the Shares pursuant to this Agreement to be integrated with prior offerings by the Company for purposes of any applicable law, regulation or stockholder approval provisions, including, without limitation, under the rules and regulations of any National Exchange on which any of the securities of the Company are listed or designated, and the Company, its subsidiaries and, to the Company’s knowledge, any of its or their Affiliates or any Person acting on its or their behalf will not, directly or indirectly, take any action or steps that would require the registration of the issuance by the Company of the Shares under the 1933 Act.

4.22 Brokers. Other than the Placement Agents, there is no broker, investment banker, financial advisor, finder or other person which has been retained by or is authorized to act on behalf of the Company that is entitled to any fee or commission in connection with the execution of this Agreement and the consummation of the transactions contemplated hereby. The Purchasers shall have no obligation with respect to any fees or with respect to any claims made by or on behalf of any broker, investment banker, financial advisor, finder or other Person that has been retained by or is authorized to act on behalf of the Company for fees of a type contemplated in this Section 4.19 that may be due in connection with the transactions contemplated by this Agreement.

4.23 Reliance by Purchasers. The Company acknowledges that each Purchaser will rely upon the truth and accuracy of, and the Company’s compliance with, the representations, warranties, agreements, acknowledgments and understandings of the Company set forth in this Agreement.

SECTION 5. Covenants.

5.01 Further Assurances. At or prior to Closing, each party agrees to cooperate and generally do such reasonable acts and things in good faith as may be necessary to timely satisfy each of the conditions to be satisfied by it as provided in Section 6 of this Agreement and effectuate the intents and purposes of this Agreement subject to the terms and conditions hereof.

5.02 Disclosure of Transactions and Other Material Information. The Company, on or before 9:00 a.m., New York City time, on the Business Day immediately following the date of this Agreement (the

“**Disclosure Deadline**”), shall issue one or more press releases (the “**Press Release**”) and file with the Commission a Current Report on Form 8-K (collectively, the “**Disclosure Document**”), which Current Report on Form 8-K shall include as exhibits this Agreement, the Purchase Agreement and the Press Release, disclosing all material terms of the transactions contemplated hereby and any other material nonpublic information within the meaning of the federal securities laws that the Company, Travers or their respective officers, directors, employees, agents, including the Placement Agents, or any other Person acting at the direction or on behalf of the Company or Travers has provided to the Purchasers in connection with the transactions contemplated by this Agreement or the Purchase Agreement prior to the filing of the Disclosure Document (which includes the material terms of the transactions contemplated hereby, the material terms of the Purchase Agreement and the transactions contemplated thereby and any other material non-public information made available to the Purchasers). The Company represents and warrants that, from and after the issuance of the Disclosure Document, no Purchaser shall be in possession of any material, nonpublic information received from the Company, Travers or their respective officers, directors, employees, agents, including Placement Agents, or other Person acting at their direction or on their behalf. The Company shall not, and shall cause Travers and its officers, directors, employees, agents, including the Placement Agents, and any other Person acting at their direction or on their behalf not to, publicly disclose the name of any Purchaser or any affiliate or investment adviser of any Purchaser, or include the name of any Purchaser or any affiliate or investment adviser of any Purchaser without the prior written consent (including by e-mail) of such Purchaser (i) in any press release or marketing materials, or (ii) in any filing with the Commission or any regulatory agency or trading market, except (A) as required by the federal securities laws, rules or regulations, (B) to the extent such disclosure is required by other laws, rules or regulations, at the request of the staff of the Commission or regulatory agency or under regulations of any national securities exchange on which the Company’s securities are listed for trading or (C) to the extent such disclosure contains only information previously approved in accordance with this Section 5.02, and in the case of any disclosure made pursuant to clause (ii), the Company will provide such Purchaser with prior written notice (including by e-mail) of and an opportunity to review and comment on the applicable portion of such filing. Upon the earlier of (i) the Disclosure Deadline, (ii) the issuance of the Press Release and (iii) the filing of the Disclosure Document, each Purchaser shall no longer be subject to any confidentiality or similar obligations under any current agreement, whether written or oral, with the Company, Travers or their respective officers, directors, Affiliates, employees or agents, or any other Person, including the Placement Agents, acting at their direction or on their behalf.

5.03 Listing. The Company shall use its commercially reasonable efforts to take all steps necessary to cause the Shares to be approved for listing on Nasdaq as promptly as possible, but in any event prior to Closing.

5.04 Expenses. The Company and each Purchaser is liable for, and will pay, its own expenses incurred in connection with the negotiation, preparation, execution and delivery of this Agreement, including, without limitation, attorneys’ and consultants’ fees and expenses.

5.05 Blue Sky Laws. The Company, on or before the Closing Date, shall take such action as the Company shall reasonably determine is necessary in order to obtain an exemption for or to qualify the Shares for sale to each Purchaser at the Closing pursuant to this Agreement under applicable securities or “blue sky” laws of the states of the United States (or to obtain an exemption from such qualification). The Company shall make all filings and reports relating to the offer and sale of the Shares required under applicable securities or “blue sky” laws of the states of the United States following the Closing Date.

5.06 Equal Treatment of Purchasers. No consideration shall be offered or paid to any Purchaser to amend or consent to a waiver or modification of any provision of this Agreement unless the same consideration is also offered to all of the Purchasers. This provision constitutes a separate right granted to each Purchaser by the Company and negotiated separately by each Purchaser and shall not in any way be construed as the Purchasers acting in concert or as a group with respect to the purchase, disposition or voting of shares of Common Stock or otherwise.

SECTION 6. Conditions of Closing.

6.01 Conditions of the Purchasers' Obligations at the Closing. The obligations of each Purchaser under Section 2 hereof are subject to the fulfillment, at or prior to the Closing, of all of the following conditions, unless otherwise waived by such Purchaser solely as to itself.

(a) Representations and Warranties. The representations and warranties of the Company contained in this Agreement shall be true and correct in all respects on the Effective Date, and shall be true and correct in all material respects on and as of the Closing Date with the same force and effect as though such representations and warranties had been made on and as of the Closing Date (except (i) to the extent expressly made as of an earlier date in which case such representations and warranties shall be true and correct as of such earlier date and (ii) representations and warranties that are qualified as to materiality or Material Adverse Effect, which representations and warranties shall be true and correct in all respects on and as of the Closing Date (except to the extent that such representations and warranties shall be true and correct as of such earlier date)).

(b) Performance. The Company shall have performed and complied in all material respects with all covenants, agreements, obligations and conditions contained in this Agreement that are required to be performed or complied with by it on or prior to the Closing Date.

(c) Compliance Certificate. The Chief Executive Officer of the Company shall have delivered to the Purchasers at the Closing Date a certificate, in form and substance reasonably acceptable to the Purchasers, certifying that the conditions specified in Sections 6.01(a), 6.01(b), 6.01(d), 6.01(f), 6.01(g), 6.01(h), and 6.01(n) of this Agreement have been fulfilled.

(d) Qualification under Securities Laws; Consents. All registrations, qualifications, permits and approvals, if any, required under applicable securities laws shall have been obtained for the lawful execution, delivery and performance of this Agreement. The Company shall have obtained in a timely fashion any and all other consents, permits, approvals, registrations and waivers necessary for consummation of the purchase and sale of the Shares and issuance of the Closing Shares (including the waiver of any applicable registration rights that could affect the rights of the Purchasers under this Agreement), all of which shall be and remain so long as necessary in full force and effect.

(e) Secretary's Certificate. The Secretary of the Company shall have delivered to the Purchasers at the Closing a certificate, in form and substance reasonably acceptable to the Purchasers (such consent not to be unreasonably withheld, conditioned or delayed), certifying (i) the certificate of incorporation and bylaws of the Company, (ii) authorization of the Board of Directors of the Company approving this Agreement and the transactions contemplated under this Agreement (including the Purchase Agreement) and (iii) as to certificates evidencing the good standing of the Company in Delaware issued by the Secretary of State of Delaware as of a date within five Business Days of the Closing Date.

(f) Transaction. All conditions to the closing of the Transaction shall have been satisfied or waived (other than the Closing hereunder and other than those conditions which, by their nature, are to be satisfied at the closing of the transactions contemplated by the Purchase Agreement), and the closing of the Transaction shall be set to occur immediately prior to the Closing hereunder. No amendment or waiver of the Purchase Agreement (as the same exists as of the date hereof in the form provided to the Purchaser) shall have occurred that would reasonably be expected to materially and adversely affect the economic benefits the Purchaser would reasonably expect to receive with regard to the Shares that Purchaser is acquiring pursuant to this Agreement.

(g) No Injunction. No statute, rule, regulation, executive order, decree, ruling or injunction shall have been enacted, entered, promulgated or endorsed by any Governmental Entity that prohibits the consummation of any of the transactions contemplated by this Agreement or the Purchase Agreement.

(h) No Suspension. No suspension by The Nasdaq Stock Market, LLC ("*Nasdaq*") of the qualification of the Common Stock for offering or sale or trading, or initiation or threatening of any proceedings by Nasdaq for any of such purposes, shall have occurred.

(i) Other Subscription Agreements. There has been no amendment, modification or waiver of one or more of the other Subscription Agreements (including via a side letter or other agreement) that materially benefits one or more other Purchasers unless the Purchaser has been offered the same benefits.

(j) Registration Rights Agreement. The Company shall have delivered the fully executed Registration Rights Agreement and such agreement shall be in full force and effect.

(k) Opinion of Company Counsel. The Purchasers shall have received from Gibson, Dunn & Crutcher LLP, counsel for the Company, an opinion, dated as of the Closing, in substantially the form of Exhibit C hereto.

(l) Nasdaq. The Company shall have filed with Nasdaq a Listing of Additional Shares notification form for the listing of the Shares and the Closing Shares, a copy of which shall have been made available to the Purchasers upon request and the listing of the Shares and the Closing Shares shall have been approved for listing, subject to official notice of issuance.

(m) Transfer Agent Materials. The Company shall have furnished all required materials to its transfer agent to reflect the issuance of the Shares at the Closing and Traverser shall have furnished all required materials to its transfer agent to reflect the issuance of the Closing Shares at the Effective Time (as defined in the Purchase Agreement).

(n) No Material Adverse Effect. Since the Effective Date, no Material Adverse Effect shall have occurred.

6.02 Conditions of the Company's Obligations. The obligations of the Company under Section 2 hereof are subject to the fulfillment, at or prior to the Closing, of all of the following conditions, any of which may be waived in whole or in part by the Company in its absolute discretion.

(a) Representations and Warranties. The representations and warranties of the Purchasers contained in this Agreement shall be true and correct as of the Effective Date and true and correct in all material respects on and as of the Closing Date with the same effect as though such representations and warranties had been made on and as of the Closing Date (except to the extent expressly made as of an earlier date in which case such representations and warranties shall be true and correct in all material respects as of such earlier date).

(b) Performance. Each Purchaser shall have performed and complied in all material respects with all covenants, agreements, obligations and conditions contained in this Agreement that are required to be performed or complied with by it on or prior to the Closing Date.

(c) Qualification under Securities Laws. All registrations, qualifications, permits and approvals, if any, required under applicable securities laws shall have been obtained for the lawful execution, delivery and performance of this Agreement.

(d) Transaction. All conditions to the closing of the Transaction shall have been satisfied or waived (other than the Closing hereunder and other than those conditions which, by their nature, are to be satisfied at the closing of the transactions contemplated by the Purchase Agreement), and the closing of the Transaction shall be set to occur immediately prior to the Closing hereunder.

(e) Financing Amount. The Company shall receive at Closing the Financing Amount.

SECTION 7. Transfer Restrictions; Restrictive Legend.

7.01 Transfer Restrictions. Each Purchaser agrees not to sell, transfer, pledge, hypothecate or otherwise dispose of all or any part of the Shares unless, prior thereto (a) a registration statement on the appropriate form under the 1933 Act and applicable state securities laws with respect to the Shares proposed to be transferred shall then be effective or (b) the Company has received an opinion from counsel to the Company reasonably satisfactory to

the Company, that such registration is not required because such transaction is exempt from registration under the 1933 Act, and the rules promulgated by the Commission thereunder, including under Rule 144, and with all applicable state securities laws. It is understood that the certificates or book entries evidencing the Shares shall bear substantially the following legend:

“THESE SHARES HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED, OR ANY APPLICABLE STATE SECURITIES LAWS. THEY MAY NOT BE SOLD, OFFERED FOR SALE, PLEDGED OR HYPOTHECATED IN THE ABSENCE OF A REGISTRATION STATEMENT IN EFFECT WITH RESPECT TO THE SHARES UNDER SUCH ACT OR APPLICABLE STATE SECURITIES LAWS OR A VALID EXEMPTION FROM REGISTRATION UNDER SUCH ACT OR APPLICABLE STATE SECURITIES LAWS.”

SECTION 8. Definitions

. Unless the context otherwise requires, the terms defined in this Section 8 shall have the meanings specified for all purposes of this Agreement.

“**1933 Act**” means the Securities Act of 1933, as amended, together with the rules and regulations thereunder.

“**1934 Act**” means the Securities Exchange Act of 1934, as amended, together with the rules and regulations thereunder.

“**Affiliate**” shall have the meaning ascribed to such term in Rule 12b-2 of the General Rules and Regulations under the 1934 Act.

“**Business Day**” means any day except (a) a Saturday, Sunday or (b) any day on which banks in the City of New York are authorized or required by law to remain closed or executive order to be closed; provided, however, that Lincoln’s Birthday (February 12) and Election Day shall not be excluded from the definition of Business Day by virtue of this clause (b).

“**Financing Amount**” means \$210,000,000.

“**National Exchange**” means the Nasdaq Global Select Market, the Nasdaq Global Market, the Nasdaq Capital Market, or the New York Stock Exchange.

“**Person**” means an individual or corporation, partnership, trust, incorporated or unincorporated association, joint venture, limited liability company, joint stock company, government (or an agency or subdivision thereof) or other entity of any kind.

“**Purchase Price**” means an amount equal to \$26.25.

“**Purchaser Majority**” means, prior to the Closing, the Purchasers committed to purchase at least a majority the Shares, provided that each Purchaser who has committed to purchase at least \$20,000,000 of the Shares is included in such majority and, following the Closing, both (i) the Purchasers who hold at least a majority of the Shares still held by the Purchasers, and (ii) each Purchaser (A) whose Subscription Amount exceeds \$20,000,000 and (B) who continues to hold at least fifty percent (50%) of the Shares purchased on the Closing Date.

“**Registration Rights Agreement**” means the Registration Rights Agreement, in the form attached hereto as Exhibit A, to be entered into at the Closing among the Company and each Purchaser.

“**Rule 144**” means Rule 144 promulgated by the Commission pursuant to the 1933 Act, as such Rule may be amended from time to time, or any similar rule or regulation hereafter adopted by the Commission having substantially the same effect as Rule 144.

SECTION 9. Miscellaneous

9.01 Waivers and Amendments. Neither this Agreement, nor any provision hereof, may be changed, waived, amended or modified orally or by course of dealing, but only by an instrument in writing executed by the Company and each Purchaser. No waiver of any default with respect to any provision, condition or requirement of this Agreement shall be deemed to be a continuing waiver in the future or a waiver of any subsequent default or a waiver of any other provision, condition or requirement hereof, nor shall any delay or omission of any party to exercise any right hereunder in any manner impair the exercise of any such right.

9.02 Notices. All notices, requests, consents, and other communications under this Agreement shall be in writing and shall be deemed delivered (a) when delivered, if delivered personally, (b) four Business Days after being sent by registered or certified mail, return receipt requested, postage prepaid, (c) one Business Day after being sent via a reputable nationwide overnight courier service guaranteeing next Business Day delivery, (d) on the date of transmission, if such notice or communication is delivered via electronic mail at the e-mail address specified in this Section 9.02 prior to 5:00 P.M., New York City time, on a Business Day or (e) the next Business Day after the date of transmission, if such notice or communication is delivered by electronic mail at the e-mail address specified in this Section 9.02 on a day that is not a Business Day or after 5:00 P.M., New York City time, on any Business Day, in each case to the intended recipient as set forth below, with respect to the Company, and to the addresses set forth on the Schedule of Purchasers with respect to the Purchasers; *provided*, in the cases of clauses (d) and (e), that notice shall not be deemed given or effective if the sender receives an automatic system-generated response that such electronic mail was undeliverable. The addresses for such notices and communications shall be as follows:

if to the Company:
Mirum Pharmaceuticals, Inc.
950 Tower Lane, Suite 1050
Foster City, CA 94404
Attention: General Counsel
Email: legal@mirumpharma.com

with a copy to (which shall not constitute notice):
Gibson, Dunn & Crutcher LLP
555 Mission Street, Suite 3000
San Francisco, CA 94105
Attention: Ryan Murr, Branden Berns
Email: rmurr@gibsondunn.com, bberns@gibsondunn.com

or at such other address as the Company or each Purchaser may specify by written notice to the other parties hereto in accordance with this Section 9.02.

9.03 Cumulative Remedies. None of the rights, powers or remedies conferred upon each Purchaser, on the one hand, or the Company, on the other hand, shall be mutually exclusive, and each such right, power or remedy shall be cumulative and in addition to every other right, power or remedy, whether conferred by this Agreement or now or hereafter available at law, in equity, by statute or otherwise. Each of the Purchasers and the Company acknowledges and agrees that, in view of the uniqueness of the securities referenced herein and the transactions contemplated hereby and by the Purchase Agreement, money damages will not provide an adequate remedy at law if this Agreement or the Purchase Agreement are not performed in accordance with their terms, and therefore agrees that, in addition to being entitled to exercise all rights provided hereunder or granted by law, including recovery of damages (money or otherwise), each of the Purchasers and the Company shall be entitled to specific performance under this Agreement, without the requirement of either proving the inadequacy of monetary damages as a remedy (or irreparable harm) or the posting of a bond. The parties hereby agree to waive in any action for specific performance of any obligation the defense that a remedy at law would be inadequate.

9.04 Successors and Assigns. All the terms and provisions of this Agreement shall be binding upon and inure to the benefit of and be enforceable by the respective parties hereto, the successors and permitted assigns of each Purchaser and the successors of the Company, whether so expressed or not. None of the Purchasers may assign its rights or obligations hereof without the prior written consent of the Company, except that a Purchaser may, without the prior consent of the Company, assign its rights to purchase the Shares hereunder to any of its

affiliates or to any other investment funds or accounts managed or advised by the investment manager who acts on behalf of Purchaser (provided each such assignee agrees to be bound by the terms of this Agreement and makes the same representations and warranties set forth in Section 3 hereof). The Company may not assign its rights or obligations hereof without the consent of each Purchaser. This Agreement shall not inure to the benefit of or be enforceable by any other person.

9.05 Exculpation of Placement Agents. Each party hereto, individually and not jointly, agrees for the express benefit of each of the Placement Agents, its affiliates and its representatives that:

(a) Each of the Placement Agents is acting solely as financial advisor to the Company in connection with the sale of the Shares and is not acting in any other capacity and is not and shall not be construed as a financial advisor or fiduciary for any Purchaser, or any other person or entity in connection with the sale of Shares.

(b) No Placement Agent or any of its affiliates or any of its representatives (i) shall be liable for any improper payment made in accordance with the information provided by the Company, (ii) has prepared any disclosure or offering document in connection with the offer and sale of the Shares by the Placement Agents or their respective affiliates, (iii) has made or will make any representation or warranty, express or implied, of any kind or character, and has not provided any advice or recommendation to the Purchasers in connection with the purchase or sale of the Shares, (iv) has any responsibilities as to the validity, accuracy, completeness, value or genuineness, as of any date, of any information, certificates or documentation delivered by or on behalf of the Company pursuant to this Agreement, the Registration Rights Agreement or the Purchase Agreement, or in connection with any of the transactions contemplated by such agreements, including any valuation, offering or marketing materials, or any omissions from such materials, (v) has any responsibilities with respect to any representations, warranties or agreements made by any person or entity under or in connection with the transactions contemplated by this Agreement or any of the documents furnished pursuant thereto or in connection therewith, or the execution, accuracy, completeness, value, genuineness, legality, validity or enforceability (with respect to any person) or any thereof, as of any date, of any information, certificates or documentation delivered by or on behalf of the Company pursuant to this Agreement, the Registration Rights Agreement or the Purchase Agreement, or in connection with any of the transactions contemplated by such agreements, including any valuation, offering or marketing materials, or any omissions from such materials, (vi) shall be liable for the business, affairs, financial condition, operations, properties or prospects of, or any other matter concerning the Company, Travers, the Transaction or the transactions contemplated by this Agreement; or (vii) shall be liable or have any obligation (including without limitation, for or with respect to any losses, claims, damages, obligations, penalties, judgments, awards, liabilities, costs, expenses or disbursements incurred by any Purchaser, the Company or any other person or entity), whether in contract, tort or otherwise to any Purchaser or to any person claiming through such Purchaser, (x) for any action taken, suffered or omitted by any of them in good faith and reasonably believed to be authorized or within the discretion or rights or powers conferred upon it by this Agreement, the Registration Rights Agreement or the Purchase Agreement, (y) for anything which any of them may do or refrain from doing in connection with this Agreement, the Registration Rights Agreement or the Purchase Agreement, except for such party's own gross negligence, willful misconduct or bad faith, or (z) for anything otherwise in connection with the purchase and sale of the Shares.

(c) The Placement Agents, their respective affiliates and their respective representatives shall be entitled to rely on, and shall be protected in acting upon, any certificate, instrument, opinion, notice, letter or any other document or security delivered to any of them by or on behalf of the Company.

9.06 Headings. The headings of the Sections and paragraphs of this Agreement have been inserted for convenience of reference only and do not constitute a part of this Agreement.

9.07 Governing Law; Jurisdiction. This Agreement shall be governed by, and construed in accordance with, the laws of the State of Delaware, regardless of the laws that might otherwise govern under applicable principles of conflicts of laws. IN ANY ACTION OR PROCEEDING BETWEEN ANY OF THE PARTIES ARISING OUT OF OR RELATING TO THIS AGREEMENT OR ANY OF THE CONTEMPLATED TRANSACTIONS, EACH OF THE PARTIES: (A) IRREVOCABLY AND UNCONDITIONALLY CONSENTS AND SUBMITS TO THE EXCLUSIVE JURISDICTION AND VENUE OF THE COURT OF CHANCERY OF THE STATE OF DELAWARE OR, TO THE EXTENT SUCH COURT DOES NOT HAVE SUBJECT MATTER

JURISDICTION, THE SUPERIOR COURT OF THE STATE OF DELAWARE OR THE UNITED STATES DISTRICT COURT FOR THE DISTRICT OF DELAWARE, (B) AGREES THAT ALL CLAIMS IN RESPECT OF SUCH ACTION OR PROCEEDING SHALL BE HEARD AND DETERMINED EXCLUSIVELY IN ACCORDANCE WITH CLAUSE (A) OF THIS SECTION 9.07, (C) WAIVES ANY OBJECTION TO LAYING VENUE IN ANY SUCH ACTION OR PROCEEDING IN SUCH COURTS, (D) WAIVES ANY OBJECTION THAT SUCH COURTS ARE AN INCONVENIENT FORUM OR DO NOT HAVE JURISDICTION OVER ANY PARTY, (E) AGREES THAT SERVICE OF PROCESS UPON SUCH PARTY IN ANY SUCH ACTION OR PROCEEDING SHALL BE EFFECTIVE IF NOTICE IS GIVEN IN ACCORDANCE WITH SECTION 9.02 OF THIS AGREEMENT AND (F) IRREVOCABLY WAIVES THE RIGHT TO TRIAL BY JURY.

9.08 Survival. The representations and warranties of the Company and the Purchasers contained in Sections 3 and 4 and the agreements and covenants set forth in Sections 5 and 9 shall survive the Closing for the applicable statute of limitations (unless such covenant or agreement terminates earlier in accordance with its terms), which shall not be extended by Section 8106(c) of Title 10 of the Delaware Code or any similar law. Each Purchaser shall be responsible only for its own representations, warranties, agreements and covenants hereunder.

9.09 Counterparts; Effectiveness. This Agreement may be executed in any number of counterparts and by different parties hereto in separate counterparts, with the same effect as if all parties had signed the same document. All such counterparts (including counterparts delivered by facsimile or other electronic format) shall be deemed an original, shall be construed together and shall constitute one and the same instrument. This Agreement shall become effective when each party hereto shall have received counterparts hereof signed by all of the other parties hereto.

9.10 Entire Agreement. This Agreement, together with the Registration Rights Agreement, contains the entire agreement among the parties hereto with respect to the subject matter hereof and, except as set forth below, this agreement supersedes and replaces all other prior agreements, written or oral, among the parties hereto with respect to the subject matter hereof. Notwithstanding the foregoing or anything to the contrary in this Agreement and subject to Section 5.02, this Agreement shall not supersede any confidentiality or other non-disclosure agreements that may be in place between the Company and any Purchaser as of the date hereof.

9.11 Severability. If any provision of this Agreement shall be found by any court of competent jurisdiction to be invalid or unenforceable, the parties hereby waive such provision to the extent that it is found to be invalid or unenforceable. Such provision shall, to the maximum extent allowable by law, be modified by such court so that it becomes enforceable, and, as modified, shall be enforced as any other provision hereof, all the other provisions hereof continuing in full force and effect.

9.12 Independent Nature of Purchasers' Obligations and Rights. The obligations of each Purchaser under this Agreement are several and not joint with the obligations of any other Purchaser, and no Purchaser shall be responsible in any way for the performance of the obligations of any other Purchaser under this Agreement. Nothing contained herein, and no action taken by any Purchaser pursuant hereto, shall be deemed to constitute the Purchasers as, and the Company acknowledges that the Purchasers do not so constitute, a partnership, an association, a joint venture or any other kind of entity, or create a presumption that the Purchasers are in any way acting in concert or as a group, and the Company will not assert any such claim with respect to such obligations or the transactions contemplated by this Agreement, and the Company acknowledges that the Purchasers are not acting in concert or as a group with respect to such obligations or the transactions contemplated by this Agreement. The Company acknowledges and each Purchaser confirms that it has independently participated in the negotiation of the transaction contemplated hereby with the advice of its own counsel and advisors. Each Purchaser shall be entitled to independently protect and enforce its rights, including, without limitation, the rights arising out of this Agreement, and it shall not be necessary for any other Purchaser to be joined as an additional party in any proceeding for such purpose. The Company has elected to provide all Purchasers with the same terms for the convenience of the Company and not because it was required or requested to do so by any Purchaser.

9.13 Termination. This Agreement shall terminate and be void and of no further force and effect, and all obligations of the parties hereunder shall terminate without any further liability on the part of any party in respect thereof, upon the earlier to occur of (a) such date and time that the Purchase Agreement is terminated in accordance with its terms, (b) upon the mutual written agreement of the Company and the Purchaser

Majority, (c) if, on the Closing Date, any of the conditions of Closing set forth in Section 6 have not been satisfied as of the time required hereunder to be so satisfied or waived by the party entitled to grant such waiver and, as a result thereof, the transactions contemplated by this Agreement are not consummated, or (d) if the Closing has not occurred on or before October 14, 2023, other than as a result of a Willful Breach of a Purchaser's obligations hereunder; *provided, however*, that nothing herein shall relieve any party to this Agreement of any liability for common law fraud or for any Willful Breach of any representation, warranty, covenant, obligation or other provision contained in this Agreement. "**Willful Breach**" means a deliberate act or deliberate failure to act, taken with the actual knowledge that such act or failure to act would result in or constitute a material breach of this Agreement.

9.14 No Third-Party Beneficiaries. This Agreement is intended for the benefit of the parties hereto and their respective successors and permitted assigns and is not for the benefit of, nor may any provision hereof be enforced by, any other Person; provided, however, that each of the Placement Agents will be entitled to rely, as an express third-party beneficiary, on the representations and warranties of the Purchasers and the Company set forth in Section 3 and Section 4 hereof, the covenants set forth in Section 5 hereof and Sections 9.04, 9.05, 9.08, 9.12 and 9.13 hereof.

[Signature pages follow]

IN WITNESS WHEREOF, the parties hereto have caused this Subscription Agreement to be duly executed as of the Effective Date.

MIRUM PHARMACEUTICALS, INC.

By: /s/ Christopher Peetz
Name: Christopher Peetz
Title: President and Chief Executive Officer

[Signature Page to Subscription Agreement]

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Exhibit A

Form of Registration Rights Agreement

**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: August 7, 2023

By: /s/ Christopher Peetz

Christopher Peetz
President and Chief Executive Officer
(Principal Executive and Financial Officer)

**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 June 30, 2023, 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: August 7, 2023

By: _____ /s/ Christopher Peetz

Christopher Peetz
President and Chief Executive Officer
(Principal Executive and Financial Officer)
