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, 2022

OR

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

For the transition period from _____to _____

Commission File Number: 001-38981

Mirum Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**83-1281555**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
950 Tower Lane, Suite 1050, Foster City, California	**94404**
(Address of principal executive offices)	**(Zip Code)**

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

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

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

As of August 1, 2022 the registrant had 32,751,748 shares of common stock, $0.0001 par value per share, outstanding.

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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 our only U.S. Food and Drug Administration ("FDA") approved product and the success of our business depends, in part, on our ability to market and sell Livmarli profitably.
- As a company we currently have limited marketing and sales experience. If we are unable to adequately maintain and scale our marketing and sales capabilities or enter into or maintain rights pursuant to agreements with third parties to market and sell our products, we may not be able to generate viable product revenues. Even if we adequately establish and further maintain such capabilities, market acceptance of our products may be lower than expected.
- Livmarli may fail to achieve the broad degree of physician and patient adoption and use necessary for commercial success.
- We rely completely on third parties to supply, manufacture and distribute drug supplies for Livmarli, including certain sole-source suppliers and manufacturers.
- We have a very limited operating history, and we have incurred significant losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future.
- 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 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.
- 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 applications for marketing authorization with regulatory authorities may not be accepted or may require additional studies, regulatory actions, or manufacturing requirements to be completed before marketing authorization is granted.
- Even if we obtain regulatory approval for our product candidates, our product candidates may not gain market acceptance among physicians, patients, tertiary care centers, transplant centers and others in the medical community.
- 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 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.
- 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.
- 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, 2022 (Unaudited)	December 31, 2021 (Note 2)
Assets		
Current assets:		
Cash and cash equivalents	$ 55,268	$ 31,340
Short-term investments	69,685	125,201
Accounts receivable	13,990	3,267
Inventory	4,524	1,513
Prepaid expenses and other current assets	3,778	5,271
Total current assets	147,245	166,592
Restricted cash equivalents	100,000	100,000
Long-term investments	—	4,983
Property and equipment, net	832	981
Operating lease right-of-use assets	1,362	1,569
Intangible assets, net	46,072	18,740
Other assets	1,722	1,786
Total assets	$ 297,233	$ 294,651
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 8,119	$ 9,166
Accrued expenses	34,539	30,723
Operating lease liabilities	752	711
Derivative liability	1,764	1,996
Total current liabilities	45,174	42,596
Revenue interest liability, net	135,716	129,923
Operating lease liabilities, noncurrent	1,517	1,903
Other liabilities	4,139	17
Total liabilities	186,546	174,439
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, $0.0001 par value; 10,000,000 shares authorized, and zero shares issued and outstanding as of June 30, 2022 and December 31, 2021, respectively	—	—
Common stock, $0.0001 par value; 200,000,000 shares authorized; 32,744,923 shares issued and 32,689,255 shares outstanding, excluding 55,668 shares subject to repurchase as of June 30, 2022; and 30,705,060 shares issued and 30,582,596 shares outstanding, excluding 122,464 shares subject to repurchase as of December 31, 2021	3	3
Additional paid-in capital	431,573	377,403
Accumulated deficit	(320,687)	(257,159)
Accumulated other comprehensive loss	(202)	(35)
Total stockholders' equity	110,687	120,212
Total liabilities and stockholders' equity	$ 297,233	$ 294,651

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,	
	2022	2021	2022	2021
Revenue:				
Product sales, net	$ 17,484	$ —	$ 28,376	$ —
License revenue	—	11,000	2,000	11,000
Total revenue	17,484	11,000	30,376	11,000
Operating expenses:				
Cost of sales	2,524	—	4,948	—
Research and development	25,432	35,048	49,520	73,182
Selling, general and administrative	20,969	13,353	40,085	22,832
Total operating expenses	48,925	48,401	94,553	96,014
Loss from operations	(31,441)	(37,401)	(64,177)	(85,014)
Other income (expense):				
Interest income	293	80	362	229
Interest expense	(3,875)	(4,776)	(7,649)	(8,157)
Change in fair value of derivative liability	232	(1,272)	232	(938)
Other income (expense), net	1,299	(514)	1,145	(530)
Net loss before income taxes	(33,492)	(43,883)	(70,087)	(94,410)
(Benefit) provision for income taxes	(6,570)	11	(6,559)	16
Net loss	$ (26,922)	$ (43,894)	$ (63,528)	$ (94,426)
Net loss per share, basic and diluted	$ (0.84)	$ (1.45)	$ (2.00)	$ (3.13)
Weighted-average shares of common stock outstanding, basic	32,164,174	30,274,749	31,732,596	30,190,352
Weighted-average shares of common stock outstanding, diluted	32,179,171	30,274,749	31,740,136	30,190,352

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)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Net loss	$ (26,922)	$ (43,894)	$ (63,528)	$ (94,426)
Other comprehensive loss:				
Unrealized loss on available-for-sale investments	(22)	(16)	(115)	(90)
Cumulative translation adjustments	(49)	3	(52)	(6)
Comprehensive loss	$ (26,993)	$ (43,907)	$ (63,695)	$ (94,522)

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 and per share data)

	Common Stock		Additional Paid-In	Accumulated	Accumulated Other Comprehensive	Total Stockholders'
	Shares	Amount	Capital	Deficit	Income (Loss)	Equity
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	32,689,255	$ 3	$ 431,573	$ (320,687)	$ (202)	$ 110,687

	Common Stock		Additional Paid-In	Accumulated	Accumulated Other Comprehensive	Total Stockholders'
	Shares	Amount	Capital	Deficit	Income (Loss)	Equity
Balance as of December 31, 2020	29,776,544	$ 3	$ 345,180	$ (173,171)	$ 83	$ 172,095
Issuance of common stock in connection with common stock option exercises	12,535	—	80	—	—	80
Issuance of common stock in public offering, net of issuance costs of $476	375,654	—	7,038	—	—	7,038
Restricted common stock vested in the period	33,396	—	—	—	—	—
Stock-based compensation	—	—	5,285	—	—	5,285
Net loss	—	—	—	(50,532)	—	(50,532)
Other comprehensive loss	—	—	—	—	(83)	(83)
Balance as of March 31, 2021	30,198,129	$ 3	$ 357,583	$ (223,703)	$ —	$ 133,883
Issuance of common stock in connection with common stock option exercises	41,668	—	295	—	—	295
Restricted common stock vested in the period	33,400	—	—	—	—	—
Issuance of common stock in connection with employee stock purchase plan	43,976	—	643	—	—	643
Stock-based compensation	—	—	4,823	—	—	4,823
Net loss	—	—	—	(43,894)	—	(43,894)
Other comprehensive loss	—	—	—	—	(13)	(13)
Balance as of June 30, 2021	30,317,173	$ 3	$ 363,344	$ (267,597)	$ (13)	$ 95,737

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,	
	2022	**2021**
Operating activities		
Net loss	$ (63,528)	$ (94,426)
Reconciliation of net loss to net cash used in operating activities:		
Stock-based compensation	13,379	10,108
Depreciation and amortization	941	169
Amortization of operating lease right-of-use assets	207	191
Net accretion of discounts on investments	(103)	(22)
Non-cash interest expense related to revenue interest liability	7,649	8,157
Change in fair value of derivative liability	(232)	938
Change in fair value of contingent liabilities associated with acquisition	(1,299)	—
Deferred income taxes associated with an acquisition	(6,580)	—
Change in operating assets and liabilities:		
Accounts receivable	(10,723)	—
Prepaid expenses and other current assets	1,493	103
Inventory	(1,247)	—
Other assets	(272)	(162)
Accounts payable, accrued expenses and other liabilities	819	9,856
Operating lease liabilities	(345)	(321)
Net cash used in operating activities	(59,841)	(65,409)
Investing activities		
Purchase of investments	(14,812)	(129,336)
Proceeds from maturities of investments	75,300	70,100
Proceeds from paydown of investments	—	2,000
Purchase of property and equipment	(17)	(3)
Net cash provided by (used in) investing activities	60,471	(57,239)
Financing activities		
Proceeds from issuance of common stock in at-the-market offerings, net of issuance costs	21,289	—
Proceeds from issuance of common stock in public offerings, net of issuance costs	—	6,914
Proceeds from issuance of common stock pursuant to equity award plans	3,917	1,018
Proceeds from revenue interest liability, net of issuance costs	—	64,574
Payments on revenue interest liability	(1,856)	—
Net cash provided by financing activities	23,350	72,506
Effect of exchange rate on cash, cash equivalents and restricted cash equivalents	(52)	(6)
Net increase (decrease) in cash, cash equivalents and restricted cash equivalents	23,928	(50,148)
Cash, cash equivalents and restricted cash equivalents at beginning of period	131,340	142,086
Cash, cash equivalents and restricted cash equivalents at end of period	$ 155,268	$ 91,938
Supplemental disclosure of cash flow information:		
Inventory purchases included in accrued liabilities	$ 1,764	$ —
Transaction costs from acquisition in accrued liabilities	$ 176	$ —
Operating cash flows paid for operating lease	$ 438	$ 426
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	$ —

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

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 received U.S. Food and Drug Administration ("FDA") approval for LIVMARLI® (maralixibat) oral solution ("Livmarli"), the first and only FDA-approved medication for the treatment of cholestatic pruritus in patients with Alagille syndrome ("ALGS") one year of age and older, on September 29, 2021.

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.

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, 2022, the Company had an accumulated deficit of $320.7 million and cash, cash equivalents, restricted cash equivalents and investments of $225.0 million. The Company believes that its cash, unrestricted cash equivalents and investments of $125.0 million as of June 30, 2022, provide sufficient capital resources to continue its operations for at least 12 months from the issuance date of the accompanying unaudited condensed consolidated financial statements. 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 consolidated balance sheet as of December 31, 2021 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, 2021, as filed with the SEC on March 9, 2022.

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.

In December 2019, a novel strain of coronavirus, which causes COVID-19, was identified. Due to the rapid and global spread of the virus, on March 11, 2020, the World Health Organization declared the COVID-19 outbreak a pandemic. To slow the proliferation

of COVID-19, governments have implemented extraordinary measures, which include the mandatory closure of businesses, restrictions on travel and gatherings, and quarantine and physical distancing requirements.

There were no significant estimates contained in the preparation of the Company's unaudited condensed consolidated financial statements or impacts to the Company's unaudited condensed consolidated financial statements that were directly a result of the COVID-19 pandemic or the ongoing Ukraine-Russia conflict. 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.

Significant Accounting Policies

There have been no significant changes to the accounting policies during the six months ended June 30, 2022, 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, except as discussed 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 consists of deposits placed in a segregated bank account as required under the terms of the Company's Revenue Interest Purchase Agreement ("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 a Rare Pediatric Disease Priority Review Voucher in December 2021.

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):

	As of June 30, 2022	As of December 31, 2021
Cash and cash equivalents	$ 55,268	$ 31,340
Restricted cash equivalents	100,000	100,000
Total cash, cash equivalents, and restricted cash equivalents	$ 155,268	$ 131,340

Intangible Assets, Net

The Company accounts for acquisitions of an asset that does not (or a group of assets 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 and 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 becomes impaired, the carrying value is written down to its 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.

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

	June 30, 2022		
	Gross Carrying Value	Accumulated Amortization	Net Carrying Amount
Intangible asset - milestone payments	$ 19,000	$ (779)	$ 18,221
Intangible assets - Satiogen acquisition	28,107	(256)	27,851
Total intangible assets	$ 47,107	$ (1,035)	$ 46,072

	December 31, 2021		
	Gross Carrying Value	Accumulated Amortization	Net Carrying Amount
Intangible asset - milestone payments	$ 19,000	$ (260)	$ 18,740
Total intangible assets	$ 19,000	$ (260)	$ 18,740

Amortization expense was $0.5 million and $0.8 million for the three and six months ended June 30, 2022 and was included in cost of sales on our accompanying unaudited condensed consolidated statements of operations. There was no expense recorded for the three and six months ended June 30, 2021.

The following table summarizes the estimated future amortization expense associated with our intangible assets as of June 30, 2022 (in thousands):

	Amount
2022 (remaining six months)	$ 2,083
2023	4,165
2024	4,165
2025	4,165
2026	4,165
Thereafter	27,329
	$ 46,072

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. 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 in 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 attributable to common stockholders 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 attributable to common stockholders 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 is calculated by dividing net loss by the weighted-average number of shares of common stock and potential dilutive securities outstanding during the period.

The following table sets for the computation of basic and diluted earnings per share (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)
Add: Change in fair value of Holdback Indemnification liability	199	199
Net loss, diluted	$ (27,121)	$ (63,727)
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	32,179,171	31,740,136
Net loss per share, basic	$ (0.84)	$ (2.00)
Net loss per share, diluted	$ (0.84)	$ (2.00)

Basic and diluted net loss per share were the same for the three and six months ended June 30, 2021.

In addition to the equity instruments included above, 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, 2022	As of December 31, 2021
Options to purchase common stock and restricted stock units	8,897,778	6,940,566
Common stock subject to repurchase	55,668	122,464
Employee stock purchase plan contingently issuable	25,562	23,116
Shares issuable as contingent consideration as part of asset acquisition	199,993	—
Total	9,179,001	7,086,146

Recently Adopted Accounting Pronouncements

On January 1, 2022, the Company adopted Accounting Standards Update ("ASU") No. 2020-06, *Debt with Conversion and Other Options (Subtopic 470-20) and Derivatives and Hedging-Contracts in Entity's Own Equity (Subtopic 815-40)* ("ASU 2020-06"), which simplifies the accounting for convertible instruments. The guidance removes certain accounting models that separate the embedded conversion features from the host contract for convertible instruments. There was no impact on the accompanying consolidated financial statements as of the adoption date, January 1, 2022.

Recent Accounting Pronouncements Not Yet Adopted

In June 2016, the FASB issued 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 requires the use of forward-looking expected credit loss models based on historical experience, current conditions, and reasonable and supportable forecasts that affect the collectability of the reported amount, which may result in earlier recognition of credit losses under the new guidance. 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. Subsequent to the issuance of ASU 2016-13, the FASB issued ASU 2018-19, *Codification Improvements to Topic 326, Financial Instruments - Credit Losses*. This ASU does not change the core principle of the guidance in

ASU 2016-13, instead these amendments are intended to clarify and improve operability of certain topics included within the credit losses guidance. The FASB also subsequently issued ASU No. 2019-04, *Codification Improvements to Topic 326, Financial Instruments—Credit Losses, Derivatives and Hedging (Topic 815), and Financial Instruments (Topic 842),* which did not change the core principle of the guidance in ASU 2016-13 but clarified that expected recoveries of amounts previously written off and expected to be written off should be included in the valuation account and should not exceed amounts previously written off and expected to be written off. In March 2020, the 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. The guidance is effective for fiscal years, and interim periods within those years, beginning after December 15, 2019 for public business entities, excluding smaller reporting companies. For smaller reporting companies, the guidance will be effective during the first quarter of 2023. The Company is in the process of assessing the impact adoption will have on its consolidated financial statements.

In October 2021, the FASB, issued Accounting Standards Update No. 2021-08, *Business Combinations (Topic 805), Accounting for Contract Assets and Contract Liabilities from Contracts with Customers*, which requires an entity (acquirer) to recognize and measure contract assets and liabilities acquired in a business combination in accordance with Topic 606, *Revenue from Contracts with Customers*. This update is effective for fiscal years beginning after December 15, 2022, and interim periods within those fiscal years, with early adoption permitted. The amendments should be applied prospectively to business combinations occurring on or after the effective date of the amendments. The Company is currently evaluating the impact the standard will have on its consolidated financial statements.

3. Fair Value Measurements

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

	June 30, 2022			
	Level 1	Level 2	Level 3	Total
Financial assets:				
Money market fund	$ 146,620	$ —	$ —	$ 146,620
U.S. treasury bills	2,993	—	—	2,993
Commercial paper	—	51,841	—	51,841
U.S. government bonds	—	14,851	—	14,851
Total financial assets	$ 149,613	$ 66,692	$ —	$ 216,305
Financial liabilities:				
Derivative liability	—	—	1,764	1,764
Indemnification holdback	—	—	632	632
Contingent milestone liability			3,500	3,500
Total financial liabilities	$ —	$ —	$ 5,896	$ 5,896

	December 31, 2021			
	Level 1	Level 2	Level 3	Total
Financial assets:				
Money market fund	$ 128,420	$ —	$ —	$ 128,420
Commercial paper	—	115,221	—	115,221
U.S. government bonds	—	14,963	—	14,963
Total financial assets	$ 128,420	$ 130,184	$ —	$ 258,604
Financial liabilities:				
Derivative liability	—	—	1,996	1,996
Total financial liabilities	$ —	$ —	$ 1,996	$ 1,996

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, 2022 and December 31, 2021 approximate their related fair values due to the short-term maturities of these instruments. Certain financial instruments classified within Level 2 of the fair value hierarchy include the types of instruments that trade in markets that are not considered to be active, but are valued based on quoted market prices, broker or dealer quotations, or alternative pricing sources with reasonable levels of price transparency. The carrying amount of the revenue interest liability as of June 30, 2022 and December 31, 2021 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 derivative liability, Indemnification Holdback liability and Contingent Milestone liability (each as defined below) 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) contains embedded derivatives 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 June 30, 2022 and December 31, 2021. The estimated probability and timing of underlying events triggering the exercisability of the put options contained within the RIPA, forecasted cash flows and the discount rate are significant unobservable inputs used to determine the estimated fair value of the entire instrument with the embedded derivative. As of June 30, 2022 and December 31, 2021, the discount rate used for valuation of the derivative liability was 15.7%.

The following table provides a summary of the change in the estimated fair value of the Company’s derivative liability, classified as Level 3 in the fair value hierarchy (in thousands):

Balance at December 31, 2021	$	1,996
Change in fair value		—
Balance at March 31, 2022		1,996
Change in fair value		(232)
Balance at June 30, 2022	$	1,764

Indemnification Holdback and Contingent Milestone Liabilities

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”) and contingent consideration upon achievement of a certain milestone (“Contingent Milestone”). The fair value of the Indemnification Holdback and the Contingent Milestone 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, while the fair value of the Contingent Milestone was additionally determined based upon management’s estimate of the probability of the milestone being met. The fair value of the Indemnification Holdback and Contingent Milestone was initially measured on May 20, 2022, the date on which the Company completed the acquisition of Satiogen. The Company assesses the fair value of the Indemnification Holdback and Contingent Milestone each reporting period until resolution of the related contingency and changes in fair value are recorded in other income (expense), net in the accompanying unaudited condensed consolidated statements of operations.

The following table provides a summary of the changes in the estimated fair value classified as Level 3 in the fair value hierarchy (in thousands):

		Indemnification Holdback Liability		Contingent Milestone Liability
Balance as of December 31, 2021	$	—	$	—
Initial recognition		831		4,600
Change in fair value		(199)		(1,100)
Balance as of June 30, 2022	$	632	$	3,500

The Indemnification Holdback and Contingent Milestone liabilities are included in other liabilities in the accompanying unaudited condensed consolidated balance sheets as of June 30, 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, 2022			
	Amortized Cost	Unrealized Gain	Unrealized Loss	Estimated Fair Value
Cash equivalents and investments:				
Money market fund	$ 146,620	$ —	$ —	$ 146,620
U.S. treasury bills	2,995		(2)	2,993
Commercial paper	51,841	—	—	51,841
U.S. government bonds	15,000	—	(149)	14,851
Total cash equivalents and investments	$ 216,456	$ —	$ (151)	$ 216,305
Classified as:				
Cash equivalents				$ 46,620
Cash equivalents - restricted				100,000
Short-term investments				69,685
Total cash equivalents, restricted cash equivalents and investments				$ 216,305

	December 31, 2021			
	Amortized Cost	Unrealized Gain	Unrealized Loss	Estimated Fair Value
Cash equivalents and investments:				
Money market fund	$ 128,420	$ —	$ —	$ 128,420
Commercial paper	115,221	—	—	115,221
U.S. government bonds	14,999	—	(36)	14,963
Total cash equivalents and investments	$ 258,640	$ —	$ (36)	$ 258,604
Classified as:				
Cash equivalents				$ 28,420
Cash equivalents - restricted				100,000
Short-term investments				125,201
Long-term investments				4,983
Total cash equivalents, restricted cash equivalents and investments				$ 258,604

As of June 30, 2022, the remaining contractual maturities of available-for-sale debt securities were as follows (in thousands):

	Estimated Fair Value
Due within one year	$ 69,685
One to two years	—
Total	$ 69,685

During the three and six months ended June 30, 2022 and 2021, 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 other-than-temporary impairment losses on these securities.

5. Balance Sheet Components

Inventory

The following is a summary of the Company's inventory by category (in thousands):

	June 30, 2022	December 31, 2021
Raw material	$ —	$ 856
Work in progress	4,286	570
Finished goods	238	87
Total inventory	$ 4,524	$ 1,513

Accrued Expenses

Accrued expenses consist of the following (in thousands):

	June 30, 2022	December 31, 2021
Accrued clinical trials	$ 10,797	$ 6,732
Accrued professional service fees	5,029	2,458
Accrued contract manufacturing and non-clinical costs	6,659	4,635
Accrued compensation and related benefits	6,823	9,988
Accrued rebates payable	3,485	265
Accrued royalties payable	1,746	345
Accrued collaboration funding	—	6,300
Total accrued expenses	$ 34,539	$ 30,723

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.

The Company may also be 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. The Company was entitled to receive an additional $35.0 million upon FDA approval of Livmarli, which it elected to forgo.

As consideration for such payments, the Purchasers have the right to receive certain revenue interests (the "Revenue Interests") from the Company based on annual product sales, net of Livmarli, which will be tiered payments (the "Revenue Interest Payments") based on whether such 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 will initially be 9.75% (at Tier 1) and 2.0% (at Tier 2 and Tier 3) of such annual net sales. If the Purchasers have received Revenue Interest Payments in an amount equal to or greater than 110.0% of the total payments actually made by the Purchasers to the Company, exclusive of transaction expenses (the "Cumulative Purchaser Payments"), on or prior to December 31, 2026, the Revenue Interests shall be reduced to 2.0% at Tier 1 and 0.0% at Tier 3 for all subsequent calendar years beginning on January 1, 2027. If the Purchasers have not received Revenue Interest Payments in an amount equal to or greater than 110.0% of the Cumulative Purchaser Payments on or prior to December 31, 2026, the Revenue Interests shall be increased for all subsequent calendar years beginning on January 1, 2027 to a single defined rate (with no separate tiers) that would have provided the Purchasers with an amount equal to 110.0% of the Cumulative Purchaser Payments on or prior to December 31, 2026 had such rate applied to Tier 1 of initial Revenue Interest Payments. The Purchasers' rights to receive the Revenue Interest Payments shall terminate on the date on which the Purchasers have received Revenue Interest Payments of 195.0% of the Cumulative Purchaser Payments, unless the RIPA is terminated earlier.

Under the RIPA, the Company has an option (the "Call Option") to terminate the RIPA and repurchase future Revenue Interests at any time upon advance written notice. Additionally, the Purchasers have 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 will be 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 will be 175.0% 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 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 contains 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 are 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 Options under the RIPA that are exercisable by Purchasers upon certain contingent events were determined to be embedded derivatives 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 will be amortized to interest expense over the expected term of the debt using the effective interest method.

As of June 30, 2022 and December 31, 2021, $135.7 million and $129.9 million, respectively, was recorded as a revenue interest liability on the accompanying unaudited condensed consolidated balance sheets. The Company imputes interest expense associated with this liability using the effective interest rate method. The effective interest rate is 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 may vary during the term of the agreement depending on a number of factors, including the level of forecasted product sales, net. The Company evaluates the interest rate quarterly based on its current product sales, net forecasts utilizing the prospective method. A significant increase or decrease in product sales, net will materially impact the revenue interest liability, interest expense and the time period for repayment. The Company recorded interest expense related to this arrangement of $3.9 million and $4.8 million for the three months ended June 30, 2022 and 2021, respectively, and $7.6 million and $8.2 million for the six months ended June 30, 2022 and 2021, respectively.

The Company incurred $0.9 million of issuance costs in connection with the RIPA, which are 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.

The following table summarizes the revenue interest liability activity during the three and six months ended June 30, 2022 (in thousands):

Revenue interest liability at December 31, 2021	$	129,923
Interest expense recognized		3,774
Revenue interest payments		(757)
Revenue interest liability at March 31, 2022	$	132,940
Interest expense recognized		3,875
Revenue interest payments		(1,099)
Revenue interest liability at June 30, 2022	$	135,716

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, and made an upfront payment to Shire of $7.5 million and issued Shire 1,859,151 shares of redeemable common stock with an estimated fair value of $7.0 million, or $3.76 per share. 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. There were no Livmarli development or regulatory milestones achieved during the three and six months ended June 30, 2022 compared to zero and $17.0 million for the three and six months ended June 30, 2021, respectively. There were no volixibat development and regulatory milestones achieved during the three and six months ended June 30, 2022 compared to zero and $2.0 million for the three and six months ended June 30, 2021, respectively. As of June 30, 2022, no milestones had been accrued as there were no potential milestones yet considered probable.

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 is 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 will be required to pay a low single-digit royalty on net sales. The Company's royalty obligations continue 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 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.

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. If the achievement of a certain milestone is not met on or prior to June 30, 2025 in accordance with the terms of the definitive acquisition agreement, then no shares of common stock will be issued. As of June 30, 2022, the milestone had not been met. Through the transaction, the Company obtained all Satiogen licensing payments and Satiogen-owned intellectual property relating to Livmarli and volixibat. The transaction will result 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 comprised 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 in the accompanying unaudited condensed consolidated balance sheet as of June 30, 2022.

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. As of June 30, 2022, there were no 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 is 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. The value of the Indemnification Holdback liability and the Contingent Milestone liability are remeasured at each reporting period until settled, with resulting changes in the fair value recorded in other income (expense) in the unaudited condensed consolidated statement of operations.

The Company acquired Satiogen in a tax-free reorganization, whereby the Company did not receive a step-up in tax basis of the acquired intangible assets, resulting in a deferred tax liability of $6.6 million. The deferred tax liability provided an additional source of taxable income to support the realization of the pre-existing deferred tax assets. As a result, a portion of the Company's valuation allowance was released and a $6.6 million tax benefit was recorded for the three and six months ended June 30, 2022.

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, 2022, 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, was recorded as license revenue in the accompanying consolidated statements of operations. 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 (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 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. For the three and six months ended June 30, 2022, no adjustments were made to the transaction price. 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, 2022, such research and development funding of $0.7 million was recorded as a receivable which was included in accounts receivable on the accompanying unaudited condensed consolidated balance sheets. 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 on the accompanying unaudited condensed consolidated statement of operations for the six months ended June 30, 2022.

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 in the accompanying unaudited condensed consolidated financial statements of operations. 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 will re-evaluate the transaction price at each reporting period as uncertain events are resolved and other changes in circumstances occur. For the three and six months ended June 30, 2022, no adjustments were made to the transaction price.

Licensing Agreement with Takeda

In September 2021, the Company entered into an exclusive licensing agreement with Takeda Pharmaceutical Company Limited ("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.

Option, License and Collaboration Agreement with Vivet

In April 2021, the Company entered into an Option, License and Collaboration Agreement ("Vivet Collaboration Agreement") with Vivet Therapeutics SAS ("Vivet"). Pursuant to the Vivet Collaboration Agreement, Vivet granted the Company the exclusive option, at the Company's discretion, to develop and subsequently commercialize Vivet's two proprietary AAV gene therapy programs for PFIC, subtypes 3 and 2. Under the terms of the Vivet Collaboration Agreement, the Company paid an upfront fee of $4.2 million and agreed to provide funding to support certain research and development costs associated with the two gene therapy programs through July 2023. In December 2021, the Company elected not to exercise its option, terminating the agreement. The Company made a final payment of $6.3 million in January 2022 under the terms of the agreement, which was reflected in accrued expenses at December 31, 2021 on the accompanying unaudited condensed consolidated balance sheets.

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 in 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, 2022, the Company recorded an aggregate ROU asset of $1.4 million and an aggregate lease liability of $2.3 million in the accompanying unaudited condensed consolidated balance sheets. The weighted-average remaining lease term is 2.6 years.

As of June 30, 2022, undiscounted future minimum payments under the Company's operating leases are as follows (in thousands):

	Undiscounted Rent Payments
2022 (remaining six months)	$ 449
2023	916
2024	925
2025	230
Total undiscounted lease payments	2,520
Less: imputed interest	(251)
Total lease liability	$ 2,269

Rent expense was $0.2 million for the three months ended June 30, 2022 and 2021, respectively, and $0.4 million and $0.3 million for the six months ended June 30, 2022 and 2021, respectively. Variable lease payments for operating expenses were immaterial for the three and six months ended June 30, 2022 and 2021.

10. 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, in an at the market offering, 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 three and six months ended June 30, 2022, the Company issued and sold 165,018 and 1,160,915 shares of common stock pursuant to the Sales Agreement at a weighted-average price of $24.78 and $19.01 per share, respectively, resulting in aggregate gross proceeds to the Company of $4.1 million and $22.1 million. The net proceeds for the three and six months ended June 30, 2022, to the Company after deducting sales commissions to SVB Securities and other issuance expenses were approximately $3.9 million and $21.3 million, respectively. As of June 30, 2022, the Company issued and sold an aggregate of 1,466,884 shares of common stock pursuant to the Sales Agreement at a weighted-average price of $19.54 per share, resulting in aggregate gross proceeds to the Company of $28.7 million. The net proceeds to the Company after deducting sales commissions to SVB Securities and other issuance expenses were approximately $27.6 million. The remaining capacity under the Sales Agreement is approximately $46.3 million as of June 30, 2022.

In December 2020, the Company completed an underwritten public offering of its common stock pursuant to the Shelf Registration. The Company sold 3,750,000 shares of common stock at a price of $20.00 per share, resulting in net proceeds of $70.0 million after deducting underwriting discounts, commissions and offering expenses. In addition, the Company granted the underwriters an option, exercisable for 30 days, to purchase up to 562,500 additional shares of its common stock at the public offering price, less the underwriting discounts and commissions. In January 2021, the underwriters partially exercised their option and purchased 375,654 shares of the Company's common stock at a price of $20.00 per share, resulting in net proceeds of $7.1 million after deducting underwriting discounts.

As of June 30, 2022 and December 31, 2021, 55,668 shares and 122,464 shares of common stock, respectively, were subject to repurchase by the Company. The unvested stock liability related to these shares is immaterial to all periods presented.

Common Stock Reserved for Issuance

Common stock reserved for issuance is as follows:

	As of June 30, 2022	As of December 31, 2021
Stock options and restricted stock units issued and outstanding	8,897,778	6,940,566
Reserved for future stock awards or option grants	1,819,116	2,434,619
Reserved for employee stock purchase plan	1,142,089	911,138
Common stock held back in connection with asset acquisition	32,494	—
Common stock issuable as contingent consideration in connection with asset acquisition	199,993	—
	12,091,470	10,286,323

11. 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 ("RSUs") 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, 2022, 1,089,020 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 RSUs 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, 2022, 730,096 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, 2022 (in thousands, except share and per share data):

	Number of Shares	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life (in Years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2021	6,870,617	$ 12.56	8.0	$ 32,637
Granted	1,894,350	$ 17.56		
Exercised	(193,544)	$ 14.95		
Canceled and forfeited	(194,026)	$ 18.72		
Outstanding as of June 30, 2022	8,377,397	$ 13.49	8.0	$ 52,133
Vested and exercisable as of June 30, 2022	3,967,754	$ 10.06	7.2	$ 37,590

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, 2022 and 2021 was $12.37 and $13.97 per share, respectively. The total intrinsic value of options exercised during the six months ended June 30, 2022 and 2021 was $1.7 million and $0.7 million, respectively. As of June 30, 2022, the total unrecognized stock-based compensation related to unvested stock option awards granted was $50.5 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 Company estimated expected volatility based on the historical volatility of a group of similar companies that are publicly traded. The historical volatility data was computed using the daily closing prices for the selected 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,	
	2022	2021	2022	2021
Exercise price	$21.36-$27.46	$16.88-$18.18	$15.50-$27.46	$16.88-$21.22
Expected term (in years)	5.5-6.1	5.5-6.1	5.5-6.1	5.5-6.1
Expected volatility	80.86%-83.20%	83.82%-92.55%	80.86%-83.20%	83.82%-94.06%
Risk-free interest rate	2.78%-3.25%	0.78%-1.12%	1.46%-3.25%	0.62%-1.12%
Expected dividend yield	—	—	—	—

Restricted Stock Units

The following table summarizes the activity under the Company's RSUs for the six months ended June 30, 2022:

	Number of Shares	Weighted-Average Grant Date Fair Value per Share
Unvested and Outstanding as of December 31, 2021	69,949	$ 18.57
Granted	469,602	$ 17.38
Vested	—	$ -
Cancelled/Forfeited	(19,170)	$ 17.11
Unvested and Outstanding as of June 30, 2022	520,381	17.54

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"). The ESPP became effective on July 17, 2019. 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. During the three and six months ended June 30, 2022, 76,099 shares were issued under the ESPP. As of June 30, 2022, the Company had 1,142,089 shares available for future issuance under the ESPP. The stock-based compensation related to the ESPP for the three and six months ended June 30, 2022 was $0.2 million and $0.3 million, respectively.

Restricted 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. The modified shares have a four-year vesting period and a measurement date fair value of $2.94 per share. For the three months ended June 30, 2022 and 2021, 33,398 shares and 33,400 shares vested, respectively. For the six months ended June 30, 2022 and 2021, 66,796 shares and 66,796 shares vested, respectively. As of June 30, 2022, the total unrecognized compensation expense related to unvested restricted stock was $0.2 million expected to be recognized over a weighted-average period of approximately 0.4 year.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Selling, general and administrative	$ 4,225	$ 2,808	$ 8,201	$ 5,350
Research and development	2,416	2,015	5,001	4,758
Total	$ 6,641	$ 4,823	$ 13,202	$ 10,108

12. 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.

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, 2021, which was filed with the Securities and Exchange Commission ("SEC") on March 9, 2022. 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 lead product LIVMARLI (maralixibat) oral solution ("Livmarli"), a novel, oral, minimally-absorbed agent designed to selectively inhibit ASBT, also known as the ileal bile acid transporter ("IBAT"), is approved for the treatment of cholestatic pruritus in patients with Alagille syndrome ("ALGS") 1 year of age and older in the United States. We market and commercialize Livmarli in the United States through our specialized and focused commercial team. We have also filed for approval in Europe of Livmarli for the treatment of cholestasis in patients with ALGS and plan to commercialize Livmarli in Western Europe with our international team based in Switzerland. We have 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"). Livmarli has been granted breakthrough designation for ALGS and PFIC type 2.

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 for the treatment of intrahepatic cholestasis of pregnancy ("ICP"), 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 apical sodium bile acid transporter ("ASBT") and bile acid markers such as 7αC4, fecal bile acids and cholesterol, demonstrating potent biological activity.

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 a limited operating history and incurred significant operating losses since our inception and expect to continue to incur significant operating losses for the foreseeable future. We have only one product approved for commercial sale and have not generated significant revenues from product sales, net as of June 30, 2022. Since inception, we have funded our operations to date primarily through debt, equity, revenue interest financings and, to a lesser extent, cash from our product sales, net and collaboration revenue.

Financing Transactions

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, from time to time, in an at the market offering, 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 three and six months ended June 30, 2022, we issued and sold 165,018 and 1,160,915 shares of common stock pursuant to the Sales Agreement at a weighted-average price of $24.78 and $19.01 per share, respectively, resulting in aggregate gross proceeds to us of $4.1 million and $22.1 million. The net proceeds for the three and six months ended June 30, 2022, to us after deducting sales commissions to SVB Securities and other issuance expenses were approximately $3.9 million and $21.3 million, respectively. As of June 30, 2022, we issued and sold an aggregate of 1,466,884 shares of common stock pursuant to the Sales Agreement at a weighted-average price of $19.54 per share, resulting in aggregate gross proceeds to us of $28.7 million. The net proceeds to us after deducting sales commissions to SVB Securities and other issuance expenses were approximately $27.6 million. The remaining capacity under the Sales Agreement is approximately $46.3 million as of June 30, 2022.

In December 2020, we completed an underwritten public offering of our common stock pursuant to the Shelf Registration. We sold 3,750,000 shares of common stock at a public offering price of $20.00 per share, resulting in net proceeds of $70.0 million after deducting underwriting discounts, commissions and offering expenses. In addition, we granted the underwriters an option, exercisable for 30 days, to purchase up to 562,500 additional shares of our common stock at the public offering price, less the underwriting discounts and commissions. In January 2021, the underwriters exercised their option for 375,654 shares of our common stock resulting in net proceeds of $7.1 million after deducting underwriting discounts.

Financial Overview

Our net loss was $26.9 million and $43.9 million for the three months ended June 30, 2022 and 2021, respectively, and $63.5 million and $94.4 million for the six months ended June 30, 2022 and 2021, respectively. As of June 30, 2022, we had an accumulated deficit of $320.7 million, compared to $257.2 million as of December 31, 2021. As of June 30, 2022, we had cash, cash equivalents, restricted cash equivalents and investments of $225.0 million, compared to $261.5 million as of December 31, 2021, in each case, $100.0 million of which is restricted from use under the terms of the Revenue Interest Purchase Agreement ("RIPA"), 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 expect our expenses and operating losses will increase substantially as we conduct our ongoing and planned clinical trials, continue our research and development activities, continue commercial activities for Livmarli, and seek regulatory approvals for our product candidates, as well as hire additional personnel and protect our intellectual property. In addition, as our product candidates progress through development and toward commercialization, we will need to make milestone payments to the licensors and other third parties from whom we have in-licensed or acquired our product candidates. 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. Arrangements that include upfront payments may require deferral of revenue recognition to a future period until we perform obligations under these arrangements. The event-based milestone and other contingent payments represent variable consideration, and we use the most likely amount method or expected value method to estimate this variable consideration, depending on the nature of the contingency and the variable payments. Given the high degree of uncertainty around the occurrence of these events, we generally determine the milestone and other contingent amounts to be fully constrained until the uncertainty associated with these payments is resolved. We will recognize revenue from sales-based royalty payments when or as the sales occur. We will re-evaluate the transaction price in each reporting period as uncertain events are resolved and other changes in circumstances occur. The timing of these activities may fluctuate significantly. As a result, our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending in particular on the timing of our collaboration event-based milestone and other contingent payments and milestone payments to our licensors.

We expect our product sales from Livmarli will increase in the future. However, the timing and amount of product sales are unknown. Accordingly, until such time as we can generate substantial product revenues to support our cost structure and sustain operating activities, if ever, we expect to finance our cash needs through equity offerings, debt financings or other capital sources, collaborations, licenses and other similar arrangements. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Our failure to raise capital or enter into such other arrangements when needed could have a negative impact on our financial condition and on our ability to pursue our business plans and strategies. If we are unable to raise additional capital when needed, we could be forced to delay, limit, reduce or terminate the development of one or more of our product candidates or future commercialization efforts or grant rights to develop and market our product candidates even if we would otherwise prefer to develop and market such product candidates ourselves.

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.

COVID-19 and Other Geopolitical Events

The coronavirus (including its variants, "COVID-19") pandemic has had a significant economic impact across the global marketplace presenting challenges to maintaining business continuity and dramatically changing the ways in which we live and

interact with others. While vaccines have become widely available in certain countries, and businesses and economies have reopened, the status of global economic recovery remains uncertain and unpredictable, and will continue to be impacted by developments in the pandemic including any subsequent waves of outbreak or new variant strains of the COVID-19 virus which may require re-closures or other preventative measures. As public health directives surrounding the pandemic have relaxed, our offices have reopened and we have begun permitting travel and in-person events, taking into consideration government restrictions, employee safety, and health risks. Our approach may vary among geographies depending on appropriate health protocols, and may change at any time. Additionally, our efforts to reopen our offices safely may not be successful, could expose our employees to health risks, and could involve additional costs or liability.

We are working diligently to ensure the advancement of all of our clinical development programs in the safest manner possible. While we are unable to reliably estimate the duration or extent of any potential business disruption or financial impact during this time, we remain committed to (i) prioritizing the safety, health and well-being of patients, their caregivers, healthcare providers and our employees; (ii) ensuring patients are well supported and have continued uninterrupted access to our product candidates, for which we currently do not expect any supply disruption; and (iii) advancing our clinical trials. Examples include a "Work from Home Policy" for our employees and access to home health care to assist families with safer participation in our trials. Despite these efforts, the COVID-19 pandemic may have long-term effects on the nature of the office environment, remote working and clinical trials, which may present risks for our strategy, operations, development activities, talent recruiting and retention, and workplace culture.

Following the recent invasion of Ukraine by 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 response to the invasion, 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 COVID-19 or the ongoing Ukraine-Russia conflict for the six months ended June 30, 2022, there may be potential impacts to our business in the future that are highly uncertain and difficult to predict such as temporary closures of our offices or those of our third-party manufacturers or suppliers, disruptions 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 products, 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, changes in availability and cost of credit, continued supply chain disruptions and our ability to raise capital and conduct business development activities. The ultimate impact of the COVID-19 pandemic and the ongoing Ukraine-Russia conflict and related sanctions, including 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 throughout 2022.

We continue to believe that existing cash, cash equivalents and investments, excluding restricted cash equivalents, 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 the COVID-19 pandemic, other adverse geopolitical and macroeconomic events, such as the ongoing Ukraine-Russia conflict and related sanctions, 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 Pharmaceutical Company Limited ("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 Pharmaceuticals, Inc. ("Satiogen" and altogether, the "Satiogen License"), Pfizer Inc., and Sanofi, collectively the Assigned License Agreements ("Assigned License Agreements"). In partial consideration for the rights

granted to us under the Shire License Agreement, we made an upfront payment to Shire of $7.5 million and issued Shire 1,859,151 shares of our common stock with an estimated fair value of $7.0 million.

Under the Shire License Agreement and Assigned License Agreements, to date, we have paid aggregate development and regulatory milestones of $51.0 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 will result in a reduction of total licensing royalty obligations for Livmarli and volixibat.

The total potential consideration for the acquisition consisted of a combination of 841,792 shares of common stock, 199,993 of which is subject to achievement of a milestone, 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, was approved by the FDA in September 2021 for the treatment of cholestatic pruritus in patients with ALGS one year of age and older. We recorded our first product sales of Livmarli in October 2021. We expect our product sales of Livmarli will increase as a result of our continued commercial activities. However, as it is early in our product launch, we do not yet have a trend and while we believe revenue will increase as we further engage with health care providers in the United States and globally, we cannot predict revenues with any certainty.

Our revenue from product sales, net further depends on our prescription mix of U.S. commercial payors, Medicaid and free drugs under our patient assistance program. Our experience to date in our recent product launch is not sufficient to allow us to reliably estimate the payor mix and resulting gross to net adjustments.

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 in which achievement of the milestones becomes probable. 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.

We anticipate that our research and development expenses will increase in the future as we continue the clinical and development activity for further indications of Livmarli, as well as expand the clinical and development activity associated with our volixibat pipeline. These increases will likely include increased costs related to hiring of additional development personnel and fees to outside clinical development organizations that support clinical trial activity.

Selling, General and Administrative Expense

Selling expenses consist of professional fees related to preparation and commercialization of Livmarli, finished goods warehousing costs, as well as salaries and related employee benefits for commercial employees, including stock-based compensation.

General and administrative expenses consist primarily of salaries and employee-related costs, including stock-based compensation, for personnel in executive, finance and other administrative functions. Other significant costs include facility-related costs, legal fees relating to intellectual property and corporate matters, professional fees for accounting and consulting services and insurance costs.

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 and accounting activities.

Interest Income

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

Interest Expense

Interest expense for the three and six months ended June 30, 2022 was related to the RIPA. Costs during the period consist 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. We impute 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.

Change in Fair Value of Derivative Liability

Change in fair value of derivative liability consists of the gain or loss from remeasurement of the compound derivative liability related to the revenue interest liability during the period.

Other Income (Expense), Net

Other income (expense), net consists of gain or loss from remeasurement of the liability 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 judgements, 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, 2022 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, except as described below.

Intangible Assets, Net

Intangible assets, net is measured at their fair values as of the acquisition date or, in the case of commercial milestone payments, the date they become due. Intangible assets are generally amortized on a straight-line basis over their estimated useful lives. We base the useful lives and related amortization expense on the period of time we estimate the assets will generate net product sales or otherwise be used. We also periodically review the lives assigned to our intangible assets to ensure that our initial estimates do not exceed any revised estimated periods from which we expect to realize cash flows from the technologies. If a change were to occur in any of the above-mentioned factors or estimates, the likelihood of a material change in our reported results would increase.

We evaluate our intangible assets with finite lives for indications of impairment whenever events or changes in circumstances indicate that the carrying value may not be recoverable. Factors that could trigger an impairment review include significant under-performance relative to expected historical or projected future operating results, significant changes in the manner of our use of the acquired assets or the strategy for our overall business or significant negative industry or economic trends. If this evaluation indicates that the value of the intangible asset may be impaired, we make an assessment of the recoverability of the net carrying value of the asset over its remaining useful life. If this assessment indicates that the intangible asset is not recoverable, based on the estimated undiscounted future cash flows of the technology over the remaining amortization period, we reduce the net carrying value of the related intangible asset to fair value and may adjust the remaining amortization period.

We make significant judgments in relation to the valuation of intangible assets resulting from asset acquisitions, particularly in the forecasts of future operating results that are used in the discounted cash flow valuation models. It is possible that plans may change and estimates used may prove to be inaccurate. If our actual results, or the plans and estimates used in future impairment analyses, are lower than the original estimates used to assess the recoverability of these assets, we could incur additional impairment charges.

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, 2022 and 2021

The following table summarizes our results of operations for the three months ended June 30, 2022 and 2021 (in thousands):

	Three Months Ended June 30,		
	2022	2021	Change
Revenue:			
Product sales, net	$ 17,484	$ —	$ 17,484
License revenue	—	11,000	(11,000)
Total revenue	17,484	11,000	6,484
Operating expenses:			
Cost of sales	$ 2,524	$ —	$ 2,524
Research and development	25,432	35,048	(9,616)
Selling, general and administrative	20,969	13,353	7,616
Total operating expenses	48,925	48,401	524
Loss from operations	(31,441)	(37,401)	5,960
Other income (expense):			
Interest income	293	80	213
Interest expense	(3,875)	(4,776)	901
Change in fair value of derivative liability	232	(1,272)	1,504
Other income (expense), net	1,299	(514)	1,813
Net loss before income taxes	(33,492)	(43,883)	10,391
(Benefit) provision for income taxes	(6,570)	11	(6,581)
Net Loss	$ (26,922)	$ (43,894)	$ 16,972

Product Sales, Net

Product sales, net were $17.5 million for the three months ended June 30, 2022, compared to zero for the three months ended June 30, 2021 as a result of our continued sales of Livmarli following its commercial launch in the United States in September 2021.

License Revenue

There was no license revenue in the three months ended June 30, 2022, compared to $11.0 million for the three months ended June 30, 2021. The decrease in license revenue was due to the upfront license fee associated with the license agreement with CANbridge in the prior year.

Cost of Sales

For the three months ended June 30, 2022, cost of sales was $2.5 million, compared to zero for the three months ended June 30, 2021, and primarily consisted of amortization of capitalized intangible assets, royalties payable on net product sales of Livmarli under licensing agreements and indirect overhead costs associated with the manufacturing and distribution of Livmarli. There were minimal inventory costs reflected in cost of sales as inventory related to current product sold were expensed as research and development costs prior to Livmarli's approval in the United States in September 2021.

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,		
	2022	2021	Change
Product-specific costs:			
Livmarli	$ 9,746	$ 9,385	$ 361
Volixibat	4,840	4,030	810
Non product-specific costs:			
Collaboration funding	—	6,278	(6,278)
Stock-based compensation	2,416	2,015	401
Personnel	7,081	5,631	1,450
License fees (milestone payments)	—	6,161	(6,161)
Other	1,349	1,548	(199)
Total research and development expenses	$ 25,432	$ 35,048	$ (9,616)

Research and development expenses were $25.4 million for the three months ended June 30, 2022, a decrease of $9.6 million compared to the three months ended June 30, 2021. The decrease was primarily due to:

- for Livmarli programs, an increase of $0.4 million, primarily due to an increase of $2.3 million clinical trial expenses primarily for the EMBARK Phase 2b clinical trial for BA and a long term rollover study from the MARCH PFIC Phase 3 clinical trial offset by decreases of $1.2 million in manufacturing activities as a result of prior year completion of a New Drug Application ("NDA") registrational production and $0.7 million of general program expenses;
- for volixibat programs, an increase of $0.8 million, primarily due to an increase of $1.3 million clinical trial expenses for PSC and PBC offset by a decrease of $0.7 million manufacturing activities supporting clinical supply;
- for collaboration funding, a decrease of $6.3 million due to the Vivet Collaboration Agreement, which we terminated in 2021;
- for stock-based compensation, an increase of $0.4 million, related to an increase in employee headcount;
- for personnel related expenses, an increase of $1.5 million, related to an increase in employee headcount to support our development pipeline; and
- for license fees, a decrease of $6.2 million, for the three months ended June 30, 2021 consisting of a $4.2 million upfront fee associated with the Vivet Collaboration Agreement and $2.0 million due to a development milestone payment associated with initiation of the EMBARK Phase 2 clinical trial of maralixibat for BA.

Selling, General and Administrative Expenses

Selling, general and administrative expenses were $21.0 million for the three months ended June 30, 2022, an increase of $7.6 million compared to the three months ended June 30, 2021. The increase was primarily due to an increase of $5.1 million of personnel and other compensation related expenses, including an increase of $1.4 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, such as regulatory compliance, $1.5 million in advertising, promotion and patient support expenses associated with commercial activities for Livmarli, and an increase of $0.9 million in expenses primarily related to general legal and public relations, international expansion activities and other general administrative expenses.

Interest Expense

Interest expense was $3.9 million for the three months ended June 30, 2022 compared to $4.8 million for the three months ended June 30, 2021, a decrease of $0.9 million. The decrease was related to the accreted interest recognized on the revenue interest liability in connection with the RIPA.

Change in Fair Value of Derivative Liability

Change in fair value of derivative liability was $0.2 million gain for the three months ended June 30, 2022, compared to $1.3 million loss for the three months ended June 30, 2021. The change was related to the remeasurement of the derivative liability at June 30, 2022 and reflects a decrease in value of the put option associated with the RIPA resulting from the reduction in time for the put option to occur.

Other income (expense), net

Other income (expense), net was $1.3 million income for the three months ended June 30, 2022, compared to $0.5 million expense for the three months ended June 30, 3021. The change was primarily related to the remeasurement of the Indemnification Holdback and Contingent Milestone liabilities 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 (Benefit) Provision for Income Taxes

Income tax benefit was $6.6 million for the three months ended June 30, 2022 compared to income tax provision of $11.0 thousand for the three months ended June 30, 2021. The increase 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.

Results of Operations for the Six Months Ended June 30, 2022 and 2021

The following table summarizes our results of operations for the six months ended June 30, 2022 and 2021 (in thousands):

	Six Months Ended June 30,		
	2022	2021	Change
Revenue:			
Product sales, net	$ 28,376	$ —	$ 28,376
License revenue	2,000	11,000	(9,000)
Total revenue	30,376	11,000	19,376
Operating expenses:			
Cost of sales	$ 4,948	$ —	$ 4,948
Research and development	49,520	73,182	(23,662)
Selling, general and administrative	40,085	22,832	17,253
Total operating expenses	94,553	96,014	(1,461)
Loss from operations	(64,177)	(85,014)	20,837
Other income (expense):			
Interest income	362	229	133
Interest expense	(7,649)	(8,157)	508
Change in fair value of derivative liability	232	(938)	1,170
Other income (expense), net	1,145	(530)	1,675
Net loss before income taxes	(70,087)	(94,410)	24,323
(Benefit) provision for income taxes	(6,559)	16	(6,575)
Net Loss	$ (63,528)	$ (94,426)	$ 30,898

Product Sales, Net

Product sales, net were $28.4 million for the six months ended June 30, 2022, compared to zero for the six months ended June 30, 2021 as a result of our continued sales of Livmarli following its commercial launch in the United States in September 2021.

License Revenue

License revenue was $2.0 million for the six months ended June 30, 2022, compared to $11.0 million for the six months ended June 30, 2021. The decrease in license revenue was due to an $11.0 million upfront license fee in the prior year period compared to $2.0 million for the six months ended June 30, 2022 related to achievement of a regulatory milestone, both of which were associated with our license agreement with CANbridge.

Cost of Sales

For the six months ended June 30, 2022, cost of sales was $4.9 million, compared to zero for the six months ended June 30, 2021, and primarily consisted of amortization of capitalized intangible assets, royalties payable on net product sales of Livmarli under licensing agreements and indirect overhead costs associated with the manufacturing and distribution of Livmarli. There were minimal inventory costs reflected in cost of sales as inventory related to current product sold were expensed as research and development costs prior to Livmarli approval in the United States in September 2021.

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,		
	2022	2021	Change
Product-specific costs:			
Livmarli	$ 19,219	$ 19,117	$ 102
Volixibat	8,991	6,779	2,212
Non product-specific costs:			
Collaboration funding	—	6,278	(6,278)
Stock-based compensation	5,001	4,758	243
Personnel	14,247	10,544	3,703
License fees (milestone payments)	—	23,161	(23,161)
Other	2,062	2,545	(483)
Total research and development expenses	$ 49,520	$ 73,182	$ (23,662)

Research and development expenses were $49.5 million for the six months ended June 30, 2022, a decrease of $23.7 million compared to the six months ended June 30, 2021. The decrease was primarily due to:

- for Livmarli programs, an increase of $0.1 million, primarily due to an increase of $3.4 million in clinical trial expenses primarily for the EMBARK Phase 2b clinical trial for BA and a long term rollover study from the MARCH PFIC Phase 3 clinical trial offset by decreases of $3.3 million in manufacturing activities as a result of prior year completion of an NDA registrational production and $0.2 million of general program expenses;
- for volixibat programs, an increase of $2.2 million, primarily due to an increase of $2.6 million clinical trial expenses for PSC, PBC and ICP offset by a decrease of $0.6 million manufacturing activities supporting clinical supply;
- for collaboration funding, a decrease of $6.3 million due to the Vivet Collaboration Agreement, which we terminated in 2021;
- for personnel related expenses, an increase of $3.7 million, related to an increase in employee headcount to support our development pipeline;
- for license fees, a decrease of $23.2 million, related to developmental milestone payments for the six months ended June 30, 2021 consisting of $15.0 million associated with the acceptance of our NDA for filing of Livmarli for the treatment of cholestatic pruritus in patients with ALGS one year of age and older, and $4.0 million associated with the initiation of the VISTAS Phase 2b clinical trial of volixibat in PSC and the EMBARK Phase 2 clinical trial of maralixibat for BA, and $4.2 million in an upfront fee associated with the Vivet Collaboration Agreement; and
- for other expense, a decrease of $0.5 million, primarily due to outside consulting expenses and general overhead expenses.

Selling, General and Administrative Expenses

Selling, general and administrative expenses were $40.1 million for the six months ended June 30, 2022, an increase of $17.3 million compared to the six months ended June 30, 2021. The increase was primarily due to an increase of $10.7 million of personnel and other compensation related expenses, including an increase of $2.9 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, such as regulatory compliance, $4.0 million in advertising, promotion and patient support expenses associated with commercial activities for Livmarli, and an increase of $2.6 million in expenses primarily related to general legal and public relations, international expansion activities and other general administrative expenses.

Interest Expense

Interest expense was $7.7 million for the six months ended June 30, 2022 compared to $8.2 million for the six months ended June 30, 2021, a decrease of $0.5 million. The decrease was related to the accreted interest recognized on the revenue interest liability in connection with the RIPA.

Change in Fair Value of Derivative Liability

Change in fair value of derivative liability was $0.2 million gain for the six months ended June 30, 2022, compared to a $0.9 million loss for the six months ended June 30, 2021. The change was related to the remeasurement of the derivative liability at June 30, 2022 and reflects a decrease in value of the put option associated with the RIPA resulting from the reduction in time for the put option to be occur.

Other income (expense), net

Other income (expense), net was $1.1 million income for the six months ended June 30, 2022, compared to $0.5 million expense for the six months ended June 30, 3021. The change was primarily related to the remeasurement of the Indemnification Holdback and Contingent Milestone liabilities associated with the Satiogen acquisition 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 (Benefit) Provision for Income Taxes

Income tax benefit was $6.6 million for the six months ended June 30, 2022, compared to income tax provision of $16.0 thousand for the six months ended June 30, 2021. The increase 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.

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 collaboration revenue. We had $225.0 million of cash, cash equivalents, restricted cash equivalents and investments as of June 30, 2022, compared to $261.5 million as of December 31, 2021, in each case, inclusive of $100.0 million restricted cash equivalents. Since inception, we have incurred operating losses and negative cash flows from operations. As of June 30, 2022, we had an accumulated deficit of $320.7 million, compared to $257.2 million as of December 31, 2021.

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, from time to time, in an at the market offering, 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 three and six months ended June 30, 2022, we issued and sold 165,018 and 1,160,915 shares of common stock pursuant to the Sales Agreement at a weighted-average price of $24.78 and $19.01 per share, respectively, resulting in aggregate gross proceeds to us of $4.1 million and $22.1 million. The net proceeds for the three and six months ended June 30, 2022, to us after deducting sales commissions to SVB Securities and other issuance expenses were approximately $3.9 million and $21.3 million, respectively. As of June 30, 2022, we issued and sold an aggregate of 1,466,884 shares of common stock pursuant to the Sales Agreement at a weighted-average price of $19.54 per share, resulting in aggregate gross proceeds to us of $28.7 million. The net proceeds to us after deducting sales commissions to SVB Securities and other issuance expenses were approximately $27.6 million. The remaining capacity under the Sales Agreement is approximately $46.3 million as of June 30, 2022.

In December 2020, we completed an underwritten public offering of our common stock pursuant to the Shelf Registration. We sold 3,750,000 shares of common stock at a public offering price of $20.00 per share, resulting in net proceeds of $70.0 million after deducting underwriting discounts, commissions and offering expenses. In addition, we granted the underwriters an option, exercisable for 30 days, to purchase up to 562,500 additional shares of our common stock at the public offering price, less the underwriting discounts and commissions. In January 2021, the underwriters exercised their option for 375,654 shares of our common stock resulting in net proceeds of $7.1 million after deducting underwriting discounts.

Based on our current and anticipated level of operations, we believe our cash, unrestricted 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, unrestricted cash equivalents and investments include money market funds, government agency securities, corporate debt 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 commercial launch 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, which consist primarily of research and development expenditures, and to a lesser extent, selling, general and administrative expenditures, including commercial launch 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 by the FDA for the treatment of cholestatic pruritus in patients with ALGS one year of age and older, 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 equity securities, revenue interest financings and collaboration arrangements and, to a lesser extent, from product revenue from the sale of Livmarli. Until such time, if ever, as we can generate substantial product revenue from sales of Livmarli, our current product candidates or any future product candidates, if approved, we expect to finance our cash needs through a combination of equity offerings, debt financings, revenue interest purchase agreements and potential collaboration, license or development agreements. Our primary cash needs are for day-to-day operations, to pay our debt obligations under the RIPA 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 the RIPA, 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 the COVID-19 pandemic and actions taken to slow its spread, as well as adverse geopolitical and macroeconomic developments, such as 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, 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.

Pursuant to the RIPA, the Purchasers have a right to receive Revenue Interests based on product sales, net of Livmarli. The amounts of quarterly Revenue Interest Payments will change each reporting period based upon the underlying product sales, net of Livmarli and will initially be 9.75% of product sales, net. Under the RIPA, every $100.0 million of net sales generated, less than or equal to $350 million in an annual aggregate, would result in a repayment obligation of approximately $9.8 million. Additionally, every $100.0 million of net sales generated in excess of $350.0 million in an annual aggregate would result in a repayment obligation of approximately $2.0 million. In the future, as net sales thresholds set forth in the agreement are met and the repayment percentage rate changes, the amount of the obligation and timing of payment is likely to change. A significant increase or decrease in actual and forecast net sales will materially impact the revenue interest liability, interest expense and the time period for repayment.

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,	
	2022	2021
Net cash used in operating activities	$ (59,841)	$ (65,409)
Net cash provided by (used in) investing activities	60,471	(57,239)
Net cash provided by financing activities	23,350	72,506
Effect of exchange rate on cash, cash equivalents and restricted cash equivalents	(52)	(6)
Net increase (decrease) in cash, cash equivalents and restricted cash equivalents	$ 23,928	$ (50,148)

Net Cash Used in Operating Activities

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 items of $14.0 million. Non-cash items 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 used in operating activities was $65.4 million for the six months ended June 30, 2021, reflecting our net loss of $94.4 million partially offset by non-cash items of $19.5 million. Non-cash items consisted primarily of $10.1 million of stock-based compensation, $8.2 million of effective interest expense in connection with the RIPA, $0.9 million related to the change in fair value of the derivative liability, and $0.4 million of depreciation and amortization of our fixed assets and operating lease right-of use assets. Additionally, cash used in operating activities reflected changes in net operating assets of $9.5 million, consisting primarily of a $9.9 million increase in accounts payable, accrued expenses and other liabilities primarily due to $7.7 million associated with clinical, manufacturing and commercial planning activities and $2.0 million related to a development milestone payment associated with the EMBARK Phase 2 clinical trial of maralixibat for BA, partially offset by a $0.3 million decrease in our operating lease liability.

Net Cash Provided by (Used in) Investing Activities

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 used in investing activities was $57.2 million for the six months ended June 30, 2021, primarily due to $129.3 million used in purchases of investments, partially offset by proceeds of $70.1 million from maturities of investments and proceeds of $2.0 million from paydowns of investments.

Net Cash Provided by Financing Activities

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.

Net cash provided by financing activities was $72.5 million for the six months ended June 30, 2021, due to net proceeds of $64.6 million pursuant to the RIPA, net proceeds of $6.9 million received from the underwriters when they exercised their option to purchase 375,654 shares of our common stock in January 2021 following the follow-on underwritten public offering of our common stock in December 2020, and proceeds of $1.0 million from employee equity award exercises.

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").

We will remain an emerging growth company until the earliest of (i) December 31, 2024, (ii) the last day of the fiscal year in which we have total annual gross revenue of at least $1.07 billion, (iii) the last day of the fiscal year in which we are deemed to be a "large accelerated filer" as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended ("Exchange Act"), or (iv) the date on which we have issued more than $1.0 billion in non-convertible debt securities during the prior three-year period.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

Interest Rate Risk

Our cash, cash equivalents, restricted cash equivalents and investments as of June 30, 2022 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. For instance, due to the short-term nature of the instruments in our portfolio and the low risk profile of our investments, a hypothetical change in interest rates of 100 basis points would not have a material impact on the fair market value of our cash, cash equivalents, restricted cash equivalents and investments as of June 30, 2022. In addition, we maintain significant amounts of cash and cash equivalents at one financial institution that is in excess of federally insured limits.

We have entered into the RIPA. Our primary exposure to interest rate risk is that the effective interest rate on the liability may vary during the term of the RIPA primarily due to the level of actual forecast net product sales. Due to the nature of the RIPA instrument, the total interest due under this facility is fixed. However, a significant increase or decrease in net product sales may materially impact the interest expense recognized each reporting period.

Foreign Currency Rate Risk

Our operations include activities in the United States and Switzerland. In addition, we contract with vendors that are located outside of the United States and certain invoices are denominated in foreign currencies. 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, 2022 and December 31, 2021, 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 in the six months ended June 30, 2022. We expect similar levels of assets and liabilities to be denominated in foreign currencies in future periods and, thus, do not believe changes in foreign currency exchange rates would have a material impact in future periods.

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. While we are seeing, and expect to continue to see, record inflation due to, among other things, the COVID-19 pandemic and other geopolitical and macroeconomic events, such as the ongoing military conflict between Ukraine and Russia and related sanctions, as of the date of this Quarterly Report on Form 10-Q, we do not expect anticipated changes in inflation to have a material effect on our business, financial condition or results of operations for future reporting periods other than general impacts on companies due to general economic and market conditions.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation and supervision of our Chief Executive Officer and our Chief 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 Chief Executive Officer and our Chief Financial Officer have concluded that, as of the end of the period covered by this Quarterly Report on Form 10-Q, our disclosure controls 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 Chief Executive Officer and Chief 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 Chief Executive Officer and our Chief 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, 2022 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

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

Item 1A. Risk Factors.

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

Risks Related to Commercialization of our Products

Livmarli is our only FDA-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 one year of age and older. The success of our business depends, in part, on our ability to market and sell Livmarli profitably. This depends on a number of factors, including, among others, the following:

- our ability to maintain our sales team and scale our distribution capabilities;
- the availability of adequate reimbursement for 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;
- 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.

As a company, we currently have limited marketing and sales experience. If we are unable to adequately maintain and scale our marketing and sales capabilities or enter into or maintain rights pursuant to agreements with third parties to market and sell our products, we may not be able to generate viable product revenues. Even if we adequately establish and further maintain such capabilities, market acceptance of our products may be lower than expected.

To successfully commercialize Livmarli we must 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 North America to target these centers. We are also establishing a team to target similar centers in Western Europe, if we receive approval of Livmarli in ALGS from the EMA. However, our projections of the commercial and sales needs to target these centers may not be accurate given our limited marketing and sales experience. If we are materially off from our projections, our business and operating results would be harmed.

We are in the process of establishing our capabilities in major European markets and have entered into a limited number of partner and distributor agreements in other select geographies. We will 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.

The growth and maintenance of our own sales force to market Livmarli are expensive and time-consuming. Moreover, we may not be able to successfully or adequately develop this capability for our product candidates in development. We compete with other pharmaceutical and biotechnology companies to recruit, hire, train and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our products. To the extent we also 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 or termination of rights pursuant to these agreements could delay or hinder our commercialization efforts. In the event we are unable to successfully develop, maintain and grow our own marketing and sales force or collaborate with a third-party marketing and sales organization, we would not be able to commercialize Livmarli and our other product candidates, if approved.

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. 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;
- 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 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. 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.

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. 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 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 United States 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 may fail to achieve the broad degree of physician and patient adoption and use necessary for commercial success.

The commercial success of Livmarli depends significantly on the broad adoption and use of the product by physicians and patients for the approved indication. The degree and rate of physician and patient adoption of Livmarli depends on a number of factors, including, among other things:

- patient demand for Livmarli;
- our ability to successfully compete with currently available off-label therapies, future approved therapies, and existing therapies in development and available for use through expanded access programs, including demonstrating that the relative cost, safety and efficacy of Livmarli provides an attractive alternative to such existing therapies;
- the availability of adequate reimbursement from private third-party payors for Livmarli;
- the cost of treatment with Livmarli in relation to alternative treatments and patients willingness to pay for Livmarli;
- acceptance by physicians, tertiary care centers and transplant centers and patients of Livmarli as a safe and effective treatment;
- physician and patient willingness to adopt a new therapy over other available therapies to treat cholestatic pruritus in patients with ALGS;
- patients and physicians perception of cholestatic pruritus as a condition for which available, off-label medical treatments are inadequate;
- overcoming any biases physicians or patients may have toward particular therapies for the treatment of cholestatic pruritus;
- Livmarli patients and caregivers properly using Livmarli as instructed;
- patient satisfaction with the results and administration of Livmarli and overall treatment experience;
- 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;
- the willingness of patients to pay for Livmarli relative to discretionary items;
- the prevalence and severity of side effects from the use or potential misuse of Livmarli;
- limitations or warnings contained in the FDA-approved labeling of Livmarli, and patients' and physicians' assessment of these limitations and warnings;
- the effectiveness of our sales, marketing and distribution efforts and those of the third parties with whom we contract;
- adverse publicity about Livmarli or favorable publicity about competitive products;
- potential product liability claims;
- the ability of specialty pharmacies we contract with to process prescriptions and dispense Livmarli 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 ability to manage our growth and operations to effectively support our commercialization activities.

If Livmarli fails to achieve the broad degree of physician, patient and payor adoption 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 may cause undesirable side effects or have other unexpected properties that could limit its commercial profile, result in post-approval regulatory action or expose us to product liability claims, any of which may adversely impact our business, financial condition, operating results and prospects.

If we or others identify undesirable side effects or other previously unknown problems caused by Livmarli or other products with the same or related active ingredients, a number of potentially negative consequences could result, including, among others:

- the FDA may withdraw its approval of Livmarli;
- we could be sued and held liable for harm caused to patients;

- regulatory authorities may require a recall of Livmarli or we or our potential partners may voluntarily recall the product;
- regulatory authorities may require the addition of warnings or contraindications in the product labeling, narrowing of the indication in the Livmarli label or field alerts to physicians;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients or institute a REMS;
- we may have limitations on how we promote Livmarli;
- we may be required to change the way Livmarli is administered or modify the product in some other way;
- regulatory authorities may require additional clinical trials or costly post-marketing testing and surveillance to monitor the safety or efficacy of Livmarli;
- undesirable side effects may limit physicians' or patients' willingness to initiate or continue therapy with Livmarli;
- sales of Livmarli may decrease significantly; and
- our brand and reputation may suffer.

Any of the above events resulting from undesirable side effects or other previously unknown problems could prevent us or our potential partners from achieving or maintaining market acceptance of Livmarli and could substantially increase our costs, which may adversely affect our business, financial condition, operating results 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 United States, 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 United States, 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 successful 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 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. We may face product liability suits in the future, and our insurance coverage may not be sufficient to cover our liability under any such cases.

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, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our product or product candidates. If we cannot successfully defend ourselves against product liability claims, we will incur substantial liabilities and reputational harm. 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;
- product recall or withdrawal from the market or labeling, marketing or promotional restrictions;
- impairment of our business reputation;
- substantial costs of any related litigation or similar disputes;
- distraction of management's attention and other resources from our primary business;
- substantial monetary awards to patients or other claimants against us that may not be covered by insurance; or
- 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 and when approved, or if physicians misuse or use off-label Livmarli or our other product candidates, if and when 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 and other regulatory agencies 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 or such other regulatory agencies 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 United States for cholestatic pruritus associated with ALGS in patients one year of age and older. If we are found to have promoted off-label uses of Livmarli or our other product candidates, if and when approved, we may receive warning or untitled letters and become subject to significant criminal and civil liability, which would materially harm our business. 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.

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. The federal government has levied significant civil and criminal penalties against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion and to undertake corrective remedies. 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.

We cannot, however, prevent a physician from prescribing Livmarli or our other product candidates, if and when approved, outside of their approved indication when, in the physician's independent professional medical judgment, he or she deems appropriate. Physicians or patients may also misuse Livmarli or our other product candidates, if and when approved, potentially leading to adverse results, side effects or injury, which may lead to product liability claims. If misused, we may become subject to costly litigation by physicians or their patients. Furthermore, the use of Livmarli or our other product candidates, if and when approved, for indications other than those approved by the FDA may not effectively treat such conditions, which could harm our reputation among physicians and patients.

We rely completely on third parties to supply, manufacture and distribute drug supplies for Livmarli, including certain sole-source suppliers and manufacturers.

We do not currently have, nor do we plan to acquire, the infrastructure or capability to supply, manufacture or distribute Livmarli, without third parties. Our ability to commercially supply Livmarli depends, in part, on our ability to contract with 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. If we fail to develop and maintain supply relationships with these third parties, we may be unable to successfully commercialize Livmarli.

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

- fail to supply us with product on a timely basis or in the requested amount due to unexpected damage to or destruction of facilities, equipment, deliveries 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 commercial needs;
- be unable to meet our production demands, including due to issues related to their reliance on sole-source suppliers and manufacturers;
- supply us with product that fails to meet regulatory requirements or our requirements;
- become unavailable through business interruption or financial insolvency;
- lose regulatory status as an approved source;
- be unable or unwilling to renew current supply agreements when such agreements expire on a timely basis, on acceptable terms or at all; or
- discontinue production or manufacturing of necessary drug substances or products.

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 and foreign regulatory authorities. Any need to find and qualify new suppliers or manufacturers could delay production of Livmarli indefinitely, adversely impact our ability to market Livmarli and adversely affect our business. Replacements may not be available to us on a timely basis, on acceptable terms or at all. Additionally, we and our manufacturers do not currently maintain significant inventory of drug substances and other materials. In addition, quarantines, shelter-in-place and similar government orders, or the perception that such orders, shutdowns or other restrictions on the conduct of business operations could occur, related to COVID-19 or other infectious diseases could, impact personnel at our third-party manufacturing facilities upon which we rely, or the availability or cost of materials, which could disrupt the supply chain for Livmarli. Further, our manufacturers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes, component shortages and related supply chain challenges, geopolitical developments such as the conflict between Ukraine and Russia and related sanctions, and increasing inflation rates and the responses by central banking authorities to control such inflation, among others. If our manufacturers were to encounter any of these difficulties, or otherwise fail to comply with their contractual obligations, our ability to commercialize Livmarli would be jeopardized. Any interruption in the supply of a drug substance or other material or in the manufacture of Livmarli could have a material adverse effect on our business, financial condition, operating results and prospects. The FDA has granted Livmarli concurrent release of process performance qualification batches, which is often granted for orphan drugs.

Additionally, 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 the safety of Livmarli is compromised due to a failure to adhere to applicable laws or for other reasons, we may not be able to successfully commercialize our product and we may be held liable for injuries sustained as a result. In addition, the manufacturing facilities of certain of our suppliers are located outside of the United States. This may give rise to difficulties in importing our product into the United States 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. Any of these factors could adversely impact our ability to effectively commercialize Livmarli.

Livmarli is subject to ongoing and continued regulatory oversight. Failure to comply with applicable regulatory requirements could have a material adverse impact on our business.

We are subject to ongoing FDA obligations and continued regulatory review with respect to, among other things, the manufacturing, processing, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion and recordkeeping for Livmarli. These 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").

We are also subject to FDA post-marketing requirements including the conduct and submission of non-clinical, clinical and registry studies.

In addition, manufacturers of drug and biologic products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP regulations. If we or a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where, or processes by which, the product is manufactured, a regulatory agency may impose restrictions on that product or us, including requesting that we initiate a product recall, or requiring notice to physicians, withdrawal of the product from the market or suspension of manufacturing.

We or our current and prospective partners may be subject to product recalls in the future that could harm our brand and reputation and could negatively affect our business.

We or our current and prospective partners may be subject to product recalls, withdrawals or seizures if Livmarli fails to meet specifications or is believed to cause injury or illness or if we are alleged to have violated governmental regulations including those related to manufacturing, labeling, promotion, sale or distribution. Any recall, withdrawal or seizure in the future could materially and adversely affect consumer confidence in our brand and lead to decreased demand for our product. In addition, a recall, withdrawal or seizure of Livmarli would require significant management attention, would likely result in substantial and unexpected expenditures and would harm our business, financial condition, operating results and prospects.

Our customers are concentrated and therefore the loss of a significant customer may harm our business. In addition, the actions of our customers could affect our ability to market and sell Livmarli profitably. Fluctuations in buying or distribution patterns by such customers could adversely affect the collectability of our accounts receivable, revenue, financial condition, or results of operations.

We rely on a specialty pharmacy and a single distributor for all of our sales of Livmarli. Our revenue, financial condition or results of operations may be affected by fluctuations in buying or distribution patterns of these customers. These fluctuations may result from seasonality, pricing, wholesaler inventory objectives, or other factors, including the effects of the COVID-19 pandemic. In addition, the COVID-19 pandemic may increase the proportion of uninsured patients in the United States, which may increase utilization of our patient assistance or free drug program.

Further, if any of these customers becomes subject to bankruptcy, is unable to pay us for our product or is acquired by a company that wants to terminate the relationship with us, or if we otherwise lose any of these significant customers, our revenue, results of operations and cash flows would be adversely affected. Even if we replace the loss of a significant customer, such transition may result in a decline in our revenue, results of operations and cash flows. The failure of any of these customers could adversely affect the collectability of our accounts receivable, our revenue, financial condition or results of operations.

Risks Related to Our Business and Industry

We have a very limited operating history, and we have incurred significant net losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future.

We were incorporated in May 2018 and commenced operations in November 2018, and we have a very limited operating history upon which you can evaluate our business and prospects. Our operations to date have been primarily focused on acquiring and in-licensing Livmarli and volixibat, organizing and staffing our company, business planning, raising capital, advancing Livmarli and volixibat through clinical development, preparing for commercialization of Livmarli and volixibat and subsequently, commercializing Livmarli. We have not yet demonstrated an ability to overcome many of the risks and uncertainties frequently encountered by companies in new and rapidly evolving fields, particularly in the biopharmaceutical area. Consequently, any predictions about our future performance may not be as accurate as they would be if we had a history of successfully developing and commercializing biopharmaceutical products.

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 significant net losses since our inception in May 2018. For the six months ended June 30, 2022 and 2021, we reported a net loss of $63.5 million and $94.4 million, respectively. As of June 30, 2022, we had an accumulated deficit of $320.7 million.

We expect to continue to incur significant net losses for the foreseeable future, and we expect these losses to increase as we continue our clinical development of, and seek regulatory approvals for, our product candidates and as we continue commercializing Livmarli in the United States and prepare for commercialization in Western Europe, if approved. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. Our prior net losses and expected future net losses have had and will continue to have an adverse effect on our stockholders' equity and working capital. Because of the numerous risks and uncertainties associated with drug development, we are unable to accurately predict the timing or amount of increased expenses, or when, if at all, we will be able to achieve profitability.

Our business has been and could continue to be adversely affected by the continued COVID-19 pandemic in regions where we or third parties on which we rely have significant manufacturing facilities, concentrations of clinical trial sites or other business operations. The continuing COVID-19 pandemic could adversely affect our operations, including our workforce, which is currently primarily working remotely and at our clinical trial sites, as well as the business or operations of our manufacturers, clinical research organizations ("***CROs***") ***or other third parties with whom we conduct business.***

Our business has been and could continue to be adversely affected by the global COVID-19 pandemic. In response to COVID-19, we have implemented and continue to enhance safety measures in all our facilities, including establishing clear and regular COVID-19 policies, safety protocols and updates to all employees. The effects of the COVID-19 pandemic and our safety policies may negatively impact productivity, disrupt our business, delay our clinical programs and timelines and hinder our commercialization efforts, the magnitude of which will depend, in part, on the length and severity of any restrictions and limitations on our ability to conduct our business in the ordinary course. As public health directives surrounding the pandemic have relaxed, our offices have reopened and we have begun permitting travel and in-person events, taking into consideration government restrictions, employee safety, and health risks. Our approach may vary among geographies depending on appropriate health protocols, and may change at any time. Additionally, our efforts to reopen our offices safely may not be successful, could expose our employees to health risks, and could involve additional costs or liability. While vaccines have become widely available in certain countries, and businesses and economies have reopened, the status of global economic recovery remains uncertain and unpredictable, and will continue to be impacted by developments in the pandemic including any subsequent waves of outbreak or new variant strains of the COVID-19 virus which may require re-closures or other preventative measures. The COVID-19 pandemic may also have long-term effects on the nature of the office environment and remote working, which may present risks for our strategy, operational, talent recruiting and retention, and workplace culture. These and similar, and perhaps more severe, disruptions in our operations could negatively impact our business, operating results and financial condition.

As a result of COVID-19, we may experience, or continue to experience, ongoing disruptions that could severely impact our business, clinical trials and our commercialization efforts, including:

- the inability or difficulty of our sales force to interact in person with potential prescribers of Livmarli;
- disruption of our commercial supply chain which may lead to delay or loss of revenue and increased cost;
- disruption or delay of physicians seeing patients in person which may prevent such physicians from prescribing Livmarli;
- delays or difficulties in enrolling and retaining patients in our ongoing and planned clinical trials;
- delays in receiving approval from local regulatory authorities to initiate our planned clinical trials;

- delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;
- delays in clinical sites receiving the supplies and materials needed to conduct our clinical trials, including interruption in global shipping that may affect the transport of clinical trial materials;
- changes in local regulations as part of a response to the COVID-19 outbreak which may require us to change the ways in which our clinical trials are conducted, which may result in unexpected costs, or to discontinue the clinical trials altogether;
- diversion of healthcare resources away from the conduct of clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;
- interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel imposed or recommended by federal or state governments, employers and others, or interruption of clinical trial subject visits and study procedures, the occurrence of which could affect the integrity of clinical trial data;
- interruption or delays in the operations of the FDA or other regulatory authorities, which may impact review and approval timelines;
- risk that participants enrolled in our clinical trials will acquire COVID-19 while the clinical trial is ongoing, which could impact the results of the clinical trial, including by increasing the number of observed adverse events; and
- refusal of the FDA or other regulatory authorities to accept data from clinical trials in affected geographies.

These and other disruptions in our operations and the global economy could negatively impact our business, operating results and financial condition.

Our clinical trials have been, and may in the future be, affected by the COVID-19 pandemic. Similarly, our ability to recruit and retain principal investigators and site staff who, as healthcare providers, may have heightened exposure to COVID-19, may be adversely impacted.

Our ongoing or planned clinical trials may also be impacted by interruptions or delays in the operations of the FDA and comparable foreign regulatory agencies. For example, the inability of the FDA to inspect clinical and manufacturing sites due to COVID-19-related travel restrictions may interrupt or delay our NDA and MAA reviews. Any potential interruptions or delays could adversely affect the anticipated timelines of our NDA and MAA reviews.

We and our CROs have also made certain adjustments to the operation of our trials in an effort to ensure the monitoring and safety of patients and minimize risks to trial integrity during the pandemic in accordance with the guidance issued by the FDA, and may need to make further adjustments in the future. For example, in our ongoing clinical trials, we have adopted new protocols to allow for flexibility surrounding patient visits, including to have drugs shipped to patients and for patients to virtually check-in as opposed to attending standard hospital visits. Many of these adjustments are new and untested, may not be effective, and may have unforeseen effects on the progress and completion of our clinical trials and the findings from our clinical trials. In addition, we may encounter delays in shipping of our study drug and other clinical trial materials. These events could delay our clinical trials, increase the cost of completing our clinical trials and negatively impact the integrity, reliability or robustness of the data from our clinical trials.

In addition, quarantines, shelter-in-place and similar government orders, or the perception that such orders, shutdowns or other restrictions on the conduct of business operations could occur, related to COVID-19 or other infectious diseases, could impact personnel at our CROs or third-party manufacturing facilities upon which we rely, or the availability or cost of materials, which could disrupt the supply chain for our product candidates. To the extent our suppliers and service providers are unable to comply with their obligations under our agreements with them or they are otherwise unable to deliver or are delayed in delivering goods and services to us due to the COVID-19 pandemic, our ability to continue meeting clinical supply demand for our product candidates or otherwise advancing development of our product candidates may become impaired.

The spread of COVID-19 and actions taken to reduce its spread may also materially affect us economically. While the potential economic impact brought by, and the duration of, the COVID-19 pandemic may be difficult to assess or predict, there could be a significant disruption of global financial markets, reducing our ability to access capital, which could in the future negatively affect our liquidity and financial position. In addition, the trading prices for other biopharmaceutical companies have been highly volatile as a result of the COVID-19 pandemic. As a result, we may face difficulties raising capital through sales of our common stock or such sales may be on unfavorable terms.

COVID-19 and actions taken to reduce its spread continue to evolve. The extent to which COVID-19 may impede the development and commercialization of our product candidates, affect our ability to market and sell Livmarli, reduce the productivity of our employees, disrupt our supply chains, delay our clinical trials, reduce our access to capital or limit our business development

activities, will depend on future developments, including, without limitation, the effectiveness of government policies, vaccine mandates, vaccine shortages and administration rates, the emergence of new strains or variants of the virus, and other factors that are not foreseeable. Such developments are highly uncertain and cannot be predicted with confidence and any of the foregoing could adversely affect our business, financial condition and results of operations.

In addition, to the extent the ongoing COVID-19 pandemic adversely affects our business and results of operations, it may also have the effect of heightening many of the other risks and uncertainties described in this "Risk Factors" section.

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 or comparable foreign regulatory authorities, and we may never receive such regulatory approvals for our product candidates.

Our clinical trials may not be successful and may not be completed on time or at all, and the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials. For example, in certain of our ongoing clinical trials, the primary efficacy endpoint is a patient-reported outcome or a caregiver-reported outcome measuring the decrease in severity of pruritus. The FDA or comparable foreign regulatory authority 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 or comparable foreign regulatory authorities, which may significantly, adversely and materially affect our ability to successfully commercialize our product candidates. Further, even if we make changes to the study design to address these considerations, the FDA or comparable foreign regulatory authorities may not approve our product candidates.

Even if such regulatory authorities agree with the design and implementation of our clinical trials, such regulatory authorities may change their requirements in the future. In addition, even if the clinical trials are successfully completed, the FDA or foreign regulatory authorities may not interpret the results as we do, and more clinical trials could be required before we submit our product candidates for approval. 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 results from a single Phase 3 clinical trial, if successful, would be sufficient to support an NDA supplement seeking approval for Livmarli for the treatment of pruritus associated with PFIC; however, the FDA may not agree to this approach. Even if we believe the results from our Phase 3 clinical trials are positive, the FDA may require us to conduct additional Phase 3 trials before we are able to submit an application for approval.

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

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

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

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, we are 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 ICP, 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 or in subjects under one year of age. 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. For example, in the Phase 2 INDIGO clinical trial evaluating Livmarli in PFIC1 and PFIC2 patients, the primary efficacy analysis of sBA change from baseline to week 13 did not reach statistical significance for the overall group; however, a 48-week analysis of the clinical trial demonstrated a profound treatment response in a subset of patients with nt-PFIC2. In addition, we do not have experience in conducting placebo-controlled studies for PFIC, and we are studying higher doses of Livmarli than we previously have administered in this setting, in our Phase 3 MARCH clinical trial in PFIC. Furthermore, the Phase 3 MARCH clinical trial in PFIC has enrolled additional genetic sub-types of PFIC patients that may experience different results that those seen in Phase 2 trials. As a result, we may face significant setbacks as we conduct our placebo-controlled Phase 3 clinical trial in PFIC, which may delay or prevent regulatory approval of Livmarli for this indication. Further, as a result of the COVID-19 pandemic, 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 due to COVID-19 or actions taken to slow its spread, the integrity of data from our clinical trials may be compromised or not accepted by the FDA or other regulatory authorities, which would represent a significant setback for the applicable program.

Additional safety data generated from our expanded access program 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 or comparable foreign regulatory authorities along with other information, including information about product candidate chemistry, manufacturing and controls, and our proposed clinical trial protocol, as part of an IND application or similar regulatory filing. Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates in humans. Clinical testing is expensive, time consuming and uncertain as to outcome. In addition, we may rely in part on preclinical, clinical and quality data generated by CROs, and other third parties for regulatory submissions for our product candidates. While we have or will have agreements governing these third parties' services, we have limited influence over their actual performance. If these third parties do not make data available to us, or, if

applicable, do not make regulatory submissions in a timely manner, in each case pursuant to our agreements with them, our development programs may be significantly delayed, and we may need to conduct additional studies or collect additional data independently. In either case, our development costs would increase.

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 or comparable foreign regulatory authorities disagreeing as to the design or implementation of our clinical trials or agreement to commence our clinical trials;
- the FDA or comparable foreign regulatory authorities' failure to accept our proposed manufacturing processes and suppliers and/or requirement to provide additional information regarding our manufacturing processes before providing marketing authorization;
- any failure or delay in reaching an agreement with CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;
- obtaining approval from one or more institutional review boards ("IRBs");
- 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 adverse effects;
- 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 or comparable foreign regulatory authorities to temporarily or permanently shut down due to violations of cGMP, regulations or other applicable requirements, or infections or cross-contaminations of product candidates in the manufacturing process;
- any changes to our manufacturing process, suppliers or formulation that may be necessary or desired;
- third-party vendors not performing manufacturing and distribution services in a timely manner or to sufficient quality standards;
- third-party clinical investigators losing the licenses or permits necessary to perform our clinical trials, not performing our clinical trials on our anticipated schedule or consistent with the clinical trial protocol, GCP, or other regulatory requirements;
- third-party contractors not performing data collection or analysis in a timely or accurate manner;
- third-party contractors becoming debarred or suspended or otherwise penalized by the FDA or other government or regulatory authorities for violations of regulatory requirements, in which case we may need to find a substitute contractor, and we may not be able to use some or all of the data produced by such contractors in support of our marketing applications; or
- the impact of the COVID-19 pandemic on our ongoing and planned clinical trials.

We could also encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by a Data Safety Monitoring Board for such trial or by the FDA or comparable foreign regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including 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 or comparable foreign regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. In addition, changes in regulatory requirements and policies may occur, and we may need to amend clinical trial protocols to comply with these changes. Amendments may require us to resubmit our clinical trial protocols to IRBs for reexamination, which may impact the costs, timing or successful completion of a clinical trial.

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

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA or comparable foreign regulatory authority 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 or comparable foreign regulatory authority, 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 United States and by comparable foreign regulatory authorities in foreign markets. In the United States, 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 United States or internationally, we must demonstrate with substantial evidence from adequate and well-controlled clinical trials, and to the satisfaction of the FDA or comparable foreign regulatory authorities, that such product candidates are safe and effective for their intended uses. Results from non-clinical studies and clinical trials can be interpreted in different ways. Even if we believe the non-clinical or clinical data for our product candidates are promising, such data may not be sufficient to support approval by the FDA and comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authorities, as the case may be, may also require us to conduct additional preclinical studies or clinical trials for our product candidates either prior to or post-approval, or may object to elements of our clinical development program. For example, we have also submitted an MAA for the treatment of cholestasis in patients with ALGS to the EMA and expect potential approval in the fourth quarter of 2022. The MAA is comprised of the long-term ICONIC clinical trial in patients with ALGS, which showed a significant improvement on pruritus ($p<0.0001$) and improvement on other markers of cholestatic liver disease. The ICONIC data is supported by a new analysis, which includes an aggregated cohort of Livmarli-treated patients with ALGS ($n=84$) compared to a natural history control cohort, demonstrating a statistically significant improvement in six-year event-free survival ($p<0.0001$), with events defined as biliary diversion surgery, liver transplant, hepatic decompensation (ascites requiring therapy or variceal bleeding) or death. The EMA may not agree with our interpretation of this data and we may ultimately not achieve approval of Livmarli based on this submission.

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

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

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

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

Even if we eventually complete clinical trials and receive approval of an NDA or foreign marketing application for our product candidates, the FDA or comparable foreign regulatory authority 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. The FDA or the comparable foreign regulatory authority also may approve a product candidate for a more limited indication or patient population than we originally requested, and the FDA or comparable foreign regulatory authority may not approve the labeling that we believe is necessary or desirable for the successful commercialization of a product. Any delay in obtaining, or inability to obtain, applicable regulatory approval would delay or prevent commercialization of that product candidate and would materially adversely impact our business and prospects.

If 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 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 ICP, 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. For example, while we are evaluating Livmarli in patients with different types of PFIC in our Phase 3 MARCH clinical trial in PFIC, in prior studies of Livmarli, the Phase 2 INDIGO clinical trial in particular, all of the multi-parameter responders were in the nt-PFIC2 subpopulation. Further, the primary endpoint in our Phase 3 MARCH clinical trial in PFIC is designed to evaluate Livmarli's effect on pruritus associated with nt-PFIC2. As such, even if our Phase 3 MARCH clinical trial in PFIC shows positive results in other PFIC subgroups, the design of our clinical trial may limit the ability of an NDA supplement to be approved beyond the nt-PFIC2 population, if at all. 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 United States, Livmarli's FDA-approved indication is for the treatment of cholestatic pruritus in patients with ALGS one year of age and older. The actual number of ALGS patients one year of age and older 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 United States, 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 United States, 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.

We have submitted an MAA to the EMA for approval of Livmarli for the treatment of cholestasis in patients with ALGS in the EU. As with the FDA, obtaining approval of an MAA from the European Commission, on the basis of a recommendation from the Committee for Medicinal Products for Human Use of the EMA, is a similarly lengthy and expensive process and the EMA has its own procedures for recommending such approval for product candidates. Regulatory authorities in jurisdictions outside of the United States and the EU also have requirements for approval for product candidates with which we must comply prior to marketing in those jurisdictions. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our product candidates in certain countries. 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.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval or result in significant negative consequences following marketing approval, if any.

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 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 or other comparable foreign authorities. For example, we have observed increases in alanine aminotransferase ("ALT"), levels in certain patients being treated with Livmarli with ALGS. Furthermore, the safety profile in patients under one year of age is unknown and may be different than that observed in previous clinical trials. Results of our trials could reveal a high and unacceptable severity and frequency of these or other side effects. In such an event, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval for our product candidates for any or all targeted indications. 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 Phase 1 clinical trials of volixibat, the most common AEs reported were mild to moderate GI events observed in the volixibat groups.

In addition, we maintain an expanded access program for Livmarli. Patients who receive access to unapproved drugs through compassionate use or expanded access programs have life-threatening illnesses and generally have exhausted all other available therapies. The risk for SAEs, including those which may be unrelated to Livmarli, in this patient population is high and could have a negative impact on the safety profile of Livmarli, which could cause significant delays or impair our ability to obtain regulatory approval for Livmarli outside of the United States.

Additionally, if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such product candidates, a number of potentially significant negative consequences could result, including, among other things:

- regulatory authorities may withdraw approvals of such product;
- regulatory authorities may require additional warnings on the label;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients at significant cost;
- we could be sued and held liable for harm caused to patients; and
- our reputation or the reputation of our products may suffer.

Such events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, results of operations and prospects.

If we receive regulatory approval for a product candidate, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense and 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 or a comparable foreign regulatory authority approves a product candidate, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for the approved product will be subject to extensive and ongoing regulatory requirements. For example, the FDA strictly regulates the promotional claims that may be made about drug products. In particular, a product may not be promoted for uses that are not approved by the FDA as reflected in the product's approved labeling. The FDA also requires submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with cGMP requirements and GCP for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with a product candidate, including AEs of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, 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, warning letters or holds on clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or suspension or revocation of license approvals;
- product seizure or detention, or refusal to permit the import or export of a product; and
- injunctions or the imposition of civil or criminal penalties.

The occurrence of any event or penalty described above or any similar event or penalty may inhibit our ability to commercialize our 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 and other regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval for our product candidates. 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 United States or 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.

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 United States 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.

Separately, in response to the global COVID-19 pandemic, in March 2020, the FDA announced its intention to postpone most foreign and domestic inspections of manufacturing facilities and products. In July 2020, the FDA restarted routine pre-announced surveillance inspections and remote interactive evaluations of domestic manufacturing facilities on a risk-based basis. Regulatory authorities outside the United States have adopted similar restrictions or other policy measures in response to the COVID-19 pandemic. If a prolonged government shutdown occurs, or if global health concerns continue to prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

Even if we obtain regulatory approval for our product candidates, our product candidates may not gain market acceptance among physicians, patients, tertiary care centers, transplant centers and others in the medical community.

If any one of our product candidates is approved for commercialization, its acceptance will depend on a number of factors, including, among other things:

- the clinical indications for which the product candidate is approved;
- physicians, major operators of tertiary care centers and transplant centers and patients considering the product as a safe and effective treatment;
- the potential and perceived advantages of the product over alternative treatments;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA or other regulatory authorities, including, in particular, whether the approved label is limited to the treatment of symptoms, such as pruritus, as compared to the treatment of the underlying disease;
- limitations or warnings contained in the labeling approved by the FDA or other regulatory authorities;
- the timing of market introduction of the product as well as competitive products;
- the cost of treatment in relation to alternative treatments;

- the availability of coverage and adequate reimbursement by 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;
- relative convenience and ease of administration, including as compared to alternative treatments and competitive therapies; and
- the effectiveness of our sales and marketing efforts.

If any of our product candidates are approved but fail to achieve market acceptance among physicians, patients or others in the medical community, we will not be able to generate significant revenue, which would have a material adverse effect on our business, prospects, financial condition and results of operations. In addition, even if any of our product candidates gain acceptance, the markets for the treatment of patients with our target indications for Livmarli may not be as significant as we estimate.

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 United States and some 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. In particular, there have 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 United States. 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 the CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending. At this time, we are unsure of the full impact that Affordable Care Act will have on our business. 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. Thus, the Affordable Care Act will remain in effect in its current form. Further, prior to the U.S. Supreme Court ruling, on January 28, 2021, the current administration issued an executive order that initiated a special enrollment period for purposes of obtaining health insurance coverage through the Affordable Care Act marketplace, which began on February 15, 2021 and remained open through August 15, 2021. 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. It is possible that the Affordable Care Act will be subject to judicial or Congressional challenges in the future. It is unclear how any such challenges and the health care reform measures of the current administration will impact the Affordable Care Act and our business.

In addition, 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, will remain in effect through 2030 unless additional Congressional action is taken. However, COVID-19 relief legislation, suspended the 2% Medicare sequester from May 1, 2020 through March 31, 2022. Under current legislation, the actual reduction in Medicare payments will vary from 1% in 2022 to up to 3% in the final fiscal year of this sequester. 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. In addition, Congress is considering additional health reform measures.

Further, there has been heightened governmental scrutiny in the United States 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.

At the federal level, the previous administration used several means to propose or implement drug pricing reform, including through federal budget proposals, executive orders and policy initiatives. For example, on July 24, 2020 and September 13, 2020, the previous administration announced several executive orders related to prescription drug pricing that attempt to implement several of the Administration's proposals. The FDA concurrently released a final rule and guidance in September 2020 implementing a portion of the importation executive order providing pathways for states to build and submit importation plans for drugs from Canada. Further, on November 20, 2020, the HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a new safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufacturers. The implementation of the rule has been delayed until January 1, 2027. On November 20, 2020, CMS issued an interim final rule implementing the previous administration's Most Favored Nation ("MFN") executive order, which would tie Medicare Part B payments for certain physician-administered drugs to the lowest price paid in other economically advanced countries, effective January 1, 2021. As a result of litigation challenging the MFN model, on December 27, 2021, CMS published a final rule that rescinded the MFN model interim final rule. 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. No legislation or administrative actions have been finalized to implement these principles.

At the state level, individual states in the United States 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, these new laws 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. In addition, it is possible that additional governmental action will be taken in response to the COVID-19 pandemic.

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

We plan to seek regulatory approval for our product candidates internationally and, accordingly, we expect that we will be subject to additional risks related to operating in foreign countries if 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, 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 United States;
- potential liability under the FCPA or comparable foreign regulations;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- 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, 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.

If we fail to develop and commercialize additional product candidates, we may be unable to grow our business.

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 and applicable foreign regulatory authorities. All product candidates are prone to the risks of failure that are inherent in pharmaceutical product development, including the possibility that the product candidate will not be shown to be sufficiently safe and/or effective for approval by regulatory authorities. 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, ICP, 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.

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. 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. 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 one year of age and older with ALGS in the United States. 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 Albireo have IBATis in clinical development for cholestatic liver diseases. We are aware that Albireo has received approval for odevixibat for the treatment of pruritus in patients with PFIC in the United States and for the treatment of PFIC in Europe. Albireo 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. Albireo has also announced completion of enrollment of the study of odevixibat in ALGS, more than 50% enrollment of the study of odevixibat in biliary atresia, and plans to pursue other cholestatic liver diseases. We are aware that GlaxoSmithKline plc has completed a Phase 2 trial of its IBAT in PBC patients and has initiated 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 ICP or PSC. We are aware of several agents in clinical development for the treatment of PSC, including Cymabay's seladelpar, DURECT Corporation's DUR928, Gilead Sciences Inc.'s GS-9674, HighTide Biopharmaceutical Inc.'s HTD1801, Immunic Therapeutics' IMU-838, Intercept's Ocaliva, or obeticholic acid, NGM Biopharmaceuticals Inc.'s NGM282 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, Genfit's elafibranor and NGM Biopharmaceuticals, Inc.'s NGM282 and COUR Pharmaceuticals' CNP-104. We are also aware that Cara Therapeutics' Korsuva is in Phase 2 development for the treatment of moderate-to-severe pruritus associated with chronic liver disease.

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 United States 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 United States, or a patient population of greater than 200,000 individuals in the United States, but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. 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 and ALGS in the United States. In October 2020, the FDA granted orphan drug status to Livmarli for the treatment of BA. We also received orphan drug status 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 United States 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, Albireo 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 United States. 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 United States 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.

For example, we entered into a licensing agreement with CANbridge Pharmaceuticals, Inc. (“CANbridge”), pursuant to which CANbridge has agreed to develop and commercialize Livmarli in Greater China (China, Hong Kong, Macau and Taiwan). Under the terms of the licensing agreement, CANbridge has obtained the exclusive right to develop and commercialize Livmarli within the Greater China regions for ALGS, PFIC, and BA. Further, we entered into a licensing agreement, with GC Biopharma, pursuant to which GC Biopharma has agreed to develop and commercialize Livmarli in South Korea. Under the terms of the licensing agreement, GC Biopharma has obtained the exclusive right to develop and commercialize Livmarli within South Korea for ALGS, PFIC, and BA. Lastly, we entered into an exclusive licensing agreement for the development and commercialization of Livmarli in Japan for ALGS, PFIC, and BA with Takeda. 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 trials in cholestatic indications.

Any of these 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.

We may not generate the expected benefits of our acquisition of Satiogen and it could increase our expenses.

We entered into an Agreement and Plan of Merger in May 2022 to acquire all the outstanding equity interests of Satiogen Pharmaceuticals, Inc. ("Satiogen") with the expectation that the acquisition will result in various benefits, including consolidating the economics of our commercial and pipeline programs. Achieving the anticipated benefits of the acquisition is subject to a number of uncertainties, including whether we can integrate the assets acquired.

Failure to achieve these anticipated benefits could result in increased expenses and could adversely affect our business, financial condition, operating results and prospects.

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 be manufactured or sold profitably or achieve market acceptance.

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. We are highly dependent on our management, 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 currently taking place throughout the United States as a result of the COVID-19 pandemic, 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, 2022, we had 171 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 our products 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 our products 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 and other similar foreign regulatory bodies,

including those laws that require the reporting of true, complete and accurate information to the FDA and other similar foreign regulatory bodies; (2) manufacturing standards; (3) healthcare fraud and abuse laws in the United States 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 United States and in the EU, 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 United States 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 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, 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, processing) 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 United States, federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal data privacy laws, and consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act). 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 certain rights related to their personal data. The CCPA provides for civil penalties 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 is expected to expand substantially as a result of the California Privacy Rights Act of 2020 ("CPRA"), effective January 1, 2023. The CPRA establishes a new California Privacy Protection Agency to implement and enforce the CPRA, which could increase the risk of enforcement. Other states have enacted data privacy laws. For example, Virginia passed the Consumer Data Protection Act, Colorado passed the Colorado Privacy Act, and Utah passed the Consumer Privacy Act, all of which become effective in 2023. 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 United States, an increasing number of laws, regulations, and industry standards apply to data privacy and security. For example, the EU GDPR the UK GDPR, Brazil's General Data Protection Law (Lei Geral de Proteção de Dados Pessoais, or "LGPD") (Law No. 13,709/2018), and China's Personal Information Protection Law ("PIPL") impose strict requirements for processing personal data, and violators of these laws face significant penalties.

Among the most stringent of these laws is the GDPR. 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 the personal data. In addition, the GDPR expanded the definition of what is expressly noted to constitute personal data (including by broadening the relevant definition to capture expressly the "pseudonymized" or key-coded data that is commonly processed in a clinical trial-related context).

We may be subject to the GDPR 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, as well as in connection with any 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 has 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. In the UK, the UK Data Protection Act 2018 complements the UK GDPR in this regard. This fact may lead to greater divergence on the law that applies to the processing of such personal data across the EEA and/or UK, which may increase our costs and overall compliance risk.

Certain jurisdictions have enacted data localization laws and cross-border personal data transfer laws, which could make it more difficult to transfer information across jurisdictions (such as transferring or receiving personal data that is processed subject to the GDPR or other relevant regulation that originates in other foreign jurisdictions). Existing mechanisms that facilitate cross-border personal data transfers may change or be invalidated. For example, absent appropriate safeguards or other circumstances, the EU GDPR generally restricts the transfer of personal data to countries outside of the EEA that the European Commission does not consider to provide an adequate level of data privacy and security, such as the United States. Additionally, Switzerland and the UK similarly restrict personal data transfers outside of those jurisdictions to countries such as the United States that do not provide an adequate level of personal data protection. To comply with the EU GDPR's restrictions on transfer of personal data out of Europe to such jurisdictions, we typically rely on the Standard Contractual Clauses ("SCCs") for personal data transfers approved by the European Commission that are designed to be a mechanism to facilitate personal data transfers out of the EEA to these jurisdictions in compliance with the EU GDPR. Currently, these SCCs are a valid mechanism to transfer personal data outside of the EEA. The SCCs, as well as the UK-specific international data transfer tools mentioned below, impose additional compliance burdens, such as conducting transfer impact assessments to determine whether additional contractual, technical and/or organizational measures are necessary to protect the at-issue personal data. In addition to the European Union's SCCs, the UK government has recently issued certain separate and UK-specific international data transfer tools, which take the place of, or are needed to supplement, the European Union's SCCs, in relation to transfers made subject to the UK GDPR to recipients in countries outside of the UK that the UK government does not consider to provide an adequate level of data protection – implementing these new tools may require significant resources and result in significant cost to implement and manage. If we are unable to implement a valid solution for personal data transfers from Europe, we will face increased exposure to regulatory actions, substantial fines and injunctions against transferring personal data from Europe. Inability to export personal data from Europe may also: restrict our activities in Europe; limit our ability to collaborate with partners as well as other service providers, contractors and other companies in Europe; limit our ability to receive

personal data from our European subsidiaries, limit our subsidiaries' ability to transfer personal data outside of Europe, and generally limit our ability to operate the business of our subsidiaries as a seamlessly integrated part of our wider operations; and/or require us to increase our processing capabilities within Europe at significant expense or otherwise cause us to change the geographical location or segregation of our relevant systems and operations – any or all of which could adversely affect our operations or financial results. Other countries outside of Europe (e.g. Russia, China, Brazil) have enacted or are considering enacting similar cross-border data transfer restrictions and laws requiring local data residency, which could increase the cost and complexity of delivering our services and operating our business.

Failure to comply with the requirements of the EU GDPR and UK GDPR and the applicable national data protection laws of the EEA Member States/the UK may result in fines of up to €20,000,000 / £17,500,000 or up to 4% of the total worldwide annual turnover of the preceding financial year, whichever is higher. In addition to administrative fines, a wide variety of other potential enforcement powers are available to competent authorities in respect of potential and suspected violations of the EU GDPR and UK GDPR, including extensive audit and inspection rights, and powers to order temporary or permanent bans on all or some processing of personal data carried out by non-compliant actors. The EU GDPR also provides for private litigation related to the processing of personal data that can be brought by classes of data subjects or consumer protection organizations authorized at law to represent the data subjects' interests. Implementing mechanisms to endeavor to ensure compliance with the EU GDPR and UK GDPR and relevant local legislation in EEA Member States and the UK may be onerous and may interrupt or delay our development activities, and adversely affect our business, financial condition, results of operations, and prospects. In addition to the foregoing, an actual or perceived breach of the EU GDPR, UK GDPR or other applicable privacy and data protection laws and regulations could result in regulatory investigations, reputational damage, orders to cease/change our use of data, enforcement notices, or potential civil claims including class action-type litigation.

Additionally, these obligations may be subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions. The EU GDPR, the UK GDPR, the CCPA and many other laws and regulations relating to privacy and data protection are still being tested in courts, and they are subject to new and differing interpretations by courts and regulatory officials. 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. It is possible that the EU GDPR, the UK GDPR, the CCPA or other laws and regulations relating to privacy and data protection may be interpreted and applied in a manner that is inconsistent from jurisdiction to jurisdiction or inconsistent with our current policies and practices.

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. For example, any failure by a third-party processor to comply with applicable law, regulations, or contractual obligations could result in adverse effects, including inability to or interruption in our ability to operate our business and proceedings against us by governmental entities or others.

Our 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 regulatory fines and bans on processing personal information, investigations and enforcement actions, penalties and other liabilities, claims for damages by affected individuals, orders to destroy or not use personal information, imprisonment of company officials and damage to our reputation, any of which could materially affect our business, financial condition, results of operations and growth prospects.

The withdrawal of the UK from the EU, commonly referred to as "Brexit," may adversely impact our ability to obtain regulatory approvals of our product candidates in the UK, result in restrictions or imposition of taxes and duties for importing our product candidates into the EU or the UK, and may require us to incur additional expenses in order to develop, manufacture and commercialize our product candidates in the EU or the UK.

Following the result of a referendum in 2016, the UK left the EU on January 31, 2020, commonly referred to as Brexit. Pursuant to the formal withdrawal arrangements agreed between the UK and the EU, the UK was subject to a transition period until December 31, 2020 ("Transition Period"), during which EU rules continued to apply. A trade and cooperation agreement ("Trade and Cooperation Agreement") that outlines the future trading relationship between the UK and the EU applied provisionally from January 1, 2021 and formally entered into force on May 1, 2021.

Since a significant proportion of the regulatory framework in the UK applicable to our business and our product candidates is derived from EU directives and regulations, Brexit has had, and will continue to have, a material impact on the regulatory regime with respect to the development, manufacture, importation, approval and commercialization of our product candidates in the UK. For example, Great Britain (England, Scotland and Wales) is no longer covered by the centralized procedure for obtaining EU-wide marketing authorization from the EMA and a separate marketing authorization will be required to market our product candidates in the Great Britain. Any delay in obtaining, or an inability to obtain, any marketing approvals would delay or prevent us from commercializing our product candidates and restrict our ability to generate revenue and achieve and sustain profitability.

While the Trade and Cooperation Agreement provides for the tariff-free trade of medicinal products between the UK and the EU, there are additional non-tariff costs to such trade that did not exist prior to the end of the Transition Period. Further, should the UK diverge from the EU from a regulatory perspective in relation to medicinal products, tariffs could be put into place in the future. We could therefore, both now and in the future, face significant additional expenses to operate our business, which could significantly and materially harm or delay our ability to generate revenue or achieve profitability of our business. Any further changes in international trade, tariff and import/export regulations as a result of Brexit or otherwise may impose unexpected duty costs or other non-tariff barriers on us. These developments, or the perception that any of them could occur, may significantly reduce global trade and, in particular, trade between the EU and the UK.

Unfavorable U.S. and global economic conditions could adversely affect our business, financial condition or results of operations.

Our results of operations could be adversely affected by general conditions in the U.S. and global economies, the U.S. and global financial markets and adverse geopolitical and macroeconomic developments. U.S. and global market and economic conditions have been, and continue to be, disrupted and volatile due to many factors, including the ongoing COVID-19 pandemic, component shortages and related supply chain challenges, geopolitical developments such as the conflict between Ukraine and Russia and related sanctions, and increasing inflation rates and the responses by central banking authorities to control such inflation, among others. General business and economic conditions that could affect 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.

A severe or prolonged global economic downturn could result in a variety of risks to our business. For example, inflation rates, particularly in the United States, 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 addition, the U.S. Federal Reserve has raised, and may again raise, interest rates in response to concerns about inflation, 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 following the recent invasion of Ukraine by Russia. In response to the invasion, the United States, United Kingdom and 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, 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, including as a result of the COVID-19 pandemic or otherwise, we could experience adverse effects on our business, financial condition or results of operations.

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 and certain patents and know-how related to Livmarli and volixibat from Satiogen, which we subsequently acquired in May 2022. We have in-licensed certain patents and know-how related to volixibat from Shire and Sanofi. 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 and comparable foreign regulatory authorities for product candidates in clinical development. Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of these CROs fail to comply with applicable GCP regulations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. Upon inspection, such regulatory authorities may not determine that any of our clinical trials comply with the GCP regulations. In addition, our clinical trials must be conducted with drug product produced under cGMP regulations and will require a large number of test subjects. Our failure or any failure by our CROs to comply with these regulations or to recruit a sufficient number of patients may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, our business may be implicated if any of our CROs violates federal 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.

In addition, quarantines, shelter-in-place and similar government orders, or the perception that such orders, shutdowns or other restrictions on the conduct of business operations could occur, related to COVID-19 or other infectious diseases could, impact personnel at our CROs, which could disrupt our clinical timelines, which could have a material adverse impact on our business, prospects, financial condition and results of operations.

We rely completely on third parties to manufacture our preclinical, clinical and commercial drug supplies, and 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. Instead, we rely on contract manufacturers for such production.

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 used in our current product formats. 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. As 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 any product candidates that are approved. Further, any delay in identifying and qualifying a manufacturer for commercial production could delay the potential commercialization of our product candidates, and, in the event that we do not have sufficient product to complete our planned clinical trials, it could delay such trials. The facilities used by our contract manufacturers to manufacture our product candidates must be approved by the applicable regulatory authorities, including the FDA, pursuant to inspections that will be conducted after an NDA or comparable foreign regulatory marketing application is submitted. We do not control the manufacturing process of our product candidates and are completely dependent on our contract manufacturing partners for compliance with the FDA's cGMP requirements for manufacture of both the active drug substances and finished drug product. 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. 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 FDA or any other applicable regulatory authority does not approve these facilities for the manufacture of our product candidates or if it withdraws any such approval in the future, or if our suppliers or contract manufacturers decide they no longer want to supply or manufacture for us, 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.

In addition, the manufacture of pharmaceutical products is complex and requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. In addition, manufacturers and their facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to current good manufacturing practices, or cGMPs. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up and validating initial production and absence of contamination. These problems include difficulties with production costs and yields, quality control, including stability of the product, quality assurance testing, operator error, shortages of qualified personnel, as well as compliance with strictly enforced federal, state and foreign regulations. Furthermore, if contaminants are discovered in our supply of Livmarli or volixibat or in the manufacturing facilities, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. Any stability or other issues relating to the manufacture of our product candidates may occur in the future. In addition, quarantines, shelter-in-place and similar government orders, or the perception that such orders, shutdowns or other restrictions on the conduct of business operations could occur, related to COVID-19 or other infectious diseases could, impact personnel at our third-party manufacturing facilities upon which we rely, or the availability or cost of materials, which could disrupt the supply chain for our product candidates. Further, our manufacturers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes or unstable political environments. If our manufacturers were to encounter any of these difficulties, or otherwise fail to comply with their contractual obligations, our ability to provide our product candidate to patients in clinical trials would be jeopardized. Any delay or interruption in the supply of clinical trial supplies could delay the completion of 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.

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 cash, unrestricted 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 the COVID-19 pandemic and actions taken to slow its spread, as well as adverse geopolitical and macroeconomic developments, such as the ongoing 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, 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. 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. 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, such as the RIPA with the Purchasers, 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. For instance, as part of the RIPA, the Purchasers have the right to receive certain revenue interests from us based on the net sales of Livmarli, and we have granted the Purchasers a senior security interest in certain of our assets. 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.

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, 2021, we had federal and state net operating loss ("NOL") carryforwards of approximately $150.0 million and $6.7 million, respectively. The federal NOL carryforwards do not expire, and the state NOL carryforwards will begin to expire in 2039, unless previously utilized. Our ability to utilize our NOL carryforwards and certain other tax attributes may be limited. We also have federal and state research and development credit carryforwards totaling $23.1 million and $2.1 million, respectively. The federal research and development credit carryforwards will begin to expire in 2032, unless previously utilized. The state research and development credits will 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.

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 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 United States or in many jurisdictions outside of the United States. Changes in either the patent laws or interpretations of patent laws in the United States and other countries 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 United States 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 potential 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 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 United States and most other countries 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 United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns; and
- countries other than the United States 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 United States 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 countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we cannot ensure that any of our patents, or any of our pending patent applications, if issued, or those of our licensors, will include claims having a scope sufficient to protect our products;
- we cannot ensure that we will be able to successfully commercialize our products 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 invalidity proceedings before U.S. or non-U.S. patent offices.

We cannot be certain that the claims in our issued patents and pending patent applications covering Livmarli or volixibat will be considered patentable by the USPTO, courts in the United States, or by patent offices and courts in foreign countries. Furthermore, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. 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 volixibat in the United States or in foreign countries. 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 to our patents could deprive us of exclusive rights necessary for the successful commercialization of Livmarli or volixibat. Furthermore, even if they are unchallenged, our patents may not adequately protect our intellectual property, provide exclusivity for Livmarli or volixibat 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 volixibat is threatened, it could dissuade companies from collaborating with us to develop, or threaten our ability to commercialize, Livmarli or volixibat.

Further, if we encounter delays in our development efforts, including our clinical trials, the period of time during which we could market Livmarli or volixibat under patent protection would be reduced. In addition, patents have a limited lifespan. In the

United States, 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 United States 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, a 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 is developing 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. It remains unclear what impact the America Invents Act will have on the operation of our business. Moreover, 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, such as volixibat, 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 United States, Europe and other countries covering the methods of treating cholestatic liver diseases using IBATis, including Livmarli and volixibat. A patent based on any of these patent applications may never be issued. We do not have patents or patent applications covering Livmarli as a composition-of-matter. Therefore, the primary patent-based intellectual property protection for our Livmarli program will be any patents granted on the 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 United States 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 countries 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 United States and other governments may, at any time, enact changes to law and regulation 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 countries throughout the world would be prohibitively expensive. In addition, the laws of some foreign countries do not protect intellectual property rights in the same manner and to the same extent as laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States. 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 United States. These products may compete with our products 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 countries. For example, unlike other countries, China has a heightened requirement for patentability, and specifically requires a detailed description of medical uses of a claimed drug. In India, unlike the United States, there is no link between regulatory approval for a drug and its patent status. In addition to India, certain countries in Europe and developing countries, including China, have compulsory licensing laws under which a patent owner may be compelled to

grant licenses to third parties. In those countries, 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.

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

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us, either on reasonable terms, or at all. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment, or at all. If we are unable to successfully obtain rights to required third-party intellectual property rights on commercially reasonable terms, our ability to commercialize our products, 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 United States, 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 our products 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 United States, 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 United States may be maintained in secrecy until the patents are issued;
- patent applications in the United States 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 of our product candidates or the use thereof;
- identification of third-party patent rights that may be relevant to our technology is difficult because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims;
- patent applications are typically not published until 18 months after the priority date; and
- publications in the scientific literature often lag behind actual discoveries.

Furthermore, the scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history and can involve other factors such as expert opinion. Our interpretation of the relevance or the scope of claims in a patent or a pending application may be incorrect, which may negatively impact our ability to market our products. Further, we may incorrectly determine that our technologies, products, 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 United States 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 United States. 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 countries 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 countries where the laws may not protect those rights as fully as in the United States.

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 United States, 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 relevant 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.

Because of the expense and uncertainty of litigation, we may not be in a position to enforce our intellectual property rights against third parties.

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

We rely on trade secrets to protect our proprietary technologies, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect. We rely in part on confidentiality agreements with our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors, and inventions agreements with employees, consultants and advisors, to protect our trade secrets and other proprietary information. In addition to contractual measures, we try to protect the confidential nature of our proprietary information using commonly accepted physical and technological security measures. Despite these efforts, we cannot provide any assurances that all such agreements have been duly executed, and these agreements may not effectively prevent disclosure of confidential information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its Transparency Initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all. Costly and time-consuming litigation could be necessary to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

In addition, such security measures may not provide adequate protection for our proprietary information, for example, in the case of misappropriation of a trade secret by an employee, consultant, customer or third party with authorized access. Our security measures may not prevent an employee, consultant or customer from misappropriating our trade secrets and providing them to a competitor, and recourse we take against such misconduct may not provide an adequate remedy to protect our interests fully. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. Unauthorized parties may also attempt to copy or reverse engineer certain aspects of our products 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 United States 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 will over time be disseminated within the industry through independent development, the publication of journal articles and the movement of personnel skilled in the art from company to company or academic to industry scientific positions. Though our agreements with third parties typically restrict the ability of our advisors, employees, collaborators, licensors, suppliers, third-party contractors and consultants to publish data potentially relating to our trade secrets, our agreements may contain certain limited publication rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position. Because from time to time we expect to rely on third parties in the development, manufacture, and distribution of our products 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 United States 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, it may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA. Furthermore, in many countries, 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 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 to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks. Similar requirements exist in Europe.

Any collaboration arrangements that we have or may enter into in the future may not be successful, which could adversely affect our ability to develop and commercialize our products.

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

- collaborators have significant discretion in determining the efforts and resources that they will apply to collaborations;
- collaborators may conduct their own clinical trials which may not be compliant, may not be successful or may generate contradictory results;
- collaborators may not pursue development and commercialization of our products 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;
- disputes may arise between us and a collaborator that causes the delay or termination of the research, development or commercialization of our current or future products or that results in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated, and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable current or future products;
- collaborators may own or co-own intellectual property covering our products 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 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 its trading began on July 18, 2019 and to August 3, 2022 has ranged from a low of $6.84 to a high of $27.50. 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 Western Europe, if approved, 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;
- general political, health and economic conditions, including the COVID-19 pandemic, economic slowdowns, recessions, inflation, 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. Further, the RIPA limits our ability to declare dividends, including to whom they can be paid and the aggregate amount payable.

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.

Sales of a substantial number of shares of our common stock in the public market 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 reduce the market price of our common stock. As of June 30, 2022, there were 32,689,255 shares of our common stock outstanding, excluding 55,668 shares subject to repurchase, as described in the notes to our consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q.

In addition, shares of common stock that are either subject to outstanding options or reserved for future issuance under our employee benefit plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, Rule 144 and Rule 701 under the Securities Act of 1933, as amended ("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 payment obligations under the RIPA may adversely affect our financial position or results of operations and our ability to raise additional capital which in turn may increase our vulnerability to adverse regulatory developments or economic or business downturns.

As described in "Management's Discussion and Analysis of Financial Condition and Results of Operations—Liquidity and Capital Resources," on December 8, 2020, we entered into the RIPA. Pursuant to the RIPA, the Purchasers paid us $50.0 million on closing and $65.0 million in April 2021, less certain transaction expenses. We may also be 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. As consideration for such payments, the Purchasers will have a right to receive certain Revenue Interests from us based on annual net sales of Livmarli, which will be tiered payments (the "Revenue Interest Payments") based on whether such annual net sales 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 will initially be 9.75% (at Tier 1) and 2.00% (at Tier 2 and Tier 3) of such annual net sales in the Covered Territory; provided that (i) if the Purchasers have received Revenue Interest Payments in an amount equal to or greater than 110.0% of the total payments actually made by the Purchasers to us, exclusive of transaction expenses (the "Cumulative Purchaser Payments"), on or prior to December 31, 2026, the Revenue Interests shall be reduced to 2.00% at Tier 1 and 0.00% at Tier 3 for all subsequent calendar years beginning on January 1, 2027 and (ii) if the Purchasers have not received Revenue Interest Payments in an amount equal to or greater than 110.0% of the Cumulative Purchaser Payments on or prior to December 31, 2026, the Revenue Interests shall be increased for all subsequent calendar years beginning on January 1, 2027 to a single defined rate (with no separate tiers) that would have provided the Purchasers with an amount equal to 110.0% of the Cumulative Purchaser Payments on or prior to December 31, 2026 had such rate applied to Tier 1 of initial Revenue Interest Payments. The Purchasers' rights to receive the Revenue Interest Payments shall terminate on the date on which the Purchasers have received Revenue Interest Payments of 195.0% of the Cumulative Purchaser Payments, unless the RIPA is terminated earlier. The RIPA and the revenue interest stream payable to the Purchasers could have important negative consequences to the holders of our securities. For example, a portion of our cash flow from operations will be needed to pay certain revenue interests to the Purchasers and will not be available to fund future operations. Additionally, we may have increased vulnerability to adverse general economic and industry conditions. Payment requirements under the RIPA will increase our cash outflows. Our future operating performance is subject to market conditions and business factors that are beyond our control. If our cash inflows 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. If we raise funds by selling additional equity, such sale would result in dilution to our stockholders. If we are required to secure funding,

we may not be able to do so on terms acceptable to us, or at all. Failure to pay certain amounts to the Purchasers when due would result in a default under the RIPA and result in foreclosure on certain of our assets which would have a material adverse effect on our operations and financial condition. The RIPA contains customary affirmative and negative non-financial covenants and events of default, including covenants and restrictions that, among other things, grant a senior security interest in our assets and restrict our ability to incur liens, incur additional indebtedness, make loans and investments, engage in mergers and acquisitions, and engage in asset sales. Additionally, the Purchasers under the RIPA have an option (the "Put Option") to terminate the RIPA and to require us to repurchase future Revenue Interests upon enumerated events such as a bankruptcy event, an uncured material breach, a material adverse effect (which can include adverse developments related to the regulatory approval of our product candidates) or a change of control. The triggering of the Put Option, including by our failure to comply with these covenants, could permit the Purchasers to declare certain amounts to be immediately due and payable. Further, if we are liquidated, the Purchasers' right to repayment would be senior to the rights of the holders of our common stock. Any triggering of the Put Option or other declaration by the Purchasers of an event of default under the RIPA could significantly harm our financial condition, business and prospects and could cause the price of our common stock to decline.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to the Shelf Registration, Sales Agreement 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.

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 $46.3 million as of June 30, 2022. 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, 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.

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.

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 United States of America 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 United States of America 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, or 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). We may 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 email, and other functions. 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.

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 and online and offline fraud. These threats come from a variety of sources, including traditional computer "hackers," threat actors, personnel (such as through theft or misuse), sophisticated nation states, and nation-state-supported actors. Such incidents may also result from errors or malfeasance by our personnel or the personnel of the third parties with which we work, malware (including as a result of advanced persistent threat intrusions), viruses, software vulnerabilities, hacking, denial of service attacks (such as credential stuffing), social engineering (including 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. 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.

Threat actors, personnel (such as through theft or misuse), sophisticated nation-states, and nation-state-supported actors now 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. For example, we have operations and third parties upon which we rely to support our business located in unstable regions and regions experiencing (or expected to experience) geopolitical or other conflicts, including through the use of cyber-attacks.

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.

Any of the previously identified or similar threats could cause a security incident or other interruption. A security incident or other interruption could result in unauthorized, unlawful, or accidental acquisition, modification, destruction, loss, alteration, encryption, disclosure of, or access to our sensitive information.

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. There are many different and rapidly evolving cybercrime and hacking techniques, and we may be unable to anticipate attempted security breaches, identify them before our sensitive information is exploited, or react in a timely manner.

Although, to our knowledge, we have not experienced a material system failure or security incident to date, if such an event were to occur, it could result in a material disruption of our development programs and our business operations, whether due to a loss of sensitive information or other similar disruptions. 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. 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 could also have a material adverse effect on our business. 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. Such security incidents could also subject us to significant liability, harm our competitive position and delay the further commercialization and development of Livmarli and our product candidates.

If we (or a third party upon whom we rely) experience a security incident or 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. Additionally, 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.

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.

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 United States, to sell our products 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 United States 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 $1.07 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 United States of America 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.

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 other 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. 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 our products 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.

On May 20, 2022, we completed the merger and acquisition of Satiogen for total consideration of approximately $24.2 million, which consisted of, in part, (i) 609,305 shares of our common stock issued upon the closing of the acquisition as part of the upfront consideration, (ii) an additional 32,494 shares of our common stock that would have been issued upon the closing of the acquisition except the parties agreed to such shares being held back by us for 12 months from the acquisition date to satisfy certain purchase price adjustments and indemnification obligations that may arise during this period and (iii) an additional 199,993 shares of our common stock to be issued contingent upon the achievement of a certain milestone by June 30, 2025. We issued, or will issue as and when issuable in accordance with the definitive acquisition agreement, portions of the total consideration constituting shares of our common stock to the stockholders of Satiogen in existence immediately prior to the consummation of the acquisition (the "Satiogen Stockholders"), with such Satiogen Stockholders described in detail in the section entitled "Selling Stockholders" in our Registration Statement on Form S-3 filed on May 25, 2022 and declared effective by the SEC on June 9, 2022. As partial consideration for the right to receive our shares and the other acquisition consideration in accordance with the definitive acquisition agreement, the Satiogen Stockholders agreed to (i) the cessation of their respective shares of capital stock in Satiogen being issued and outstanding and (ii) the cancellation of their respective options to purchase shares of common stock of Satiogen. As a result of such agreements and the consummation of the acquisition, Satiogen became our wholly owned subsidiary. The acquisition consideration constituting shares of our common stock were issued, and, as and when issuable in accordance with the definitive acquisition agreement, will be issued, pursuant to the exemption provided in Section 4(a)(2) under the Securities Act and/or Regulation D promulgated thereunder as a transaction by an issuer not involving a public offering. We relied on the foregoing exemption from registration based in part on the representations made by each of the Satiogen Stockholders, including representations with respect to their respective status as accredited investors, as such term is defined in Rule 501(a) of the Securities Act, and their respective investment intent.

Except for limited circumstances set forth in the RIPA, the RIPA limits our ability to declare or pay dividends or make other distributions, directly or indirectly, without the consent of the Purchasers' agent.

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
3.1	Amended and Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on July 25, 2019).
3.2	Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed with the SEC on July 25, 2019).
4.1	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).
4.2	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).
31.1*	Certification of Principal Executive Officer pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Financial Officer pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1*#	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2*#	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS*	Inline XBRL Instance Document
101.SCH*	Inline XBRL Taxonomy Extension Schema 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.

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

SIGNATURES

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

Mirum Pharmaceuticals, Inc.

Date: August 4, 2022

By: /s/ Christopher Peetz
Christopher Peetz
President and Chief Executive Officer
(Principal Executive Officer)

Mirum Pharmaceuticals, Inc.

Date: August 4, 2022

By: /s/ Ian Clements, Ph.D.
Ian Clements, Ph.D.
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 31.1

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

I, Christopher Peetz, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: August 4, 2022

By: /s/ Christopher Peetz
Christopher Peetz
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

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

I, Ian Clements, Ph.D., 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 4, 2022

By: /s/ Ian Clements, Ph.D.
Ian Clements, Ph.D.
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

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

In connection with the Quarterly Report of Mirum Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the quarter ended June 30, 2022, 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 4, 2022

By: /s/ Christopher Peetz
Christopher Peetz
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

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

In connection with the Quarterly Report of Mirum Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the quarter ended June 30, 2022, 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 4, 2022

By: /s/ Ian Clements, Ph.D.
Ian Clements, Ph.D.
Chief Financial Officer
(Principal Financial and Accounting Officer)