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

FORM 10-Q

☑ **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: 001-36257

TRAVERE THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**27-4842691**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

3611 Valley Centre Drive, Suite 300

San Diego, CA 92130

(Address of Principal Executive Offices)

(888) 969-7879

(Registrant's Telephone number including area code)

N/A

Former name, former address and former fiscal year, if changed since last report

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.0001 per share	**TVTX**	**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 ☑

The number of shares of outstanding common stock, par value $0.0001 per share, of the Registrant as of August 2, 2022 was 63,957,899.

TRAVERE THERAPEUTICS, INC.

Form 10-Q
For the Fiscal Quarter Ended June 30, 2022

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FORWARD-LOOKING STATEMENTS

This report contains forward-looking statements regarding our business, financial condition, results of operations and prospects. Words such as “expects,” “anticipates,” “intends,” “plans,” “believes,” “seeks,” “estimates” and similar expressions or variations of such words are intended to identify forward-looking statements, but are not deemed to represent an all-inclusive means of identifying forward-looking statements as denoted in this report. Additionally, statements concerning future matters are forward-looking statements.

Although forward-looking statements in this report reflect the good faith judgment of our management, such statements can only be based on facts and factors currently known by us. Consequently, forward-looking statements are inherently subject to risks and uncertainties and actual results and outcomes may differ materially from the results and outcomes discussed in or anticipated by the forward-looking statements. Factors that could cause or contribute to such differences in results and outcomes include, without limitation, those specifically addressed under the headings “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in our Annual Report on Form 10-K for the fiscal year ended December 31, 2021, and in this Quarterly Report on Form 10-Q. You are urged not to place undue reliance on these forward-looking statements, which speak only as of the date of this report.

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 you are cautioned to not unduly rely upon these statements.

We file reports with the Securities and Exchange Commission ("SEC"). The SEC maintains a website (www.sec.gov) that contains reports, proxy and information statements, and other information regarding issuers that file electronically with the SEC, including us.

We undertake no obligation to revise or update any forward-looking statements in order to reflect any event or circumstance that may arise after the date of this report, except as required by law. Readers are urged to carefully review and consider the various disclosures made throughout the entirety of this quarterly report, which are designed to advise interested parties of the risks and factors that may affect our business, financial condition, results of operations and prospects.

Risk Factor Summary

Below is a summary of material factors that make an investment in our common stock speculative or risky. This summary does not address all of the risks that we face. Additional discussion of the risks summarized in this risk factor summary, and other risks that we face, can be found under the heading “Risk Factors” in Item 1A of Part II of this Quarterly Report on Form 10-Q and should be carefully considered, together with other information in this Quarterly Report on Form 10-Q and our other filings with the SEC before making investment decisions regarding our common stock.

- Our clinical trials may fail to demonstrate the safety and efficacy of our product candidates, including sparsentan and pegtibatinase (TVT-058), which could prevent or significantly delay their regulatory approval.
- Communications and/or feedback from the U.S. Food and Drug Administration (“FDA”) or European Medicines Agency (“EMA”) related to our current or planned future clinical trials does not guarantee any particular outcome from regulatory review for such clinical trials, and expedited regulatory review pathways may not actually lead to a faster development or regulatory review or approval process.
- Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful.
- An extended delay in the rate of enrollment or data collection in our ongoing Phase 1/2 Study of pegtibatinase (TVT-058), as a result of the COVID-19 pandemic or otherwise, may delay our timelines for analyzing future data from the study.
- Interim, topline and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.
- The commercial success of Chenodal, Cholbam and Thiola depends on them being considered to be effective drugs with advantages over other therapies.
- We are subject to generic competition, and recent developments relating to generic competition for pharmaceutical products could cause our product sales and business to be negatively impacted.
- Changes in reimbursement practices of third-party payers, or patients' access to insurance coverage, could affect the demand for our products and/or the prices at which they are sold.
- We are dependent on third parties to manufacture and distribute our pharmaceutical products who may not fulfill their obligations.
- If we are unable to maintain an effective and specialized sales force, we will not be able to commercialize our products in the United States successfully.
- Our products may not achieve or maintain expected levels of market acceptance or commercial success.
- If the market opportunities for our products and product candidates are smaller than we believe they are, our revenues may be adversely affected and our business may suffer.
- Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval or commercialization.
- We do not currently have patent protection for our commercial products. If we are unable to obtain and maintain protection for the intellectual property relating to our technology and products, the value of our technology and products will be adversely affected.
- We expect to rely on orphan drug status to develop and commercialize certain of our product candidates, but our orphan drug designations may not confer marketing exclusivity or other expected commercial benefits.

- Any drugs we develop may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, thereby harming our business.
- We face potential product liability exposure far in excess of our limited insurance coverage.
- We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do. Our operating results will suffer if we fail to compete effectively.
- The ongoing impacts of the COVID-19 pandemic could materially adversely affect our business, results of operations and financial condition.
- Our limited operating history makes it difficult to evaluate our future prospects, and our profitability in the future is uncertain.
- We depend on a highly experienced and skilled workforce to grow and operate our business. If we are unable to attract, retain and engage our employees, we may not be able to grow effectively.
- We will likely experience fluctuations in operating results and could incur substantial losses.
- Negative publicity regarding any of our products could impair our ability to market any such product and may require us to spend time and money to address these issues.
- We may need substantial funding and may be unable to raise capital when needed, which would force us to delay, reduce or eliminate our product development programs or commercialization efforts.
- The market price for shares of our common stock may be volatile and purchasers of our common stock could incur substantial losses.
- We may be unable to successfully integrate new products or businesses we may acquire.
- We may become involved in certain litigation matters, any of which could result in substantial costs, divert management's attention and otherwise have a material adverse effect on our business, operating results or financial condition.
- We are subject to significant ongoing regulatory obligations and oversight, which may result in significant additional expense and may limit our commercial success.
- We are subject to stringent and changing obligations related to data privacy and security. Our actual or perceived failure to comply with such obligations could lead to regulatory investigations or actions; litigation; fines and penalties; disruptions of our business; reputational harm; loss of revenue or profits; loss of customers or sales; and other adverse business consequences.
- If our information technology systems or data, or those of third parties upon which we rely, are or were compromised, we could experience adverse impacts resulting from such compromise, including, but not limited to, regulatory investigations or actions; litigation; fines and penalties; interruptions to our operations such as clinical trials; harm to our reputation; loss of revenue or profits; loss of sales and other adverse consequences.
- Our ability to use net operating loss carryforwards and certain other tax attributes to offset future taxable income and taxes may be subject to limitations.
- Business disruptions could seriously harm our future revenue and financial condition and increase our costs and expenses.
- Our indebtedness could adversely affect our financial condition.
- We may be unable to raise the funds necessary to repurchase the 2025 Notes and 2029 Notes for cash following a fundamental change, or to pay any cash amounts due upon conversion, and our future indebtedness may limit our ability to repurchase the 2025 Notes and 2029 Notes or pay cash upon their conversion.

PART I - FINANCIAL INFORMATION

Item 1. Financial Statements

TRAVERE THERAPEUTICS, INC. AND SUBSIDIARIES
CONDENSED CONSOLIDATED BALANCE SHEETS
(in thousands, except par value and share amounts)

	June 30, 2022	December 31, 2021
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 179,759	$ 165,753
Marketable debt securities, at fair value	373,414	387,129
Accounts receivable, net	16,689	15,914
Inventory, net	7,632	7,313
Prepaid expenses and other current assets	9,283	6,718
Total current assets	586,777	582,827
Property and equipment, net	10,080	11,106
Operating lease right of use assets	21,910	23,196
Intangible assets, net	149,920	148,435
Other assets	10,807	11,069
Total assets	$ 779,494	$ 776,633
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 11,848	$ 15,144
Accrued expenses	82,694	75,180
Deferred revenue, current portion	12,503	16,268
Business combination-related contingent consideration, current portion	7,300	7,400
Operating lease liabilities, current portion	4,123	3,908
Other current liabilities	6,024	6,188
Total current liabilities	124,492	124,088
Convertible debt	374,690	226,581
Deferred revenue, less current portion	16,235	20,379
Business combination-related contingent consideration, less current portion	68,400	59,700
Operating lease liabilities, less current portion	29,359	31,497
Other non-current liabilities	9,605	12,276
Total liabilities	622,781	474,521
Commitments and Contingencies (See Note 13)		
Stockholders' Equity:		
Preferred stock $0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of June 30, 2022 and December 31, 2021	—	—
Common stock $0.0001 par value; 200,000,000 shares authorized; 63,838,050, and 62,491,498 issued and outstanding as of June 30, 2022 and December 31, 2021, respectively	6	6
Additional paid-in capital	1,036,533	1,068,634
Accumulated deficit	(878,744)	(765,966)
Accumulated other comprehensive loss	(1,082)	(562)
Total stockholders' equity	156,713	302,112
Total liabilities and stockholders' equity	$ 779,494	$ 776,633

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

TRAVERE THERAPEUTICS, INC. AND SUBSIDIARIES
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except share and per share amounts)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Net product sales	$ 50,950	$ 54,617	$ 97,393	$ 102,024
License and collaboration revenue	3,217	—	5,261	—
Total revenue	54,167	54,617	102,654	102,024
Operating expenses:				
Cost of goods sold	2,051	1,651	4,189	3,296
Research and development	59,681	51,807	116,292	99,753
Selling, general and administrative	52,979	34,965	99,767	71,743
Change in fair value of contingent consideration	4,907	1,509	13,987	10,096
Total operating expenses	119,618	89,932	234,235	184,888
Operating loss	(65,451)	(35,315)	(131,581)	(82,864)
Other income (expenses), net:				
Interest income	782	988	1,060	1,397
Interest expense	(2,972)	(4,852)	(5,487)	(10,173)
Loss on early extinguishment of debt	—	—	(7,578)	—
Other income (expense), net	662	216	688	(877)
Total other expense, net	(1,528)	(3,648)	(11,317)	(9,653)
Loss before income tax provision	(66,979)	(38,963)	(142,898)	(92,517)
Income tax provision	(53)	(49)	(105)	(362)
Net loss	$ (67,032)	$ (39,012)	$ (143,003)	$ (92,879)
Basic and diluted net loss per common share	$ (1.05)	$ (0.64)	$ (2.26)	$ (1.59)
Basic and diluted weighted average common shares outstanding	63,638,385	60,571,259	63,387,009	58,431,770
Comprehensive loss:				
Net loss	$ (67,032)	$ (39,012)	$ (143,003)	$ (92,879)
Foreign currency translation gain (loss)	1,416	(227)	1,487	875
Unrealized loss on marketable debt securities	(803)	(152)	(2,007)	(614)
Comprehensive loss	$ (66,419)	$ (39,391)	$ (143,523)	$ (92,618)

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

TRAVERE THERAPEUTICS, INC. AND SUBSIDIARIES
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(unaudited, in thousands)

	For the Six Months Ended June 30,	
	2022	**2021**
Cash Flows From Operating Activities:		
Net loss	$ (143,003)	$ (92,879)
Adjustments to reconcile net loss to net cash used in operating activities:		
Share based compensation	20,823	15,204
Depreciation and amortization	15,200	12,694
Change in estimated fair value of contingent consideration	13,987	10,096
Payments from change in fair value of contingent consideration	(4,247)	(3,602)
Amortization of debt discount and issuance costs	766	5,522
Loss on allowance for inventory	1,741	962
Loss on early extinguishment of debt	7,578	—
Other	2,368	3,226
Changes in operating assets and liabilities:		
Accounts receivable	(777)	4,056
Tax receivable	(132)	17,011
Inventory	(2,060)	(764)
Prepaid expenses and other current and non-current assets	(2,204)	(1,224)
Change in lease assets and liabilities, net	(492)	5,492
Accounts payable	(2,917)	(632)
Accrued expenses	9,070	5,313
Deferred revenue, current and non-current	(5,700)	—
Other current and non-current liabilities	(2,127)	(453)
Net cash used in operating activities	(92,126)	(19,978)
Cash Flows From Investing Activities:		
Proceeds from the sale/maturity of marketable debt securities	217,325	242,064
Purchase of marketable debt securities	(206,529)	(406,000)
Purchase of fixed assets	(148)	(4,598)
Purchase of intangible assets	(16,579)	(8,979)
Net cash used in investing activities	(5,931)	(177,513)
Cash Flows From Financing Activities:		
Payment of guaranteed minimum royalty	(1,050)	(1,050)
Payment of acquisition-related contingent consideration	(1,271)	(1,399)
Proceeds from issuances of 2029 convertible senior notes	316,250	—
Payment of debt issuance costs	(9,882)	—
Repurchase of 2025 convertible senior notes including premium	(211,324)	—
Proceeds from exercise of stock options	947	3,074
Proceeds from issuances under the employee stock purchase plan	1,529	1,275
Proceeds from the issuance of common stock, net of issuance costs	—	189,278
Proceeds from the issuance of common stock in At-the-Market equity offering, net of issuance costs	19,545	4,878
Net cash provided by financing activities	114,744	196,056
Effect of exchange rate changes on cash	(2,681)	(49)
Net increase (decrease) in cash and cash equivalents	14,006	(1,484)
Cash and cash equivalents, beginning of year	165,753	84,772
Cash and cash equivalents, end of period	$ 179,759	$ 83,288

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

TRAVERE THERAPEUTICS, INC. AND SUBSIDIARIES

CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY

(unaudited, in thousands, except share amounts)

	Three Months Ended June 30, 2022						Three Months Ended June 30, 2021					
	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount					Shares	Amount				
Balance - March 31	**63,510,277**	**$ 6**	**$1,021,542**	**$ (1,695)**	**$ (811,712)**	**$ 208,141**	**60,435,730**	**$ 6**	**$1,002,687**	**$ (262)**	**$ (639,742)**	**$ 362,689**
Share based compensation	—	—	12,352	—	—	12,352	—	—	7,288	—	—	7,288
Issuance of common stock under the equity incentive plan and proceeds from exercise	250,598	—	824	—	—	824	176,259	—	319	—	—	319
Employee stock purchase program purchase and expense	77,175	—	1,815	—	—	1,815	98,887	—	1,496	—	—	1,496
Foreign currency translation adjustments	—	—	—	1,416	—	1,416	—	—	—	(227)	—	(227)
Unrealized loss on marketable debt securities	—	—	—	(803)	—	(803)	—	—	—	(152)	—	(152)
Other	—	—	—	—	—	—	—	—	(98)	—	—	(98)
Net loss	—	—	—	—	(67,032)	(67,032)	—	—	—	—	(39,012)	(39,012)
Balance - June 30	**63,838,050**	**$ 6**	**$1,036,533**	**$ (1,082)**	**$ (878,744)**	**$ 156,713**	**60,710,876**	**$ 6**	**$1,011,692**	**$ (641)**	**$ (678,754)**	**$ 332,303**

	Six Months Ended June 30, 2022						Six Months Ended June 30, 2021					
	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount					Shares	Amount				
Balance - December 31	**62,491,498**	**$ 6**	**$1,068,634**	**$ (562)**	**$ (765,966)**	**$ 302,112**	**52,248,431**	**$ 5**	**$ 797,985**	**$ (902)**	**$ (585,875)**	**$ 211,213**
Cumulative-effect adjustment from adoption of ASU 2020-06	—	—	(74,945)	—	30,225	(44,720)	—	—		—	—	
Share based compensation	—	—	20,287	—	—	20,287	—	—	14,767	—	—	14,767
Issuance of common stock under the equity incentive plan and proceeds from exercise	567,777	—	947	—	—	947	646,872	—	3,074	—	—	3,074
Employee stock purchase program purchase and expense	77,175	—	2,065	—	—	2,065	98,887		1,710	—	—	1,710
Equity offering, net of issuance costs	—	—	—	—	—	—	7,532,500	1	189,278	—	—	189,279
Issuance of common stock under At-The-Market offering, net of issuance costs of $0.6 million and $0.2 million	701,600	—	19,545	—	—	19,545	184,186	—	4,878	—	—	4,878
Foreign currency translation adjustments	—	—	—	1,487	—	1,487	—	—	—	875	—	875
Unrealized loss on debt securities	—	—	—	(2,007)	—	(2,007)	—	—	—	(614)	—	(614)
Net loss	—	—	—	—	(143,003)	(143,003)	—	—	—	—	(92,879)	(92,879)
Balance - June 30	**63,838,050**	**$ 6**	**$1,036,533**	**$ (1,082)**	**$ (878,744)**	**$ 156,713**	**60,710,876**	**$ 6**	**$1,011,692**	**$ (641)**	**$ (678,754)**	**$ 332,303**

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

TRAVERE THERAPEUTICS, INC. AND SUBSIDIARIES
NOTES TO THE UNAUDITED CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

NOTE 1. DESCRIPTION OF BUSINESS

Organization and Description of Business

Travere Therapeutics, Inc. (“we”, “our”, “us”, “Travere” and the “Company”) refers to Travere Therapeutics, Inc., a Delaware corporation, as well as our direct and indirect subsidiaries. Travere is a fully integrated biopharmaceutical company headquartered in San Diego, California focused on identifying, developing and delivering life-changing therapies to people with rare diseases. We regularly evaluate and, where appropriate, act on opportunities to expand our product pipeline through licenses and acquisitions of products in areas that will serve patients with serious or rare diseases and that we believe offer attractive growth characteristics.

The ongoing novel coronavirus (COVID-19) pandemic has resulted in travel restrictions, quarantines, “stay-at-home” and “shelter-in-place” orders and extended shutdown of certain businesses around the world. While the impact of the COVID-19 pandemic did not have a material adverse effect on our financial position or results of operations for the six months ended June 30, 2022, these governmental actions and similar actions that may be enacted in the future, and the widespread economic disruption arising from the pandemic, have the potential to materially impact our business and influence our business decisions. The extent and duration of the pandemic is unknown, and the future effects on our business are uncertain and difficult to predict. The Company is continuing to monitor the events and circumstances surrounding the COVID-19 pandemic, which may require adjustments to the Company’s estimates and assumptions in the future.

Clinical Programs:

Sparsentan is a novel investigational product candidate and has been granted Orphan Drug Designation for the treatment of focal segmental glomerulosclerois (FSGS) and immunoglobulin A nephropathy (IgAN) in the U.S. and Europe. Sparsentan is currently being evaluated in two pivotal Phase 3 clinical studies in rare kidney diseases.

Pegtibatinase (TVT-058) is a novel investigational human enzyme replacement candidate being evaluated for the treatment of classical homocystinuria (HCU). Pegtibatinase has been granted Rare Pediatric Disease, Fast Track and Breakthrough Therapy designations by the FDA, as well as orphan drug designation in the United States and European Union. Pegtibatinase is currently being evaluated in the Phase 1/2 COMPOSE Study to assess its safety, tolerability, pharmacokinetics, pharmacodynamics and clinical effects in patients with classical HCU. The Company acquired pegtibatinase as part of the November 2020 acquisition of Orphan Technologies Limited.

Chenodal (chenodeoxycholic acid or CDCA) is a naturally occurring bile acid that is approved for the treatment of people with radiolucent stones in the gallbladder. In January 2020, we randomized the first patients in our Phase 3 RESTORE Study to evaluate the effects of Chenodal in adult and pediatric patients with cerebrotendinous xanthomatosis (CTX), and the study enrollment remains open. The pivotal study is intended to support an NDA submission for marketing authorization of Chenodal for CTX in the United States.

Preclinical Programs:

We are a participant in two Cooperative Research and Development Agreements ("CRADAs"), which form a multi-stakeholder approach to pool resources with leading experts, and incorporate the patient perspective early in the therapeutic identification and development process. We have partnered with the National Institutes of Health’s National Center for Advancing Translational Sciences ("NCATS") and leading patient advocacy organizations, CDG Care and Alagille Syndrome Alliance, aimed at the identification of potential small molecule therapeutics for NGLY1 deficiency and Alagille syndrome ("ALGS"), respectively. There are no treatment options currently approved for these diseases.

Approved products:

- Chenodal (chenodiol tablets) is approved in the United States for the treatment of patients suffering from gallstones in whom surgery poses an unacceptable health risk due to disease or advanced age.
- Cholbam® (cholic acid capsules) is approved in the United States for the treatment of bile acid synthesis disorders due to single enzyme defects and is further indicated for adjunctive treatment of patients with peroxisomal disorders.
- Thiola® and Thiola EC® (tiopronin tablets) are approved in the United States for the prevention of cystine (kidney) stone formation in patients with severe homozygous cystinuria.

NOTE 2. BASIS OF PRESENTATION AND SIGNIFICANT ACCOUNTING POLICIES

The accompanying unaudited condensed consolidated financial statements should be read in conjunction with the audited consolidated financial statements and notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2021, filed with the SEC on February 24, 2022. The accompanying condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States (“GAAP”) for interim financial information, the instructions for Form 10-Q and the rules and regulations of the SEC. Accordingly, since they are interim statements, the accompanying condensed consolidated financial statements do not include all of the information and notes required by GAAP for annual financial statements, but reflect all adjustments consisting of normal, recurring adjustments, that are necessary for a fair statement of the financial position, results of operations and cash flows for the interim periods presented. Interim results are not necessarily indicative of the results that may be expected for any future periods. The December 31, 2021 balance sheet information was derived from the audited financial statements as of that date. Certain reclassifications have been made to the prior period consolidated financial statements to conform to the current period presentation.

A summary of the significant accounting policies applied in the preparation of the accompanying condensed consolidated financial statements follows:

Principles of Consolidation

The unaudited condensed consolidated financial statements represent the consolidation of the accounts of the Company and its subsidiaries in conformity with GAAP. All intercompany accounts and transactions have been eliminated in consolidation.

Revenue Recognition

The Company recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that an entity determines are within the scope of Accounting Standards Codification ("ASC") 606, Revenue from Contracts with Customers ("ASC 606"), the entity performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect substantially all the consideration it is entitled to in exchange for the goods or services it transfers to the customer. See Note 3 and Note 4 for further discussion.

Payments received under collaboration and licensing agreements may include non-refundable fees at the inception of the arrangements, milestone payments for specific achievements and royalties on the sale of products. At the inception of arrangements that include milestone payments, the Company uses judgement to evaluate whether the milestones are probable of being achieved and estimates the amount to include in the transaction price utilizing the most likely amount method. If it is probable that a significant revenue reversal will not occur, the estimated amount is included in the transaction price. Milestone payments that are not within the Company or the licensee’s control, such as regulatory approvals are not included in the transaction price until those approvals are received. At the end of each reporting period, the Company re-evaluates the probability of achievement of development milestones and any related constraint and adjusts the estimate of the overall transaction price, if necessary. The Company recognizes aggregate sales-based milestones and royalty payments from product sales at the later of when the related sales occur or when the performance obligation to which the sales-based milestone or royalty has been allocated has been satisfied. If it is probable that a significant revenue reversal will not occur, the Company estimates the sales-based milestone and royalty payments using the most likely amount method.

The Company utilizes significant judgement to develop estimates of the stand-alone selling price for each distinct performance obligation based upon the relative stand-alone selling price. Variable consideration that relates specifically to the Company’s efforts to satisfy specific performance obligations is allocated entirely to those performance obligations. The stand-alone selling price for license-related performance obligations requires judgement in developing assumptions to project probability-weighted cash flows based upon estimates of forecasted revenues, clinical and regulatory timelines and discount rates. The stand-alone selling price for clinical development performance obligations is based on forecasted expected costs of satisfying a performance obligation plus an appropriate margin.

If the licenses to intellectual property are determined to be distinct from the other performance obligations identified in the arrangement and have stand-alone functionality, the Company recognizes revenues from non-refundable, upfront fees allocated to the license when the license is transferred to the licensee and the licensee is able to benefit from the license. For licenses that are not distinct from other promises, the Company applies judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue from non-refundable, upfront fees. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the related revenue recognition accordingly.

The selection of the method to measure progress towards completion requires judgment and is based on the nature of the products or services to be provided. Revenue is recorded proportionally as costs are incurred. The Company generally utilizes the cost-to-cost method of progress because it best measures the transfer of control to the customer which occurs as the Company incurs costs. Under the cost-to-cost measure of progress, the extent of progress towards completion is measured based on the ratio of costs incurred to date to the total estimated costs at completion of the performance obligation. The Company uses judgment to estimate the total costs expected to complete the clinical development performance obligations, which include subcontractor costs, labor, materials, other direct costs and an allocation of indirect costs. The Company evaluates these cost estimates and the progress each reporting period and adjusts the measure of progress, if necessary.

Research and Development Expenses

Research and development includes expenses related to sparsentan, pegtibatinase, and the Company's other pipeline programs. The Company expenses all research and development costs as they are incurred. The Company's research and development costs are composed of salaries and bonuses, benefits, share-based compensation, license fees, milestones under license agreements, costs paid to third-party contractors to perform research, conduct clinical trials, and develop drug materials and delivery devices, and associated overhead expenses and facilities costs. The Company charges direct internal and

external program costs to the respective development programs. The Company also incurs indirect costs that are not allocated to specific programs because such costs benefit multiple development programs and allow us to increase our pharmaceutical development capabilities. These consist of internal shared resources related to the development and maintenance of systems and processes applicable to all of our programs.

Clinical Trial Expenses

The Company records expenses in connection with clinical trials under contracts with contract research organizations (CROs) that support conducting and managing clinical trials. The financial terms and activities of these agreements vary from contract to contract and may result in uneven expense levels. Generally, these agreements set forth activities that drive the recording of expenses such as start-up and initiation activities, enrollment and treatment of patients, or the completion of other clinical trial activities.

Expenses related to clinical trials are accrued based on our estimates and/or representations from service providers regarding work performed, including actual level of patient enrollment, completion of patient studies and progress of the clinical trials. Other incidental costs related to patient enrollment or treatment are accrued when reasonably certain. If the amounts we are obligated to pay under our clinical trial agreements are modified (for instance, as a result of changes in the clinical trial protocol or scope of work to be performed), the Company adjusts its accruals accordingly on a prospective basis. Revisions to the Company's contractual payment obligations are charged to expense in the period in which the facts that give rise to the revision become reasonably certain.

The Company currently has three Phase 3 clinical trials in process that are in varying stages of activity, with ongoing non-clinical support trials. As such, clinical trial expenses will vary depending on all the factors set forth above and may fluctuate significantly from quarter to quarter.

Intangible Assets with Cost Accumulation Model

In 2014, the Company entered into a license agreement with Mission Pharmacal in which the Company obtained the exclusive right to license the trademark of Thiola. The acquisition of the Thiola license qualified as an asset acquisition under the principles of ASC 805, Business Combinations ("ASC 805") in effect at the time of acquisition. The license agreement requires the Company to make royalty payments based on net sales of Thiola. The liability for royalties in excess of the annual contractual minimum is recognized in the period in which the royalties become probable and estimable, which is typically in the period corresponding with the respective sales. The Company records an offsetting increase to the cost basis of the asset under the cost accumulation model. The additional cost basis is subsequently amortized over the remaining life of the license agreement.

Consistent with all prior periods since Thiola was acquired, the Company has not accrued any liability for future royalties in excess of the annual contractual minimum at June 30, 2022 as such royalties are not yet probable and estimable.

Variable Interest Entity

The Company reviews each investment and collaboration agreement to determine if it has a variable interest in the entity. In assessing whether the Company has a variable interest in the entity as a whole, the Company considers and makes judgements regarding the purpose and design of the entity, the value of the licensed assets to the entity, the value of the entity’s total assets and the significant activities of the entity. If the Company has a variable interest in the entity as a whole, the Company assesses whether or not the Company is a primary beneficiary of that variable interest entity (“VIE”), based on a number of factors, including: (i) which party has the power to direct the activities that most significantly affect the VIE’s economic performance, (ii) the parties’ contractual rights and responsibilities pursuant to the collaboration agreement, and (iii) which party has the obligation to absorb losses of or the right to receive benefits from the VIE that could be significant to the VIE. If the Company determines that it is the primary beneficiary of a VIE at the onset of the collaboration, the collaboration is treated as a business combination and the Company consolidates the financial statements of the VIE into the Company’s consolidated financial statements. On a quarterly basis, the Company evaluates whether it continues to be the primary beneficiary of the consolidated VIE. If the Company determines that it is no longer the primary beneficiary of a consolidated VIE, it deconsolidates the VIE in the period in which the determination is made.

Assets and liabilities recorded as a result of consolidating the financial results of the VIE into the Company’s consolidated balance sheet do not represent additional assets that could be used to satisfy claims against the Company’s general assets or liabilities for which creditors have recourse to the Company’s general assets.

Adoption of New Accounting Standards

In August 2020, the FASB issued ASU No. 2020-06, Accounting for Convertible Instruments and Contracts in an Entity's Own Equity. The ASU includes amendments to the guidance on convertible instruments and the derivative scope exception for contracts in an entity's own equity in Subtopic 815-40 and simplifies the accounting for convertible instruments which include beneficial conversion features or cash conversion features by removing certain separation models in Subtopic 470-20. Additionally, the ASU will require entities to use the "if-converted" method when calculating diluted earnings per share for convertible instruments. The ASU is effective for fiscal years beginning after December 15, 2021, including interim periods within those fiscal years. The adoption of the new standard impacted the Company's accounting for its Convertible Senior Notes Due 2025 (2025 Notes), discussed in Note 10, which were previously accounted for using the cash conversion model applied under ASC 470-20, Debt with Conversion and Other Options ("ASC 470-20"). The Company adopted ASU 2020-06 on January 1, 2022 using the modified retrospective method. The cumulative effect of the accounting change as of January 1, 2022 increased the carrying amount of the 2025 Notes by $44.7 million, reduced additional paid-in capital by $74.9 million, and reduced accumulated deficit by $30.2 million.

NOTE 3. REVENUE RECOGNITION

Product Sales, Net

Product sales consist of Bile Acid products (Chenodal and Cholbam) and Tiopronin products (Thiola and Thiola EC). The Company sells its products through direct-to-patient distributors worldwide, with the United States and Canada representing 98% and 2% of net product sales, respectively, and rest of world representing less than 1% of net product sales, based on the product shipment destination.

Revenues from product sales are recognized when the customer obtains control of the Company's product, which occurs upon delivery to the customer. The Company receives payments from its product sales based on terms that generally are within 30 days of delivery of product to the patient.

Deductions from Revenue

Revenues from product sales are recorded at the net sales price, which includes provisions resulting from discounts, rebates and co-pay assistance that are offered to customers, health care providers, payers and other indirect customers relating to the Company's sales of its products. These provisions are based on the amounts earned or to be claimed on the related sales and are classified as a reduction of accounts receivable (if the amount is payable to a customer) or as a current liability (if the amount is payable to a party other than a customer). Where appropriate, these reserves take into consideration the Company's historical experience, current contractual and statutory requirements and specific known market events and trends. Overall, these reserves reflect the Company's best estimates of the amount of consideration to which it is entitled based on the terms of the contract. If actual results in the future vary from the Company's provisions, the Company will adjust the provision, which would affect net product revenue and earnings in the period such variances become known. Our historical experience is that such adjustments have been immaterial.

Government Rebates: We calculate the rebates that we will be obligated to provide to government programs and deduct these estimated amounts from our gross product sales at the time the revenues are recognized. Allowances for government rebates and discounts are established based on actual payer information, which is reasonably estimated at the time of delivery, and the government-mandated discounts applicable to government-funded programs. Rebate discounts are included in other current liabilities in the accompanying consolidated balance sheets.

Commercial Rebates: We calculate the rebates that we incur due to contracts with certain commercial payers and deduct these amounts from our gross product sales at the time the revenues are recognized. Allowances for commercial rebates are established based on actual payer information, which is reasonably estimated at the time of delivery. Rebate discounts are included in other current liabilities in the accompanying consolidated balance sheets.

Prompt Pay Discounts: We offer discounts to certain customers for prompt payments. We accrue for the calculated prompt pay discount based on the gross amount of each invoice for those customers at the time of sale.

Product Returns: Consistent with industry practice, we offer our customers a limited right to return product purchased directly from the Company, which is principally based upon the product's expiration date. Generally, shipments are only made upon a patient prescription thus returns are minimal.

Co-pay Assistance: We offer a co-pay assistance program, which is intended to provide financial assistance to qualified commercially insured patients with prescription drug co-payments required by payers. The calculation of the accrual for co-pay assistance is based on an identification of claims and the cost per claim associated with product that has been recognized as revenue.

The following table summarizes net product sales for the three and six months ended June 30, 2022 and 2021 (*in thousands*):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Bile acid products	$ 25,534	$ 24,974	$ 50,609	$ 46,938
Tiopronin products	25,416	29,643	46,784	55,086
Total net product sales	$ 50,950	$ 54,617	$ 97,393	$ 102,024

NOTE 4. COLLABORATION AND LICENSE AGREEMENTS

On September 15, 2021, the Company entered into a license and collaboration agreement ("License Agreement") with Vifor (International) Ltd. ("Vifor Pharma"), pursuant to which the Company granted an exclusive license to Vifor Pharma for the commercialization of sparsentan in Europe, Australia and New Zealand ("Licensed Territories"). Vifor Pharma also has first right of negotiation to expand the licensed territories into Canada, China, Brazil and/ or Mexico. Under the terms of the License Agreement, the Company received an upfront payment of $55.0 million and will be eligible for up to $135.0 million in aggregate regulatory and market access related milestone payments and up to $655.0 million in aggregate sales-based milestone payments for a total potential value of up to $845.0 million. The Company is also entitled to receive tiered double-digit royalties of up to 40 percent of annual net sales of sparsentan in the Licensed Territories.

Under the License Agreement, Vifor Pharma will be responsible for all commercialization activities in the Licensed Territories. The Company remains responsible for the worldwide clinical development of sparsentan through regulatory approval as defined and will retain all rights to sparsentan in the United States and rest of world outside of the Licensed Territories. Development costs for any post regulatory approval development activities, subject to approval by both parties, will be borne by the Company and Vifor Pharma as defined, respectively. The License Agreement will remain in effect, unless terminated earlier, until the expiration of all royalty terms for sparsentan in the licensed territories. Each party has the right to terminate the License Agreement for the other party's uncured material breach, insolvency or if the time required for performance under the License Agreement by the other party is extended due to a force majeure event that continues for six months or more.

The Company assessed the License Agreement and determined that it meets both criteria to be considered a collaborative agreement within the Scope of ASC 808, *Collaborative Arrangements* of active participation by both parties and exposures to significant risks and rewards dependent on the commercial success of the activities. Both parties participate on joint steering and other committees overseeing the collaboration activities. Also, both parties are exposed to significant risks and rewards based on the economic outcomes of regulatory approvals and commercialization of sparsentan.

The Company determined the transaction price under the License Agreement totaled $55.0 million, consisting of the fixed non-refundable upfront payment. The variable regulatory and access related milestones were excluded from the transaction price given the substantial uncertainty related to their achievement. Sales-based milestone payments and royalties on net sales were excluded from the transaction price and will be recognized at the later of when the related sales occur or when the performance obligation to which the sales-based milestone or royalty has been allocated have been satisfied.

The Company concluded that Vifor Pharma represented a customer and applied relevant guidance from ASC 606 to evaluate the accounting under the License Agreement. In accordance with this guidance, the Company concluded that the promise to grant the license is distinct from the promise to provide clinical development services resulting in two performance obligations. As a result, the Company allocated $12.0 million of the transaction price, based on the performance obligations' relative standalone selling prices, to the license, which was recognized in full in 2021. The remaining $43.0 million of the transaction price was allocated to the clinical development activities and recorded as deferred revenue, which will be recognized over the development period based upon the ratio of costs incurred to date to the total estimated costs. For the three and six month ended June 30, 2022, the Company recognized $3.2 million and $5.3 million, respectively, in license and collaboration revenue, based upon the ratio of costs incurred to total estimated costs.

Deferred revenue related to the clinical development activities as of June 30, 2022 was $28.7 million. Of this amount, $12.5 million was classified as current as of June 30, 2022, based upon amounts expected to be realized within the next year.

In February 2021, the Company entered into a limited co-promotion agreement with Albireo Pharma, Inc. ("Albireo"), whereby the Company's Cholbam dedicated sales representatives devoted a portion of their efforts to promoting Albireo's product, Bylvay (odevixibat), in the United States following the July 2021 launch of the product. The initial term of the arrangement was two years from the July 2021 launch of Bylvay, terminable at will by either party after one year following launch. In June 2022, the Company and Albireo mutually agreed to terminate the co-promotion agreement upon the one year anniversary of the launch, with such termination effective July 20, 2022. For the three and six months ended June 30, 2022, the Company recognized $0.8 million and $1.5 million, respectively, offset against selling, general, and administrative expenses. For the three and six months ended June 30, 2021, the Company recognized $0.5 million and $0.5 million, respectively, offset against selling, general, and administrative expenses.

NOTE 5. MARKETABLE DEBT SECURITIES

The Company's marketable debt securities as of June 30, 2022 and December 31, 2021 were composed of available-for-sale corporate and government debt securities. These securities are carried at fair value, with the unrealized gains and losses reported in accumulated other comprehensive income (loss), unless an impairment is determined to be the result of credit-related factors or the Company intends to sell the security or it is more likely than not that the Company will be required to sell the security before recovery. The amortized cost of marketable debt securities is adjusted for amortization of premiums and accretion of discounts to maturity. Such amortization and accretion is included in interest income. Realized gains and losses and declines in value that are determined to be the result of credit losses, if any, on available-for-sale securities are included in other income or expense. Unrealized losses that are determined to be credit-related are also recorded as an allowance against the amortized cost basis. The cost of securities sold is based on the specific identification method. Interest and dividends on securities classified as available-for-sale are included in interest income. All available-for-sale securities are classified as current assets, even if the maturity when acquired by the Company is greater than one year due to the ability to liquidate within the next 12 months.

During the six months ended June 30, 2022, investment activity for the Company included $217.3 million in maturities and $206.5 million in purchases, all relating to debt-based marketable securities.

Marketable debt securities consisted of the following (*in thousands*):

	June 30, 2022	December 31, 2021
Marketable debt securities:		
Commercial paper	$ 160,850	$ 127,379
Corporate debt securities	206,139	233,319
Securities of government sponsored entities	6,425	26,431
Total marketable debt securities	$ 373,414	$ 387,129

The following is a summary of short-term marketable debt securities classified as available-for-sale as of June 30, 2022 (*in thousands*):

	Remaining Contractual Maturity (in years)	Amortized Cost	Unrealized Gains	Unrealized Losses	Aggregate Estimated Fair Value
Marketable debt securities:					
Commercial paper	Less than 1	$ 161,361	$ —	$ (511)	$ 160,850
Corporate debt securities	Less than 1	163,995	—	(1,243)	162,752
Securities of government-sponsored entities	Less than 1	5,500	—	(81)	5,419
Total maturity less than 1 year		330,856	—	(1,835)	329,021
Corporate debt securities	1 to 2	43,997	—	(610)	43,387
Securities of government-sponsored entities	1 to 2	1,035	—	(29)	1,006
Total maturity 1 to 2 years		45,032	—	(639)	44,393
Total available-for-sale marketable debt securities		$ 375,888	$ —	$ (2,474)	$ 373,414

The following is a summary of short-term marketable debt securities classified as available-for-sale as of December 31, 2021 (*in thousands*):

	Remaining Contractual Maturity (in years)	Amortized Cost	Unrealized Gains	Unrealized Losses	Aggregate Estimated Fair Value
Marketable debt securities:					
Commercial paper	Less than 1	$ 127,435	$ —	$ (56)	$ 127,379
Corporate debt securities	Less than 1	113,001	—	(98)	112,903
Securities of government-sponsored entities	Less than 1	21,909	—	(5)	21,904
Total maturity less than 1 year		262,345	—	(159)	262,186
Corporate debt securities	1 to 2	120,705	—	(289)	120,416
Securities of government-sponsored entities	1 to 2	4,549	—	(22)	4,527
Total maturity 1 to 2 years		125,254	—	(311)	124,943
Total available-for-sale securities		$ 387,599	$ —	$ (470)	$ 387,129

The primary objective of the Company's investment portfolio is to preserve capital and liquidity while enhancing overall returns. The Company's investment policy limits interest-bearing security investments to certain types of instruments issued by institutions with primarily investment grade credit ratings and places restrictions on maturities and concentration by asset class and issuer.

The Company reviews the available-for-sale marketable debt securities for declines in fair value below the cost basis each quarter. For any security whose fair value is below its amortized cost basis, the Company first evaluates whether it intends to sell the impaired security, or will otherwise be more likely than not required to sell the security before recovery. If either are true, the amortized cost basis of the security is written down to its fair value at the reporting date. If neither circumstance holds true, the Company assesses whether any portion of the unrealized loss is a result of a credit loss. Any amount deemed to be attributable to credit loss is recognized in the income statement, with the amount of the loss limited to the difference between fair value and amortized cost and recorded as an allowance for credit losses. The portion of the unrealized loss related to factors other than credit losses is recognized in other comprehensive income (loss).

The following is a summary of available-for-sale marketable debt securities in an unrealized loss position with no credit losses reported as of June 30, 2022 (*in thousands*):

	Less Than 12 Months		12 Months or Greater		Total	
Description of Securities	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
Commercial paper	$ 160,850	$ 511	$ —	$ —	$ 160,850	$ 511
Corporate debt securities	177,471	1,601	28,668	252	206,139	1,853
Securities of government-sponsored entities	5,445	90	980	20	6,425	110
Total	$ 343,766	$ 2,202	$ 29,648	$ 272	$ 373,414	$ 2,474

The following is a summary of available-for-sale marketable debt securities in an unrealized loss position with no credit losses reported as of December 31, 2021 (*in thousands*):

Description of Securities	Less Than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
Commercial paper	$ 122,380	$ 56	$ —	$ —	$ 122,380	$ 56
Corporate debt securities	231,879	387	—	—	231,879	387
Securities of government-sponsored entities	26,431	27	—	—	26,431	27
Total	$ 380,690	$ 470	$ —	$ —	$ 380,690	$ 470

As of June 30, 2022 and December 31, 2021, the amortized cost of the available-for-sale marketable debt securities in an unrealized loss position was $375.9 million and $381.2 million, respectively.

As of June 30, 2022 and December 31, 2021, the Company does not intend to sell these investments and it is not more likely than not that the Company will be required to sell the investments before recovery of their amortized cost basis. The increase in unrealized losses for the six months ending June 30, 2022 was primarily due to increases in short-term interest rates. The Company does not believe the unrealized losses incurred during the period are due to credit-related factors. The credit ratings of the securities held remain of the highest quality. Moreover, the Company continues to receive payments of interest and principal as they become due, and our expectation is that those payments will continue to be received timely. Uncertainty surrounding the COVID-19 pandemic, as well as other factors unknown to us at this time, may cause actual results to differ and require adjustments to the Company's estimates and assumptions in the future.

NOTE 6. VARIABLE INTEREST ENTITIES

On March 8, 2022, the Company entered into a Collaboration Agreement with PharmaKrysto Limited ("PharmaKrysto"), a privately held pre-clinical stage company related to PharmaKrysto's early-stage cystinuria discovery program, and concurrently therewith entered into a Stock Purchase Agreement with PharmaKrysto (together, the "Agreements"). Pursuant to the terms of the Agreements, the Company paid PharmaKrysto's shareholders $0.6 million in cash to purchase 5% of the outstanding common shares of PharmaKrysto and $0.4 million to PharmaKrysto as a one-time signing fee. Under the Collaboration Agreement, the Company will fund all research and development expenses for the pre-clinical activities associated with the cystinuria program, which are estimated to be approximately $5.0 million. The Agreements require the Company to purchase an additional 5% of the outstanding common shares for $1.0 million upon the occurrence of a specified pre-clinical milestone, and grant an option to the Company to purchase the remaining outstanding shares of PharmaKrysto for $5.0 million upon the occurrence of a subsequent pre-clinical milestone prior to expiration of the option on March 8, 2025. If the Company elects to exercise the option, it would be required to perform commercially reasonable clinical diligence obligations. In addition, it would be required to make cash milestone payments totaling up to an aggregate $16.0 million upon the achievement of certain development and regulatory milestones, plus tiered royalty payments of less than 4% on future net sales of a product, if approved. The Company has the right to terminate the Agreements and return the shares for a nominal price at any time upon 60 days' notice, subject to survival of contingent obligations, if any.

The Company determined that PharmaKrysto is a VIE because it lacks the resources to conduct the cystinuria clinical program and the limitation on the residual returns through the Company's option to purchase the remaining outstanding shares. The Company further concluded that it is the primary beneficiary of the VIE due to the Company's ultimate control over the research and development program, and its obligation, subject to continuation of the collaboration, to fund 100% of research and development costs of the program pursuant to the terms of the Collaboration Agreement.

The upfront payments were expensed to research and development and other income (expense), net upon initial consolidation. The Company consolidated other current assets and accrued liabilities of $0.3 million as of June 30, 2022. The results of operations were not significant for the three and six months ended June 30, 2022. The Company is not required to provide additional funding other than the contractually required amounts disclosed above. The creditors and beneficial holders of PharmaKrysto have no recourse to the general credit of the Company.

NOTE 7. LEASES

As of June 30, 2022, the Company had one operating lease with Kilroy Realty, L.P. (the "Landlord") for office space located in San Diego, California, which was entered into in April 2019 and subsequently amended in May 2020. Coinciding with our ability to direct the use of the office space, which occurred in phases over 2020, and utilizing a discount rate equal to our borrowing rate, the Company established ROU assets totaling $34.6 million and lease liabilities totaling $34.5 million. The total ROU asset and lease liability at measurement were each offset by lease incentives associated with tenant improvement allowances totaling $7.9 million.

The initial term of the office lease ends in August 2028, and the Landlord has granted the Company an option to extend the term of the lease by a period of 5 years. At this time, it is not reasonably certain that we will extend the term of the lease and therefore the renewal period has been excluded from the aforementioned ROU asset and lease liability measurements. The measurement of the lease term occurs from the February 2021 occupancy date of the office space delivered in September 2020. The aggregate base rent due over the initial term of the lease is approximately $49.5 million.

Following is a schedule of the future minimum rental commitments for our operating leases reconciled to the lease liability and ROU asset as of June 30, 2022 (*in thousands*):

	June 30, 2022
2022	$ 3,019
2023	6,200
2024	6,386
2025	6,578
2026	6,775
Thereafter	11,760
Total undiscounted future minimum payments	40,718
Present value discount	(7,236)
Total lease liability	33,482
Unamortized lease incentives	(6,067)
Cash payments in excess of straight-line lease expense	(5,505)
Total ROU asset	$ 21,910

For the three and six months ended June 30, 2022, the Company recorded $1.3 million and $2.5 million, respectively, in expense related to operating leases, including amortized tenant improvement allowances. For the three and six months ended June 30, 2021, the Company recorded $1.2 million and $2.4 million, respectively, in expense related to operating leases, including amortized tenant improvement allowances.

NOTE 8. FAIR VALUE MEASUREMENTS

Financial Instruments and Fair Value

The Company accounts for financial instruments in accordance with ASC 820, *Fair Value Measurements and Disclosures* (“ASC 820”). ASC 820 establishes a fair value hierarchy that prioritizes the inputs to valuation techniques used to measure fair value. The hierarchy gives the highest priority to unadjusted quoted prices in active markets for identical assets or liabilities (Level 1 measurements) and the lowest priority to unobservable inputs (Level 3 measurements). The three levels of the fair value hierarchy under ASC 820 are described below:

Level 1 – Unadjusted quoted prices in active markets that are accessible at the measurement date for identical, unrestricted assets or liabilities;

Level 2 – Quoted prices in markets that are not active or financial instruments for which all significant inputs are observable, either directly or indirectly; and

Level 3 – Prices or valuations that require inputs that are both significant to the fair value measurement and unobservable.

The valuation techniques used to measure the fair value of the Company’s debt securities and all other financial instruments, all of which have counter-parties with high credit ratings, were valued based on quoted market prices or model driven valuations using significant inputs derived from or corroborated by observable market data. Based on the fair value hierarchy, the Company classified debt securities within Level 2.

The Company acquired two businesses, related to the Cholbam and Chenodal products, whose purchase price included potential future payments that are contingent on the achievement of certain milestones and percentages of future net sales derived from the products acquired. The Company recorded contingent consideration liabilities at their fair value on the acquisition date and revalues them at the end of each reporting period. In estimating the fair value of the Company’s contingent consideration, the Company uses a Monte Carlo Simulation. The determination of the contingent consideration liabilities requires significant judgements including the appropriateness of the valuation model and reasonableness of estimates and assumptions included in the forecasts of future net sales and the discount rates applied to such forecasts. Changes in these estimates and assumptions could have a significant impact on the fair value of the contingent consideration liabilities.

Discount rates used to determine the fair value at June 30, 2022 and December 31, 2021 are as follows:

	Revenue Discount		Payment Discount
	Cholbam	Chenodal	
June 30, 2022	6.50%	7.00%	8.14%
December 31, 2021	6.25%	7.25%	6.48%

Based on the fair value hierarchy, the Company classified the fair value measurement of contingent consideration within Level 3 because valuation inputs are based on projected revenues discounted to a present value.

Financial instruments with carrying values approximating fair value include cash and cash equivalents, accounts receivable, and accounts payable, due to their short-term nature. As of June 30, 2022, the fair value of the Company's 2.5% Convertible Senior Notes due 2025 was $65.5 million and the fair value of the Company's 2.25% Convertible Senior Notes due 2029 was $315.5 million, which were estimated utilizing market quotations, and are considered Level 2.

The following table presents the Company's assets and liabilities, measured and recognized at fair value on a recurring basis, classified under the appropriate level of the fair value hierarchy as of June 30, 2022 (*in thousands*):

	As of June 30, 2022			
	Total carrying and estimated fair value	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Assets:				
Cash and Cash Equivalents	$ 179,759	$ 179,759	$ —	$ —
Debt securities, available-for-sale	373,414	—	373,414	—
Total	$ 553,173	$ 179,759	$ 373,414	$ —
Liabilities:				
Business combination-related contingent consideration	$ 75,700	$ —	$ —	$ 75,700
Total	$ 75,700	$ —	$ —	$ 75,700

The following table presents the Company's assets and liabilities, measured and recognized at fair value on a recurring basis, classified under the appropriate level of the fair value hierarchy as of December 31, 2021 (*in thousands*):

	As of December 31, 2021			
	Total carrying and estimated fair value	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Assets:				
Cash and Cash Equivalents	$ 165,753	$ 165,753	$ —	$ —
Debt securities, available-for-sale	387,129	—	387,129	—
Total	$ 552,882	$ 165,753	$ 387,129	$ —
Liabilities:				
Business combination-related contingent consideration	$ 67,100	$ —	$ —	$ 67,100
Total	$ 67,100	$ —	$ —	$ 67,100

The following table sets forth a summary of changes in the estimated fair value of the Company's Level 3 business combination-related contingent consideration for the six months ended June 30, 2022 (*in thousands*):

	Fair Value Measurements of Acquisition-Related Contingent Consideration (Level 3)
Balance at January 1, 2022	$ 67,100
Changes in the fair value of contingent consideration	13,987
Contractual payments	(2,685)
Contractual payments included in accrued liabilities at June 30, 2022	(2,702)
Balance at June 30, 2022	$ 75,700

For the three and six months ended June 30, 2022, the Company incurred charges of $4.9 million and $14.0 million, respectively, in operating expenses on the Condensed Consolidated Statements of Operations and Comprehensive Loss for the change in fair value of the contingent consideration liabilities. In both periods, the value changed due to the timing of future payments and changes in market driven discount rates.

For the three and six months ended June 30, 2021, the Company incurred charges of $1.5 million and $10.1 million, respectively, in operating expenses on the Condensed Consolidated Statements of Operations and Comprehensive Loss for the change in fair value of the contingent consideration liabilities. In both periods, the value changed due to the timing of future payments and changes in market driven discount rates.

NOTE 9. INTANGIBLE ASSETS

Ligand License Agreement

In 2012, the Company entered into an agreement with Ligand Pharmaceuticals, Inc. ("Ligand") for a worldwide sublicense to develop, manufacture and commercialize a drug technology compound including sparsentan (the “Ligand License Agreement”). The cost of the Ligand License Agreement, which is presented net of amortization in the accompanying Consolidated Balance Sheets in intangible assets, net, is being amortized to research and development on a straight-line basis through September 30, 2023. As consideration for the license, the Company is required to make substantial payments upon the achievement of certain milestones, totaling up to $114.1 million. Through June 30, 2022, the Company has capitalized $15.0 million for contractual milestone payments under the Ligand License Agreement. Should the Company commercialize sparsentan or any products containing related compounds, the Company will be obligated to pay to Ligand an escalating annual royalty between 15% and 17% of net sales of all such products.

As of June 30, 2022, the net book value of amortizable intangible assets was approximately $149.0 million.

The following table sets forth amortizable intangible assets as of June 30, 2022 and December 31, 2021 (*in thousands*):

	June 30, 2022	December 31, 2021
Finite-lived intangible assets	$ 299,345	$ 283,557
Less: accumulated amortization	(150,361)	(136,058)
Net carrying value	$ 148,984	$ 147,499

As of June 30, 2022 and December 31, 2021, the Company had goodwill of $0.9 million.

The following table summarizes amortization expense for the three and six months ended June 30, 2022 and 2021 (*in thousands*):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Research and development	$ 1,625	$ 288	$ 1,911	$ 574
Selling, general and administrative	5,946	5,848	12,216	11,514
Total amortization expense	$ 7,571	$ 6,136	$ 14,127	$ 12,088

NOTE 10. CONVERTIBLE NOTES PAYABLE

The composition of the Company’s convertible senior notes are as follows (*in thousands*):

	June 30, 2022	December 31, 2021
2.25% convertible senior notes due 2029	$ 316,250	$ —
2.50% convertible senior notes due 2025	68,904	276,000
Unamortized debt discount	—	(46,045)
Unamortized debt issuance costs - 2.25% convertible senior notes due 2029	(9,448)	—
Unamortized debt issuance costs - 2.50% convertible senior notes due 2025	(1,016)	(3,374)
Total convertible senior notes, net of unamortized debt discount and debt issuance costs	$ 374,690	$ 226,581

Convertible Senior Notes Due 2029

On March 11, 2022, the Company completed a registered underwritten public offering of $316.3 million aggregate principal amount of 2.25% Convertible Senior Notes due 2029 (“2029 Notes”), which includes $41.3 million aggregate principal amount of 2029 Notes sold pursuant to the full exercise of the underwriters’ option to purchase additional 2029 Notes. The Company issued the 2029 Notes under an indenture, dated as of September 10, 2018, as supplemented by the second supplemental indenture, dated as of March 11, 2022 (collectively, the “2029 Indenture”). The 2029 Notes will mature on March 1, 2029, unless earlier repurchased, redeemed, or converted. The 2029 Notes are senior unsecured obligations of the Company and bear interest at an annual rate of 2.25%, payable semi-annually in arrears on March 1 and September 1 of each year, beginning on September 1, 2022.

The Company received net proceeds from the issuance of the 2029 Notes of $306.4 million, after deducting commissions and offering expenses of $9.9 million. At June 30, 2022, accrued interest on the 2029 Notes of $2.2 million is included in accrued expenses in the accompanying Condensed Consolidated Balance Sheets. The 2029 Notes comprise the Company’s senior, unsecured obligations and are (i) equal in right of payment with the Company’s existing and future senior, unsecured indebtedness; (ii) senior in right of payment to the Company’s existing and future indebtedness that is expressly subordinated to the 2029 Notes; (iii) effectively subordinated to the Company’s existing and future secured indebtedness, to the extent of the value of the collateral securing that indebtedness; and (iv) structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables.

Holders may convert their 2029 Notes at their option only in the following circumstances: (1) during any calendar quarter commencing after the calendar quarter ending on June 30, 2022 (and only during such calendar quarter), if the last reported sale price per share of the Company’s common stock for each

of at least 20 trading days, whether or not consecutive, during the period of 30 consecutive trading days ending on, and including, the last trading day of the immediately preceding calendar quarter exceeds 130% of the conversion price on the applicable trading day; (2) during the five consecutive business days immediately after any 10 consecutive trading day period (such 10 consecutive trading day period, the “measurement period”) if the trading price per $1,000 principal amount of 2029 Notes for each trading day of the measurement period was less than 98% of the product of the last reported sale price per share of the Company’s common stock on such trading day and the conversion rate on such trading day; (3) upon the occurrence of certain corporate events or distributions of the Company’s common stock; (4) if the Company calls the 2029 Notes for redemption; and (5) at any time from, and including, December 1, 2028 until the close of business on the scheduled trading day immediately before the maturity date. The Company will settle conversions by paying or delivering, as applicable, cash, shares of the Company’s common stock, or a combination of cash and shares of the Company’s common stock, at the Company’s election, based on the applicable conversion rate. The initial conversion rate for the 2029 Notes is 31.3740 shares of the Company’s common stock per $1,000 principal amount of 2029 Notes, which represents an initial conversion price of approximately $31.87 per share. If a “make-whole fundamental change” (as defined in the 2029 Indenture) occurs, then the Company will, in certain circumstances, increase the conversion rate for a specified period of time.

The 2029 Notes will be redeemable, in whole or in part at the Company’s option at any time, and from time to time, on or after March 2, 2026 and, in the case of any partial redemption, on or before the 40th scheduled trading day before the maturity date, at a cash redemption price equal to the principal amount of the 2029 Notes to be redeemed, plus accrued and unpaid interest, if any, to, but excluding, the redemption date but only if the last reported sale price per share of the Company’s common stock exceeds 130% of the conversion price on (1) each of at least 20 trading days, whether or not consecutive, during the 30 consecutive trading days ending on, and including, the trading day immediately before the date the Company sends the related redemption notice; and (2) the trading day immediately before the date the Company sends such notice. However, the Company may not redeem less than all of the outstanding 2029 Notes unless at least $100.0 million aggregate principal amount of 2029 Notes are outstanding and not called for redemption as of the time the Company sends the related redemption notice. In addition, calling any 2029 Note for redemption will constitute a make-whole fundamental change with respect to that 2029 Note, in which case the conversion rate applicable to the conversion of that 2029 Note will be increased in certain circumstances if it is converted after it is called for redemption. If a fundamental change (as defined in the 2029 Indentures) occurs, then, except as described in the 2029 Indentures, holders may require the Company to repurchase their 2029 Notes at a cash repurchase price equal to the principal amount of the 2029 Notes to be repurchased, plus accrued and unpaid interest, if any, to, but excluding, the fundamental change repurchase date. In the event of conversion, holders would forgo all future interest payments, any unpaid accrued interest and the possibility of further stock price appreciation. Upon the receipt of conversion requests, the settlement of the 2029 Notes will be paid pursuant to the terms of the 2029 Indenture. In the event that all of the 2029 Notes are converted, the Company would be required to repay the principal amount and any conversion premium in any combination of cash and shares of its common stock at the Company’s option. In addition, calling the 2029 Notes for redemption will constitute a “make-whole fundamental change."

The Company incurred approximately $9.9 million of debt issuance costs relating to the issuance of the 2029 Notes, which were recorded as a reduction to the 2029 Notes on the Condensed Consolidated Balance Sheets. The debt issuance costs are being amortized and recognized as additional interest expense over the expected life of the 2029 Notes using the effective interest method. We determined the expected life of the debt is equal to the seven-year term of the 2029 Notes. The effective interest rate on the 2029 Notes is 2.74%.

Convertible Senior Notes Due 2025

On September 10, 2018, the Company completed a registered underwritten public offering of $276.0 million aggregate principal amount of 2.50% Convertible Senior Notes due 2025 ("2025 Notes"), and entered into a base indenture and supplemental indenture agreement ("2025 Indenture") with respect to the 2025 Notes. The 2025 Notes will mature on September 15, 2025, unless earlier repurchased, redeemed, or converted. The 2025 Notes are senior unsecured obligations of the Company and bear interest at an annual rate of 2.50%, payable semi-annually in arrears on March 15 and September 15 of each year, beginning on March 15, 2019.

The net proceeds from the issuance of the 2025 Notes were approximately $267.2 million, after deducting commissions and the offering expenses of $8.8 million payable by the Company. At June 30, 2022, accrued interest of $0.5 million is included in accrued expenses in the accompanying Condensed Consolidated Balance Sheets. The 2025 Notes comprise the Company’s senior, unsecured obligations and are (i) equal in right of payment with the Company’s existing and future senior, unsecured indebtedness; (ii) senior in right of payment to the Company’s existing and future indebtedness that is expressly subordinated to the 2025 Notes; (iii) effectively subordinated to the Company’s existing and future secured indebtedness, to the extent of the value of the collateral securing that indebtedness; and (iv) structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables.

Holders may convert their 2025 Notes at their option only in the following circumstances: (1) during any calendar quarter commencing after the calendar quarter ending on December 31, 2018 (and only during such calendar quarter), if the last reported sale price per share of the Company’s common stock for each of at least 20 trading days, whether or not consecutive, during the period of 30 consecutive trading days ending on, and including, the last trading day of the immediately preceding calendar quarter exceeds 130% of the conversion price on the applicable trading day; (2) during the five consecutive business days immediately after any 10 consecutive trading day period (“measurement period”) if the trading price per $1,000 principal amount of 2025 Notes for each trading day of the measurement period was less than 98% of the product of the last reported sale price per share of the Company’s common stock on such trading day and the conversion rate on such trading day; (3) upon the occurrence of certain corporate events or distributions on the Company’s common stock; (4) if the Company calls the 2025 Notes for redemption; and (5) at any time from, and including, May 15, 2025 until the close of business on the scheduled trading day immediately before the maturity date. The Company will settle conversions by paying or delivering, as applicable, cash, shares of the Company’s common stock, or a combination of cash and shares of the Company’s common stock, at the Company’s election, based on the applicable conversion rate.

The initial conversion rate for the 2025 Notes is 25.7739 shares of the Company’s common stock per $1,000 principal amount of 2025 Notes, which represents an initial conversion price of approximately $38.80 per share. If a “make-whole fundamental change” (as defined in the 2025 Indenture) occurs, then the Company will, in certain circumstances, increase the conversion rate for a specified period of time.

The 2025 Notes will be redeemable, in whole or in part, at the Company’s option at any time, and from time to time, on or after September 15, 2022 and, in the case of any partial redemption, on or before the 40th scheduled trading day before the maturity date, at a cash redemption price equal to the principal

amount of the 2025 Notes to be redeemed, plus accrued and unpaid interest, if any, to, but excluding, the redemption date, but only if the last reported sale price per share of the Company's common stock exceeds 130% of the conversion price on each of at least 20 trading days during the 30 consecutive trading days ending on, and including, the trading day immediately before the date the Company sends the related redemption notice. If a fundamental change (as defined in the 2025 Indenture) occurs, then, subject to certain exceptions, holders may require the Company to repurchase their 2025 Notes at a cash repurchase price equal to the principal amount of the 2025 Notes to be repurchased, plus accrued and unpaid interest, if any, to, but excluding, the fundamental change repurchase date. In the event of conversion, holders would forgo all future interest payments, any unpaid accrued interest and the possibility of further stock price appreciation. Upon the receipt of conversion requests, the settlement of the 2025 Notes will be paid pursuant to the terms of the 2025 Indenture. In the event that all of the 2025 Notes are converted, the Company would be required to repay the principal amount and any conversion premium in any combination of cash and shares of its common stock at the Company's option. In addition, calling the 2025 Notes for redemption will constitute a "make-whole fundamental change."

The Company incurred approximately $8.8 million of debt issuance costs relating to the issuance of the 2025 Notes, which were recorded as a reduction to the 2025 Notes on the Condensed Consolidated Balance Sheets. The debt issuance costs are being amortized and recognized as additional interest expense over the expected life of the 2025 Notes using the effective interest method. The Company determined the expected life of the debt is equal to the seven-year term of the 2025 Notes. The effective interest rate on the 2025 Notes is 2.98%.

On March 11, 2022, the Company completed its repurchase of $207.1 million aggregate principal amount of 2025 Notes for cash, including accrued and unpaid interest, for a total of $213.8 million. This transaction involved a contemporaneous exchange of cash between the Company and holders of the 2025 Notes participating in the issuance of the 2029 Notes. Accordingly, we evaluated the transaction for modification or extinguishment accounting in accordance with ASC 470-50, *Debt – Modifications and Extinguishments* on a creditor-by creditor basis depending on whether the exchange was determined to have substantially different terms. The repurchase of the 2025 Notes and issuance of the 2029 Notes were deemed to have substantially different terms based on the present value of the cash flows or significant difference between the value of the conversion option immediately prior to and after the exchange. Therefore, the repurchase of the 2025 Notes was accounted for as a debt extinguishment. The Company recorded a $7.6 million loss on early extinguishment of debt on its Condensed Consolidated Statements of Operations for the six months ending June 30, 2022, which includes the write-off of related deferred financing costs of $3.4 million. After giving effect to the repurchase, the total remaining principal amount outstanding under the 2025 Notes as of June 30, 2022 was $68.9 million.

The 2025 and 2029 Notes are accounted for in accordance with ASC 470-20, *Debt with conversion and Other Options* ("ASC 470-20") and ASC 815-40, *Contracts in Entity's Own Equity* ("ASC 815-40"). Under ASC 815-40, to qualify for equity classification (or nonbifurcation, if embedded) the instrument (or embedded feature) must be both (1) indexed to the issuer's stock and (2) meet the requirements of equity classification guidance. Based upon the Company's analysis, it was determined that the 2025 Notes and the 2029 Notes do not contain embedded features requiring recognition as derivatives and bifurcation, and therefore are measured at amortized cost and recorded as liabilities on the Condensed Consolidated Balance Sheets.

The 2025 and 2029 Notes do not contain any financial or operating covenants or any restrictions on the payment of dividends, the issuance of other indebtedness or the issuance or repurchase of securities by the Company. There were no events of default for the 2025 Notes or 2029 Notes at June 30, 2022.

The 2025 and 2029 Notes are classified on the Company's Condensed Consolidated Balance Sheets at June 30, 2022 as long-tern convertible debt.

The following table sets forth total interest expense recognized related to the 2025 and 2029 Notes (*in thousands*):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Contractual interest expense	$ 2,210	$ 1,725	$ 4,053	$ 3,450
Amortization of debt discount	—	2,560	—	5,071
Amortization of debt issuance costs	429	226	766	451
Total interest expense for the 2025 and 2029 Notes	$ 2,639	$ 4,511	$ 4,819	$ 8,972

NOTE 11. ACCRUED EXPENSES

Accrued expenses at June 30, 2022 and December 31, 2021 consisted of the following (*in thousands*):

	June 30, 2022	December 31, 2021
Research and development	$ 25,763	$ 26,841
Compensation related costs	22,871	25,998
Sales discounts, rebates, and allowances	14,169	7,493
Accrued royalties	7,501	8,402
Selling, general and administrative	6,244	3,144
Miscellaneous accrued expenses	6,146	3,302
Total accrued expenses	$ 82,694	$ 75,180

NOTE 12. NET LOSS PER COMMON SHARE

Basic and diluted net loss per common share is calculated by dividing net loss applicable to common stockholders by the weighted-average number of common shares outstanding during the period. The Company's potentially dilutive shares, which include outstanding stock options, restricted stock units, and shares issuable upon conversion of the 2025 Notes and 2029 Notes, are considered to be common stock equivalents and are not included in the calculation of diluted net loss per share because their effect is anti-dilutive.

Basic and diluted net loss per share is calculated as follows *(net loss amounts are stated in thousands)*:

	Three Months Ended June 30,					
	2022			2021		
	Shares	Net Loss	Loss per common share	Shares	Net Loss	Loss per common share
Basic and diluted loss per share	63,638,385	$ (67,032)	$ (1.05)	60,571,259	$ (39,012)	$ (0.64)

	Six Months Ended June 30,					
	2022			2021		
	Shares	Net Loss	Loss per common share	Shares	Net Loss	Loss per common share
Basic and diluted loss per share	63,387,009	$ (143,003)	$ (2.26)	58,431,770	$ (92,879)	$ (1.59)

The following common stock equivalents have been excluded because they were anti-dilutive:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Options	10,313,152	9,367,565	10,090,259	9,298,480
Convertible debt	11,697,952	7,113,402	10,026,309	7,113,402
Restricted stock	2,160,842	1,591,426	2,031,149	1,614,123
Total anti-dilutive shares	24,171,946	18,072,393	22,147,717	18,026,005

NOTE 13. COMMITMENTS AND CONTINGENCIES

Contingencies

In October 2021, our Kolbam distributor in France notified us that the French authorities were seeking reimbursement for a portion of Kolbam sales in France during the periods from 2015-2020. During this period, the Company had aggregate revenues from sales of Kolbam in France of approximately $8.0 million. At this time, the Company is not able to estimate the potential liability that may be incurred, if any.

Legal Proceedings

From time to time in the normal course of business, the Company is subject to various legal matters such as threatened or pending claims or litigation. Although the results of claims and litigation cannot be predicted with certainty, the Company does not believe it is a party to any claim or litigation the outcome of which, if determined adversely to it, would individually or in the aggregate be reasonably expected to have a material adverse effect on its results of operations or financial condition.

NOTE 14. SHARE-BASED COMPENSATION

Stock Options

The following table summarizes stock option activity during the six months ended June 30, 2022:

	Shares Underlying Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2021	8,886,284	$ 20.26	6.27	$ 96,577
Granted	1,543,270	27.33		
Exercised	(76,053)	12.45		
Forfeited/canceled	(101,296)	24.11		
Outstanding at June 30, 2022	10,252,205	$ 21.34	6.29	$ 53,798

At June 30, 2022, unamortized stock compensation for stock options was $39.7 million, with a weighted-average recognition period of 2.8 years.

At June 30, 2022, outstanding options to purchase 6.8 million shares of common stock were exercisable with a weighted-average exercise price per share of $19.87.

Restricted Stock Units

Service Based Restricted Stock Units

The following table summarizes the Company's service based restricted stock unit activity during the six months ended June 30, 2022:

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Unvested at December 31, 2021	1,473,949	$ 21.88
Granted	1,040,118	26.74
Vested	(474,224)	21.26
Forfeited/canceled	(35,933)	23.73
Unvested at June 30, 2022	2,003,910	$ 24.52

At June 30, 2022, unamortized stock compensation for service based restricted stock units was $42.5 million, with a weighted-average recognition period of 2.9 years.

Performance Based Restricted Stock Units

The following table summarizes the Company's performance based restricted stock unit activity during the six months ended June 30, 2022:

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Unvested at December 31, 2021	52,500	$ 18.73
Granted	117,208	27.50
Vested	(17,500)	25.26
Forfeited/canceled	—	—
Unvested at June 30, 2022	152,208	$ 24.73

At June 30, 2022, unamortized stock compensation for performance based restricted stock units was $2.7 million, with a weighted-average recognition period of 1.6 years.

Share-Based Compensation

The following table sets forth total share-based compensation for the three and six months ended June 30, 2022 and 2021 (*in thousands*):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Research and development	$ 3,684	$ 2,845	$ 6,852	$ 5,847
Selling, general and administrative	8,953	4,665	13,971	9,357
Total	$ 12,637	$ 7,510	$ 20,823	$ 15,204

NOTE 15. INCOME TAXES

For the six months ended June 30, 2022, we recognized income tax expense of $0.1 million as compared to an income tax expense of $0.4 million for the six months ended June 30, 2021.

NOTE 16. INVENTORY

Inventory, net of reserves, consisted of the following at June 30, 2022 and December 31, 2021 (*in thousands*):

	June 30, 2022	December 31, 2021
Raw materials	$ 4,673	$ 5,205
Work in process	653	255
Finished goods	2,306	1,853
Total inventory	$ 7,632	$ 7,313

The inventory reserve was $3.9 million and $4.1 million at June 30, 2022 and December 31, 2021, respectively.

NOTE 17. ACCOUNTS RECEIVABLE

Accounts receivable, net of reserves for prompt pay discounts and expected credit losses, was $16.7 million and $15.9 million at June 30, 2022 and December 31, 2021, respectively. The total reserves for both periods were immaterial.

The Company's evaluation and application of ASU No. 2016-13, Financial Instruments - Credit Losses for the current period included an assessment of our aged trade receivables balances and their underlying credit risk characteristics. Our evaluation of past events, current conditions, and reasonable and supportable forecasts about the future resulted in an expectation of immaterial credit losses.

NOTE 18. EQUITY OFFERINGS

Underwritten Public Offering of Common Stock

In February 2021, the Company sold an aggregate of 7.5 million shares of its common stock in an underwritten public offering, at a price of $26.75 per share. The net proceeds to the Company from the offering, after deducting the underwriting discounts and offering expenses, were $189.3 million.

At-the-Market Equity Offering

In February 2020, the Company entered into an Open Market Sale Agreement ("ATM Agreement") with Jefferies LLC, as agent (“Jefferies”), pursuant to which the Company may offer and sell, from time to time through Jefferies, shares of its common stock having an aggregate offering price of up to $100.0 million. Of the $100.0 million originally authorized for sale under the ATM Agreement, approximately $28.6 million were sold under the Company’s prior registration statement on Form S-3 (Registration No. 333-227182). An additional $51.9 million were sold under the Company's effective registration statement on Form S-3 (Registration Statement No. 333-259311), which included gross proceeds of $20.1 million from the settlement of 701,600 shares sold under the ATM Agreement in the six months ended June 30, 2022. As of June 30, 2022, an aggregate of $19.5 million remained eligible for sale under the ATM Agreement.

Authorized Shares of Common Stock

On May 14, 2021, in connection with the Company’s 2021 Annual Meeting of Stockholders, the Company’s stockholders approved, among other matters, a Certificate of Amendment (“Certificate of Amendment”) to the Company’s Certificate of Incorporation to increase the number of shares of common stock authorized for issuance thereunder from 100,000,000 to 200,000,000. Effective May 18, 2021, the Certificate of Amendment was filed with the Secretary of State of the State of Delaware.

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

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our unaudited condensed consolidated financial statements and related notes included in this Quarterly Report on Form 10-Q and the audited financial statements and notes thereto as of and for the year ended December 31, 2021 and the related Management's Discussion and Analysis of Financial Condition and Results of Operations, both of which are contained in our Annual Report on Form 10-K for the year ended December 31, 2021, filed with the Securities and Exchange Commission (SEC) on February 24, 2022. Past operating results are not necessarily indicative of results that may occur in future periods. In addition, see the discussion under the heading "Forward-Looking Statements" immediately preceding the consolidated financial statements included under Part I of this Quarterly Report on Form 10-Q.

Overview

We are a biopharmaceutical company headquartered in San Diego, California, focused on identifying, developing and delivering life-changing therapies to people living with rare kidney, liver, and metabolic diseases.

Uncertainty Related to the COVID-19 Pandemic

While the impact of the ongoing COVID-19 pandemic did not have a material adverse effect on our financial position or results of operations for the six months ended June 30, 2022, we have been monitoring the developments and assessing areas where there is potential for our business to be impacted. As of June 30, 2022 and as of the date of this report, the majority of our labor force is still working remotely, at least part of the time, which could, among other things, negatively impact our ability to conduct research and development activities, engage in sales-related initiatives, or efficiently conduct day-to-day operations. Remote work operations also heighten the risk of cyber-attacks and make it more difficult for companies to protect their confidential information. Circumstances arising from the pandemic have slowed and could continue to slow the pace of enrollment in our clinical trials or otherwise hinder patients' abilities to comply with the clinical trial protocols and could ultimately delay the availability of results and analysis of outcomes. Disruptions in the supply chain could negatively impact our ability to source materials or manufacture and distribute product. While to date we have not experienced a material reduction in demand for our commercialized products as a result of the pandemic, we could experience a decrease in new patient identification and increased requests for patient assistance due to increased levels of unemployment, either of which would negatively impact our revenues and hinder our cash flows. Similarly, we could face challenges with regard to healthcare programs, including access and changes in coverage. Growth in revenue could also be impeded by these factors. The financial markets have been subject to significant volatility that, together with rising interest rates, could impact our ability to enter into, modify, and negotiate favorable terms and conditions relative to equity and debt financing activities. We had $553.2 million in cash and cash equivalents and marketable debt securities as of June 30, 2022, which we believe provides sufficient capital to fund our operations for at least the next twelve months. While we have not yet experienced a material impact to date, the full magnitude of the pandemic cannot be measured at this time, and therefore any of the aforementioned circumstances, as well as other factors, may cause our results of operations to vary substantially from year to year and quarter to quarter.

Our Pipeline and Approved Products

We have a diversified pipeline designed to address areas of high unmet need in rare kidney, liver, and metabolic diseases. We invest revenues from our commercial portfolio into our pipeline with the goal of delivering new treatments for diseases with no approved therapies.

The following table summarizes the status of our clinical programs, preclinical programs and approved products, each of which is described in further detail below.

PROGRAM	THERAPEUTIC AREA	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	APPROVED
Sparsentan	FSGS					
Sparsentan	IgAN					
CDCA	CTX					
Pegtibatinase (TVT-058)*	HCU					
NGLY1 Collaboration	NGLY1 Deficiency					
ALGS Collaboration	ALGS					
Thiola EC® and Thiola® (tiopronin)	Cystinuria					
Cholbam® (cholic acid)	Bile Acid Synthesis Disorders due to single enzyme defects and Zellweger Spectrum Disorder (ZSD)					
CDCA/Chenodal® (chenodiol)**	Gallstones/CTX					

* Pegtibatinase (TVT-058) is currently in a Phase 1/2 clinical study.

** CDCA is not indicated for CTX but has received a medical necessity determination in the US by the FDA for CTX. Travere Therapeutics is conducting a Phase 3 clinical trial to examine the safety and efficacy of CDCA (Chenodal®) for the treatment of CTX.

Clinical Programs

Sparsentan

Sparsentan, a Dual Endothelin Angiotensin Receptor Antagonist (DEARA), is a novel investigational product candidate. Pre-clinical data have shown that blockade of both endothelin type A and angiotensin II type 1 pathways in forms of rare chronic kidney disease, reduces proteinuria, protects podocytes and prevents glomerulosclerosis and mesangial cell proliferation. Sparsentan has been granted Orphan Drug Designation for the treatment of FSGS and IgAN in the U.S. and Europe. Sparsentan is currently being evaluated in two pivotal Phase 3 clinical studies in rare kidney diseases, including:

- **Immunoglobulin A nephropathy ("IgAN")** is characterized by hematuria, proteinuria, and variable rates of progressive renal failure. With an estimated prevalence of more than 100,000 people in the United States and greater numbers in Europe and Asia, IgAN is the most common primary glomerular disease. Most patients are diagnosed between the ages of 16 and 35, with up to 40% progressing to end stage kidney disease within 15 years. There are currently no non-immunosuppressive treatments for IgAN approved by the FDA. The current standard of care is renin-angiotensin-aldosterone system ("RAAS") blockade with immunosuppression also being commonly used for patients with significant proteinuria or rapidly progressive glomerulonephritis. In 2018, we announced that the first patient had been dosed in the PROTECT Study, a global, randomized, multicenter, double-blind, parallel-arm, active-controlled pivotal Phase 3 clinical trial evaluating the safety and efficacy of sparsentan in 404 patients with IgAN.

  The PROTECT Study protocol provided for an unblinded analysis of at least 280 patients to be performed after 36 weeks of treatment to evaluate the primary efficacy endpoint - the change in proteinuria (urine protein-to-creatinine ratio) at week 36 from baseline. Secondary efficacy endpoints include the rate of change in eGFR following the initiation of randomized treatment over 58-week and 110-week periods, as well as the rate of change in eGFR over 52-week and 104-week periods following the first six weeks of randomized treatment in approximately 380 patients. In August 2021, we announced positive topline interim results from the ongoing Phase 3 PROTECT Study. The PROTECT Study met its pre-specified interim primary efficacy endpoint with statistical significance. After 36 weeks of treatment, patients receiving sparsentan achieved a mean reduction in proteinuria from baseline of 49.8 percent, compared to a mean reduction in proteinuria from baseline of 15.1 percent for irbesartan-treated patients ($p<0.0001$). We believe that preliminary eGFR data available at the time of the interim analysis are indicative of a potential clinically meaningful treatment effect after two years of treatment. Preliminary results at the time of the interim assessment suggested that sparsentan had been generally well-tolerated in the study and consistent with its overall observed safety profile. The PROTECT Study is fully enrolled and is scheduled to continue as planned on a blinded basis to assess the treatment effect on eGFR slope over 110 weeks in the confirmatory endpoint analysis. Topline results from the confirmatory endpoint analysis are expected in the second half of 2023.

In March 2022, we submitted a New Drug Application ("NDA") to the FDA under Subpart H for accelerated approval of sparsentan for the treatment of IgAN. In May 2022, we announced that the FDA had accepted and granted Priority Review of our NDA under Subpart H for accelerated approval of sparsentan for the treatment of IgAN. The FDA has assigned a Prescription Drug User Fee Act (PDUFA) target action date of November 17, 2022.

We and Vifor (International) Ltd. ("Vifor Pharma"), with whom we entered into a license and collaboration agreement ("License Agreement") in September 2021, are submitting an application for conditional marketing authorization ("CMA") of sparsentan for the treatment of IgAN in Europe, with a review decision, assuming acceptance of the application, expected in the second half of 2023.

- **Focal segmental glomerulosclerosis ("FSGS")** is a leading cause of end-stage kidney disease (ESKD) and nephrotic syndrome. There are currently no FDA-approved pharmacologic treatments for FSGS and there remains a high unmet need for patients living with FSGS as off-label treatments such as ACE/ARBs, steroids, and immunosuppressant agents are effective in only a subset of patients and use of some of these off-label treatments may be further inhibited by their safety profiles. Every year approximately 5,400 patients are diagnosed with FSGS and we estimate that there are more than 40,000 FSGS patients in the United States and a similar number in Europe with approximately half of them being candidates for sparsentan. In 2016, we generated positive data from our Phase 2 DUET study in FSGS. In 2018, we announced the initiation of the Phase 3 DUPLEX study of sparsentan in FSGS. The DUPLEX Study is a global, randomized, multicenter, double-blind, parallel-arm, active-controlled clinical trial evaluating the safety and efficacy of sparsentan in 371 patients. The DUPLEX Study protocol provided for an unblinded analysis of at least 190 patients to be performed after 36 weeks of treatment to evaluate the interim efficacy endpoint - the proportion of patients achieving a FSGS partial remission of proteinuria endpoint (FPRE), which is defined as urine protein-to-creatinine ratio (Up/C) ≤1.5 g/g and a >40% reduction in Up/C from baseline, at week 36. In February 2021, we announced that the ongoing Phase 3 DUPLEX Study achieved its pre-specified interim FSGS partial remission of proteinuria endpoint following the 36-week interim period. After 36 weeks of treatment, 42.0 percent of patients receiving sparsentan achieved FPRE, compared to 26.0 percent of irbesartan-treated patients (p=0.0094). A preliminary review of the results from the interim analysis suggest that, as of the data cut-off, sparsentan has been generally well-tolerated and the overall safety results have been generally comparable between treatment groups. The confirmatory primary endpoint of the DUPLEX Study to support full regulatory approval is the rate of change in eGFR over 108 weeks of treatment. As of the time of the interim analyses, available long-term eGFR data for the confirmatory endpoint were limited. Consistent with the DUPLEX Study protocol, patients will continue in a blinded manner to assess the treatment effect on eGFR slope over 108 weeks in the confirmatory endpoint analysis. The DUPLEX Study is fully enrolled and topline results from the confirmatory endpoint are expected in the first half of 2023.

  In May 2021, we provided a regulatory update regarding the sparsentan FSGS program, including feedback from the FDA that additional data would be needed to potentially support a submission for accelerated approval under subpart H. We and the FDA subsequently aligned on a plan for us to provide the FDA with additional eGFR data from a 2022 data-cut to potentially support a submission for accelerated approval for FSGS. We provided the FDA with such additional eGFR data from the ongoing DUPLEX Study in the first half of 2022 and held a subsequent Type A meeting with the FDA to discuss the data and the potential for a submission for accelerated approval. Following review of the data, the FDA has communicated that it does not deem such data sufficiently supportive for an accelerated approval submission, but indicated that the DUPLEX Study as designed maintains the potential for full approval pending completion of the study. We are planning to continue the study to completion which is expected to occur in the first half of 2023, and file for traditional approval thereafter.

  Pending completion of the DUPLEX Study and data supportive of approval, we and Vifor Pharma are targeting to submit by the end of 2023 a subsequent variation of sparsentan for the treatment of FSGS in Europe. If sparsentan receives marketing authorization in any of the licensed territories, Vifor Pharma will be responsible for all commercialization activities in such licensed territories. We remain responsible for the clinical development of sparsentan and will retain all rights to sparsentan in the United States and rest of world outside of the licensed territories, provided that Vifor Pharma has a right of negotiation to expand the licensed territories into Canada, China, Brazil and/or Mexico.

Pegtibatinase (TVT-058)

Pegtibatinase (TVT-058) is a novel investigational human enzyme replacement candidate being evaluated for the treatment of classical homocystinuria (HCU). Classical HCU is a rare metabolic disorder characterized by elevated levels of plasma homocysteine that can lead to vision, skeletal, circulatory and central nervous system complications. It is estimated that there are at least 3,500 people living with HCU in the United States with similar numbers in Europe. Pegtibatinase has been granted Rare Pediatric Disease, Fast Track and Breakthrough Therapy designations by the FDA, as well as orphan drug designation in the United States and European Union. Pegtibatinase is currently being evaluated in the Phase 1/2 COMPOSE Study, a double blind, randomized, placebo-controlled dose escalation study to assess its safety, tolerability, pharmacokinetics, pharmacodynamics and clinical effects in patients with classical HCU.

In December 2021, we announced positive topline results from the Phase 1/2 COMPOSE Study. Pegtibatinase demonstrated dose-dependent reductions in total homocysteine (tHcy) during the 12 weeks of treatment, and in the highest dose cohort to date evaluating 1.5mg/kg of pegtibatinase twice weekly (BIW), treatment with pegtibatinase resulted in rapid and sustained reductions in total homocysteine (tHcy) through 12 weeks of treatment, including a 55.1% mean relative reduction in tHcy from baseline as well as maintenance of tHcy below a clinically meaningful threshold of 100 µmol. Additionally, in a dose-dependent manner in the study to date, methionine levels were substantially reduced and cystathionine levels were substantially elevated following treatment with pegtibatinase, suggesting that pegtibatinase acts in a manner similar to the native CBS enzyme. To date in the study, pegtibatinase has been generally well-tolerated, with no discontinuations due to treatment-related adverse events.

Based on these results, the Company is in the process of engaging with regulators to establish next steps for a pivotal development program to ultimately support potential approval of pegtibatinase for the treatment of HCU. In parallel, the Company has initiated one additional cohort in the COMPOSE Study to inform and refine formulation work for future development and commercial purposes and to further evaluate the dose response curve for pegtibatinase.

We acquired pegtibatinase as part of the November 2020 acquisition of Orphan Technologies Limited.

Chenodal

Chenodal (chenodeoxycholic acid or CDCA) is a naturally occurring bile acid that is approved for the treatment of people with radiolucent stones in the gallbladder. While indicated for radiolucent stones in the gallbladder, Chenodal has been recognized as the standard of care for cerebrotendinous xanthomatosis (CTX) for more than three decades, although it is not currently labeled for this indication. CTX is a rare, progressive and underdiagnosed bile acid synthesis disorder affecting many parts of the body. In January 2020, we randomized the first patients in our Phase 3 RESTORE Study to evaluate the effects of Chenodal in adult and pediatric patients with CTX, and the study enrollment remains open. The pivotal study is intended to support an NDA submission for marketing authorization of Chenodal for CTX in the United States.

Preclinical Programs

We are a participant in two Cooperative Research and Development Agreements ("CRADAs"), which form a multi-stakeholder approach to pool resources with leading experts, and incorporate the patient perspective early in the therapeutic identification and development process. We have partnered with the National Institutes of Health's National Center for Advancing Translational Sciences ("NCATS") and leading patient advocacy organizations, CDG Care and Alagille Syndrome Alliance, aimed at the identification of potential small molecule therapeutics for NGLY1 deficiency and Alagille syndrome ("ALGS"), respectively. There are no treatment options currently approved for these diseases.

Approved Products

Thiola and Thiola EC (tiopronin)

Thiola and Thiola EC are approved by the FDA for the treatment of cystinuria, a rare genetic cystine transport disorder that causes high cystine levels in the urine and the formation of recurring kidney stones. Due to the larger stone size, cystine stones may be more difficult to pass, often requiring surgical procedures to remove. More than 80 percent of people with cystinuria develop their first stone by the age of 20. More than 25 percent will develop cystine stones by the age of 10. Recurring stone formation can cause loss of kidney function in addition to substantial pain and loss of productivity associated with renal colic and stone passage. While a portion of people living with the disease are able to manage symptoms through diet and fluid intake, the prevalence of cystinuria in the US is estimated to be 10,000 to 12,000, indicating that there may be as many as 4,000 to 5,000 affected individuals with cystinuria in the US that would be candidates for Thiola or Thiola EC.

In June 2019 we announced that the FDA approved 100 mg and 300 mg tablets of Thiola EC, an enteric-coated formulation of Thiola, to be used for the treatment of cystinuria. Thiola EC offers the potential for administration with or without food, and the ability to reduce the number of tablets necessary to manage cystinuria. Thiola EC became available to patients in July 2019.

In May 2021, a generic option for the 100 mg version of the original formulation of Thiola (tiopronin tablets) became available. While the impact to our business has been minimal to date, we are not able to estimate any future impact, including the impact of additional generic entrants, if any.

Cholbam (cholic acid)

The FDA approved Cholbam (cholic acid capsules) in March 2015, the first FDA-approved treatment for pediatric and adult patients with bile acid synthesis disorders due to single enzyme defects, and for adjunctive treatment of patients with peroxisome biogenesis disorder-Zellweger spectrum disorder. The effectiveness of Cholbam has been demonstrated in clinical trials for bile acid synthesis disorders and the adjunctive treatment of peroxisomal disorders. An estimated 200 to 300 patients are current candidates for therapy.

Chenodal (chenodiol)

Chenodal is a synthetic oral form of chenodeoxycholic acid ("CDCA"), a naturally occurring primary bile acid synthesized from cholesterol in the liver. The FDA approved Chenodal for the treatment of people with radiolucent stones in the gallbladder. In 2010, Chenodal was granted orphan drug designation for the treatment of cerebrotendinous xanthomatosis ("CTX"), a rare autosomal recessive lipid storage disease. We acquired Chenodal in March 2014.

While Chenodal is not labeled for CTX, it received a medical necessity determination in the US by the FDA and has been used as the standard of care for more than three decades. We are working to obtain FDA approval of Chenodal for the treatment of CTX and initiated a Phase 3 clinical trial for this indication in January 2020. The prevalence of CTX is estimated in the literature to be as high as 1 in 70,000 in the overall population. Pathogenesis of CTX involves deficiency of the enzyme 27-hydroxylase (encoded by the gene CYP27A1), a rate-limiting enzyme in the synthesis of primary bile acids, including CDCA, from cholesterol. The disruption of primary bile acid synthesis in CTX leads to toxic accumulation of cholesterol and cholestanol in most tissues. Patients may present with intractable diarrhea, premature cataracts, tendon xanthomas, atherosclerosis, and cardiovascular disease in childhood and adolescence. Neurological manifestations of the disease, including dementia and cognitive and cerebellar deficiencies, emerge during late adolescence and adulthood. The types, combinations and severity of symptoms can be different from person to person, and making diagnosis challenging and often delayed. Oral administration of CDCA has been shown to normalize primary bile acid synthesis in patients with CTX.

Results of Operations

Results of operations for the three and six months ended June 30, 2022 compared to the three and six months ended June 30, 2021

Net Product Sales

The following table provides information regarding net product sales (*in thousands*):

	Three Months Ended June 30,			Six Months Ended June 30,		
	2022	2021	Change	2022	2021	Change
Net product revenues by product:						
Bile acid products	$ 25,534	$ 24,974	$ 560	$ 50,609	$ 46,938	$ 3,671
Tiopronin products	25,416	29,643	(4,227)	46,784	55,086	(8,302)
Total net product revenues	$ 50,950	$ 54,617	$ (3,667)	$ 97,393	$ 102,024	$ (4,631)

The decrease in total net product revenues for the three and six months ended June 30, 2022 compared to the three and six months ended June 30, 2021 was primarily due to a decrease in Thiola sales, partially offset by an increase in sales of bile acid products.

Operating Expenses

The following table provides information regarding operating expenses (*in thousands*):

	Three Months Ended June 30,			Six Months Ended June 30,		
	2022	2021	Change	2022	2021	Change
Cost of goods sold	$ 2,051	$ 1,651	$ 400	$ 4,189	$ 3,296	$ 893
Research and development	59,681	51,807	7,874	116,292	99,753	16,539
Selling, general and administrative	52,979	34,965	18,014	99,767	71,743	28,024
Change in fair value of contingent consideration	4,907	1,509	3,398	13,987	10,096	3,891
	$ 119,618	$ 89,932	$ 29,686	$ 234,235	$ 184,888	$ 49,347

Research and development expenses

We make significant investments in research and development in support of our development programs. Research and development costs are expensed as incurred and include salaries and bonuses, benefits, non-cash share-based compensation, license fees, costs paid to third-party contractors to perform research, conduct clinical trials, and develop drug materials, and associated overhead expenses and facility costs.

For the three and six months ended June 30, 2022 as compared to the three and six months ended June 30, 2021, our research and development expenses increased by $7.9 million and $16.5 million, respectively, due to increased headcount and medical affairs activities to support the continued advancement of the sparsentan and pegtibatinase programs, including a net increase in external service provider costs of $2.0 million and $5.2 million, respectively, across all programs. Internal personnel costs in combination with increased headcount to support these programs also increased by $5.9 million and $11.4 million, respectively.

Selling, general and administrative expenses

Selling, general and administrative expenses include salaries and bonuses, benefits, non-cash share-based compensation, professional fees, rent, depreciation and amortization, travel, insurance, business development, sales and marketing programs, and other operating expenses.

For the three and six months ended June 30, 2022 as compared to the three and six months ended June 30, 2021, our selling, general and administrative expenses increased by $18.0 million and $28.0 million, respectively, largely due to increased headcount as a result of operational growth and commercial launch preparations.

Change in the valuation of contingent consideration

For the three and six months ended June 30, 2022 as compared to the three and six months ended June 30, 2021, the change in fair value of contingent consideration is due to the passage of time, updated revenue projections and changes in market driven discount rates.

Other Income (Expenses)

The following table provides information regarding other income (expenses), net (*in thousands*):

	Three Months Ended June 30,			Six Months Ended June 30,		
	2022	2021	Change	2022	2021	Change
Interest income	$ 782	$ 988	$ (206)	$ 1,060	$ 1,397	$ (337)
Interest expense	(2,972)	(4,852)	1,880	(5,487)	(10,173)	4,686
Loss on early extinguishment of debt	—	—	—	(7,578)	—	(7,578)
Other income (expense), net	662	216	$ 446	688	(877)	1,565
	$ (1,528)	$ (3,648)	$ 2,120	$ (11,317)	$ (9,653)	$ (1,664)

The change in our other income (expenses) for the three months ended June 30, 2022 as compared to the three months ended June 30, 2021 of $2.1 million is primarily due to reduced interest expense, which resulted from the adoption of ASU No. 2020-06, Accounting for Convertible Instruments and Contracts in an Entity's Own Equity ("ASU 2020-06") as debt discount is no longer amortized. The change in our other income (expenses) for the six months ended June 30, 2022 as compared to the six months ended June 30, 2021 of $1.7 million is primarily due to loss on early extinguishment of debt in connection with the partial repurchase of the Convertible Senior Notes due 2025, offset by reduced interest expense due to adoption of ASU 2020-06.

Income Tax Benefit (Provision)

For the six months ended June 30, 2022, we recognized an income tax expense of $0.1 million as compared to an income tax expense of $0.4 million for the six months ended June 30, 2021.

At June 30, 2022, we had $7.8 million of unrecognized tax benefits. We did not recognize any interest or penalties related to unrecognized tax benefits during the six months ended June 30, 2022.

Liquidity and Capital Resources

We have financed our operations through a combination of borrowings, sales of our equity securities, and revenues generated from our commercialized products and license and collaboration agreements. Research and development activities have required significant capital investment and are expected to continue to require significant cash expenditure in the future, particularly as our pipeline of drug candidates has expanded and our employee headcount has increased to support those activities. In addition, we continue to evaluate potential opportunities to expand our pipeline and approved products through licenses and acquisitions of products in areas that we believe offer attractive growth characteristics, which may require considerable upfront capital to pursue as well as possible contingent payments upon the future achievement of certain milestones.

We believe that our available cash and short-term investments as of the date of this filing will be sufficient to fund our anticipated level of operations beyond the next 12 months. Management believes that our operating results will vary from quarter to quarter and year to year depending upon various factors including revenues, general and administrative expenses, and research and development expenses.

We had the following balances at June 30, 2022 and December 31, 2021 (*in thousands*):

	June 30, 2022	December 31, 2021
Cash and Cash Equivalents	$ 179,759	$ 165,753
Marketable debt securities	373,414	387,129
Accumulated Deficit	(878,744)	(765,966)
Stockholders' Equity	156,713	302,112
Net Working Capital*	$ 462,285	$ 458,739
Net Working Capital Ratio**	4.71	4.70

* Current assets less current liabilities.
**Current assets divided by current liabilities.

Collaboration and License Proceeds

License and Collaboration Agreement with Vifor Pharma

On September 15, 2021, we entered into a License Agreement with Vifor Pharma, pursuant to which we granted an exclusive license to Vifor Pharma for the commercialization of sparsentan in the Licensed Territories. Under the terms of the License Agreement, we received an upfront payment of $55.0 million in September 2021, and will be eligible for up to $135.0 million in aggregate regulatory and market access related milestone payments and up to $655.0 million in aggregate sales-based milestone payments for a total potential value of up to $845.0 million. We are also entitled to receive tiered double-digit royalties of up to 40 percent of annual net sales of sparsentan in the Licensed Territories.

The Agreement includes a sublicense to Vifor Pharma under our license agreement with Ligand Pharmaceuticals, Inc. (“Ligand”). We remain obligated to make payments to Ligand upon achievement of certain regulatory and sales milestones, as well as an escalating annual royalty between 15 percent and 17 percent of global net sales of licensed products.

Equity Offerings

2021 Underwritten Public Offering of Common Stock

In February 2021, the Company sold an aggregate of 7.5 million shares of its common stock in an underwritten public offering, at a price of $26.75 per share. The net proceeds to the Company from the offering, after deducting the underwriting discounts and offering expenses, were $189.3 million.

At-the-Market Equity Offering

In February 2020, the Company entered into an Open Market Sale Agreement ("ATM Agreement") with Jefferies LLC, as agent (“Jefferies”), pursuant to which the Company may offer and sell, from time to time through Jefferies, shares of its common stock having an aggregate offering price of up to $100.0 million. Of the $100.0 million originally authorized for sale under the ATM Agreement, approximately $28.6 million were sold under the Company’s prior registration statement on Form S-3 (Registration No. 333-227182). An additional $51.9 million were sold under the Company's effective registration statement on Form S-3 (Registration Statement No. 333-259311), which included gross proceeds of $20.1 million from the settlement of 701,600 shares sold under the ATM Agreement in the six months ended June 30, 2022. As of June 30, 2022, an aggregate of $19.5 million remained eligible for sale under the ATM Agreement.

Authorized Shares of Common Stock

On May 14, 2021, in connection with the Company’s 2021 Annual Meeting of Stockholders, the Company’s stockholders approved, among other matters, a Certificate of Amendment (“Certificate of Amendment”) to the Company’s Certificate of Incorporation to increase the number of shares of common stock authorized for issuance thereunder from 100,000,000 to 200,000,000. Effective May 18, 2021, the Certificate of Amendment was filed with the Secretary of State of the State of Delaware.

Operating Leases

Future Minimum Rental Commitments

We have future minimum rental commitments totaling $40.7 million arising from our operating leases. These commitments represent the aggregate base rent through August 2028.

Contingent Cash Payments

Ligand License Agreement

In 2012, we entered into an agreement with Ligand Pharmaceuticals, Inc. ("Ligand") for a worldwide sublicense to develop, manufacture and commercialize a drug technology compound including sparsentan (the “Ligand License Agreement”). The cost of the Ligand License Agreement, which is presented net of amortization in the accompanying Condensed Consolidated Balance Sheets in intangible assets, net, is being amortized to research and development on a straight-line basis through September 30, 2023. As consideration for the license, we are required to make substantial payments upon the achievement of certain milestones, totaling up to $114.1 million. Through June 30, 2022, we have capitalized $15.0 million for contractual milestone payments under the Ligand License Agreement. Should we commercialize sparsentan or any products containing related compounds, we will be obligated to pay to Ligand an escalating annual royalty between 15% and 17% of net sales of all such products.

Stock Purchase and Collaboration Agreement with PharmaKrysto

On March 8, 2022, we entered into a Collaboration Agreement with PharmaKrysto Limited (“PharmaKrysto”), a privately held pre-clinical stage company related to PharmaKrysto's early-stage cystinuria discovery program, and concurrently therewith entered into a Stock Purchase Agreement with PharmaKrysto (together, the "Agreements"). Pursuant to the terms of the Agreements, we paid PharmaKrysto's shareholders $0.6 million in cash to purchase 5% of the outstanding common shares of PharmaKrysto and $0.4 million to PharmaKrysto as a one-time signing fee. Under the Collaboration Agreement, we will fund all research and development expenses for the pre-clinical activities associated with the cystinuria program, which are estimated to be approximately $5.0 million. The Agreements require us to purchase an additional 5% of the outstanding common shares for $1.0 million upon the occurrence of a specified pre-clinical milestone, and grant an option to us to purchase the remaining outstanding shares of PharmaKrysto for $5.0 million upon the occurrence of a subsequent pre-clinical milestone prior to expiration of the option on March 8, 2025. If we elect to exercise the option, we would be required to perform commercially reasonable clinical diligence obligations. In addition, we would be required to make cash milestone payments totaling up to an aggregate $16.0 million upon the achievement of certain development and regulatory milestones, plus tiered royalty payments of less than 4% on future net sales of a product, if approved. We have the right to terminate the Agreements and return the shares for a nominal price at any time upon 60 days’ notice, subject to survival of contingent obligations, if any.

Borrowings

The composition of our convertible senior notes are as follows (*in thousands*):

	June 30, 2022	December 31, 2021
2.25% convertible senior notes due 2029	$ 316,250	$ —
2.50% convertible senior notes due 2025	68,904	276,000
Unamortized debt discount	—	(46,045)
Unamortized debt issuance costs - 2.25% convertible senior notes due 2029	(9,448)	—
Unamortized debt issuance costs - 2.50% convertible senior notes due 2025	(1,016)	(3,374)
Total convertible senior notes, net of unamortized debt discount and debt issuance costs	$ 374,690	$ 226,581

Convertible Senior Notes Due 2029

On March 11, 2022, we completed a registered underwritten public offering of $316.3 million aggregate principal amount of 2.25% Convertible Senior Notes due 2029 (“2029 Notes”), which includes $41.3 million aggregate principal amount of 2029 Notes sold pursuant to the full exercise of the underwriters’ option to purchase additional 2029 Notes. We issued the 2029 Notes under an indenture, dated as of September 10, 2018, as supplemented by the second supplemental indenture, dated as of March 11, 2022 (collectively, the “2029 Indenture”). The 2029 Notes will mature on March 1, 2029, unless earlier repurchased, redeemed, or converted. The 2029 Notes are senior unsecured obligations of ours and bear interest at an annual rate of 2.25%, payable semi-annually in arrears on March 1 and September 1 of each year, beginning on September 1, 2022.

We received net proceeds from the issuance of the 2029 Notes of $306.4 million, after deducting the commissions and offering expenses of $9.9 million. At June 30, 2022, accrued interest on the 2029 Notes of $2.2 million is included in accrued expenses in the accompany Condensed Consolidated Balance Sheets. The 2029 Notes comprise our senior, unsecured obligations and are (i) equal in right of payment with our existing and future senior, unsecured indebtedness; (ii) senior in right of payment to our existing and future indebtedness that is expressly subordinated to the 2029 Notes; (iii) effectively subordinated to our existing and future secured indebtedness, to the extent of the value of the collateral securing that indebtedness; and (iv) structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables.

Holders may convert their 2029 Notes at their option only in the following circumstances: (1) during any calendar quarter commencing after the calendar quarter ending on June 30, 2022 (and only during such calendar quarter), if the last reported sale price per share of our common stock for each of at least 20 trading days, whether or not consecutive, during the period of 30 consecutive trading days ending on, and including, the last trading day of the immediately preceding calendar quarter exceeds 130% of the conversion price on the applicable trading day; (2) during the five consecutive business days immediately after any 10 consecutive trading day period (such 10 consecutive trading day period, the “measurement period”) if the trading price per $1,000 principal amount of 2029 Notes for each trading day of the measurement period was less than 98% of the product of the last reported sale price per share of our common stock on such trading day and the conversion rate on such trading day; (3) upon the occurrence of certain corporate events or distributions on our common stock; (4) if we call the 2029 Notes for redemption; and (5) at any time from, and including, December 1, 2028 until the close of business on the scheduled trading day immediately before the maturity date. We will settle conversions by paying or delivering, as applicable, cash, shares of our common stock, or a combination of cash and shares of our common stock, at our election, based on the applicable conversion rate. The initial conversion rate for the 2029 Notes is 31.3740 shares of our common stock per $1,000 principal amount of 2029 Notes, which represents an initial conversion price of approximately $31.87 per share. If a “make-whole fundamental change” (as defined in the 2029 Indenture) occurs, then we will in certain circumstances increase the conversion rate for a specified period of time.

The 2029 Notes will be redeemable, in whole or in part at our option at any time, and from time to time, on or after March 2, 2026 and, in the case of any partial redemption, on or before the 40th scheduled trading day before the maturity date, at a cash redemption price equal to the principal amount of the 2029 Notes to be redeemed, plus accrued and unpaid interest, if any, to, but excluding, the redemption date but only if the last reported sale price per share of our common stock exceeds 130% of the conversion price on (1) each of at least 20 trading days, whether or not consecutive, during the 30 consecutive trading days ending on, and including, the trading day immediately before the date we send the related redemption notice; and (2) the trading day immediately before the date we send such notice. However, we may not redeem less than all of the outstanding 2029 Notes unless at least $100.0 million aggregate principal amount of 2029 Notes are outstanding and not called for redemption as of the time we send the related redemption notice. In addition, calling any 2029 Note for redemption will constitute a make-whole fundamental change with respect to that 2029 Note, in which case the conversion rate applicable to the conversion of that 2029 Note will be increased in certain circumstances if it is converted after it is called for redemption. If a fundamental change (as defined in the 2029 Indentures) occurs, then, except as described in the 2029 Indentures, holders may require us to repurchase their 2029 Notes at a cash repurchase price equal to the principal amount of the 2029 Notes to be repurchased, plus accrued and unpaid interest, if any, to, but excluding, the fundamental change repurchase date.

In the event of conversion, holders would forgo all future interest payments, any unpaid accrued interest and the possibility of further stock price appreciation. Upon the receipt of conversion requests, the settlement of the 2029 Notes will be paid pursuant to the terms of the 2029 Indenture. In the event that all of the 2029 Notes are converted, we would be required to repay the $316.3 million principal amount and any conversion premium in any combination of cash and shares of its common stock at our option. In addition, calling the 2029 Notes for redemption will constitute a “make-whole fundamental change."

We incurred approximately $9.9 million of debt issuance costs relating to the issuance of the 2029 Notes, which were recorded as a reduction to the 2029 Notes on the Condensed Consolidated Balance Sheets. The debt issuance costs are being amortized and recognized as additional interest expense over the expected life of the 2029 Notes using the effective interest method.

Convertible Senior Notes Due 2025

On September 10, 2018, we completed a registered underwritten public offering of $276.0 million aggregate principal amount of 2.50% Convertible Senior Notes due 2025 ("2025 Notes"), and entered into a base indenture and supplemental indenture agreement ("2025 Indenture") with respect to the 2025 Notes. The 2025 Notes will mature on September 15, 2025, unless earlier repurchased, redeemed, or converted. The 2025 Notes are senior unsecured obligations of the Company and bear interest at an annual rate of 2.50%, payable semi-annually in arrears on March 15 and September 15 of each year, beginning on March 15, 2019.

The net proceeds from the issuance of the 2025 Notes were approximately $267.2 million, after deducting commissions and the offering expenses of $8.8 million payable by us. At June 30, 2022, accrued interest on the 2025 Notes of $0.5 million is included in accrued expenses in the accompany Condensed Consolidated Balance Sheets. The 2025 Notes comprise our senior, unsecured obligations and are (i) equal in right of payment with our existing and future senior, unsecured indebtedness; (ii) senior in right of payment to our existing and future indebtedness that is expressly subordinated to the 2025 Notes; (iii) effectively subordinated to our existing and future secured indebtedness, to the extent of the value of the collateral securing that indebtedness; and (iv) structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables.

Holders may convert their 2025 Notes at their option only in the following circumstances: (1) during any calendar quarter commencing after the calendar quarter ending on December 31, 2018 (and only during such calendar quarter), if the last reported sale price per share of our common stock for each of at least 20 trading days, whether or not consecutive, during the period of 30 consecutive trading days ending on, and including, the last trading day of the immediately preceding calendar quarter exceeds 130% of the conversion price on the applicable trading day; (2) during the five consecutive business days immediately after any 10 consecutive trading day period (“measurement period”) if the trading price per $1,000 principal amount of 2025 Notes for each trading day of the measurement period was less than 98% of the product of the last reported sale price per share of our common stock on such trading day and the conversion rate on such trading day; (3) upon the occurrence of certain corporate events or distributions on our common stock; (4) if we call the 2025 Notes for redemption; and (5) at any time from, and including, May 15, 2025 until the close of business on the scheduled trading day immediately before the maturity date. We will settle conversions by paying or delivering, as applicable, cash, shares of our common stock, or a combination of cash and shares of our common stock, at our election, based on the applicable conversion rate.

The initial conversion rate for the 2025 Notes is 25.7739 shares of our common stock per $1,000 principal amount of 2025 Notes, which represents an initial conversion price of approximately $38.80 per share. If a “make-whole fundamental change” (as defined in the 2025 Indenture) occurs, then we will, in certain circumstances, increase the conversion rate for a specified period of time.

The 2025 Notes will be redeemable, in whole or in part, at our option at any time, and from time to time, on or after September 15, 2022 and, in the case of any partial redemption, on or before the 40th scheduled trading day before the maturity date, at a cash redemption price equal to the principal amount of the 2025 Notes to be redeemed, plus accrued and unpaid interest, if any, to, but excluding, the redemption date, but only if the last reported sale price per share of our common stock exceeds 130% of the conversion price on each of at least 20 trading days during the 30 consecutive trading days ending on, and including, the trading day immediately before the date we send the related redemption notice. If a fundamental change (as defined in the 2025 Indenture) occurs, then, subject to certain exceptions, holders may require us to repurchase their 2025 Notes at a cash repurchase price equal to the principal amount of the 2025 Notes to be repurchased, plus accrued and unpaid interest, if any, to, but excluding, the fundamental change repurchase date.

In the event of conversion, holders would forgo all future interest payments, any unpaid accrued interest and the possibility of further stock price appreciation. Upon the receipt of conversion requests, the settlement of the 2025 Notes will be paid pursuant to the terms of the 2025 Indenture. In the event that all of the 2025 Notes are converted, we would be required to repay the $68.9 million principal amount and any conversion premium in any combination of cash and shares of its common stock at our option. In addition, calling the 2025 Notes for redemption will constitute a “make-whole fundamental change."

We incurred approximately $8.8 million of debt issuance costs relating to the issuance of the 2025 Notes, which were recorded as a reduction to the 2025 Notes on the consolidated balance sheet. The debt issuance costs are being amortized and recognized as additional interest expense over the expected life of the 2025 Notes using the effective interest method.

On March 11, 2022, we repurchased $207.1 million of aggregate principal amount of the 2025 Notes for cash, including accrued and unpaid interest, for a total of $213.8 million. This transaction involved a contemporaneous exchange of cash between us and holders of the 2025 Notes participating in the issuance of the 2029 Notes. We recorded a $7.6 million loss on early extinguishment of debt on its Condensed Consolidated Statements of Operations for the six months ending June 30, 2022, which includes the write-off of related deferred financing costs of $3.4 million. After giving effect to the repurchase, the total remaining principal amount outstanding under the 2025 Notes as of June 30, 2022 was $68.9 million.

Interest Expense

Total interest expense recognized for the three and six months ended June 30, 2022 was $3.0 million and $5.5 million, respectively. Total interest expense recognized for the three and six months ended June 30, 2021 was $4.9 million and $10.2 million, respectively.

Funding Requirements

We believe that our available cash and short-term investments as of the date of this filing will be sufficient to fund our anticipated level of operations beyond the next 12 months. This belief is based on many factors, some of which are beyond our control. Factors that may affect financing requirements include, but are not limited to:

- the rate of progress and cost of our clinical trials, preclinical studies and other discovery and research and development activities, including any delays resulting from the COVID-19 pandemic;
- the timing of, and costs involved in, seeking and obtaining marketing approvals for our products, and in maintaining quality systems standards for our products;

- increases or decreases in revenue from our marketed products, including decreases in revenue resulting from the COVID-19 pandemic and generic entrants, if any;
- debt service obligations on the 2025 Notes and 2029 Notes;
- our ability to manufacture sufficient quantities of our products to meet expected demand;
- the costs of preparing, filing, prosecuting, maintaining and enforcing any patent claims and other intellectual property rights, litigation costs and the results of litigation;
- our ability to enter into collaboration, licensing or distribution arrangements and the terms and timing of these arrangements;
- the potential need to expand our business, resulting in additional payroll and other overhead expenses;
- the potential in-licensing of other products or technologies; and
- the emergence of competing technologies or other adverse market or technological developments.

Future capital requirements will also depend on the extent to which we acquire or invest in additional complementary businesses, products and technologies.

Cash Flows

Cash Flows from Operating Activities

Cash used in operating activities for the six months ended June 30, 2022 was $92.1 million compared to cash used of $20.0 million for the six months ended June 30, 2021. The increase in cash used was primarily attributable to increased research and development and sales, general and administrative expenses.

Cash Flows from Investing Activities

Cash used in investing activities for the six months ended June 30, 2022 was $5.9 million compared to cash used of $177.5 million for the six months ended June 30, 2021. The change was due to the decrease in net purchases of marketable debt securities as a result of the net decrease in financing proceeds.

Cash Flows from Financing Activities

Cash provided by financing activities for the six months ended June 30, 2022 was $114.7 million compared to cash provided of $196.1 million for the six months ended June 30, 2021. The decrease in cash provided was due to the February 2021 issuance of stock through an underwritten public offering that provided $189.3 million, offset by net proceeds of $95.4 million from the March 2022 issuance of the 2029 Notes and repurchase of the 2025 Notes.

Other Matters

Adoption of New Accounting Standards

See Note 2 to our unaudited Condensed Consolidated Financial Statements in this report for a discussion of adoption of new accounting standards.

Recently Issued Accounting Pronouncements

See Note 2 to our unaudited Condensed Consolidated Financial Statements in this report for a discussion of recently issued accounting pronouncements.

Critical Accounting Estimates

Our consolidated financial statements are prepared in accordance with accounting principles generally accepted in the United States of America. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and assumptions that affect the reported amount of assets, liabilities, revenue, costs and expenses, and related disclosures. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions and conditions. See our Annual Report on Form 10-K for the fiscal year ended December 31, 2021 for information about critical accounting estimates as well as a description of our other significant accounting policies.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Our primary exposure to market risk is related to changes in interest rates. As of June 30, 2022, we had cash equivalents and marketable debt securities of approximately $553.2 million, consisting of money market funds, U.S. government agency debt, corporate debt and commercial paper. This exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments are in short-term marketable securities. Our marketable securities are subject to interest rate risk and will fall in value if market interest rates increase. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, a change in interest rates of 100 basis points would have approximately a $1.7 million impact on our investments.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in our reports required by the Exchange Act of 1934, as amended, is recorded, processed, summarized and reported within the timelines specified in the SEC's 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. In designing and evaluating the disclosure controls and procedures, management recognized that any controls and procedures, no matter how well designed and operated, can only provide reasonable assurance of achieving the desired control objectives, and in reaching a reasonable level of assurance, management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

As required by SEC Rule 13a-15(b), we carried out an evaluation, under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, of the effectiveness of the design and operation of our disclosure controls and procedures as of the end of the quarter covered by this report. Based on the foregoing, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

An evaluation was also performed under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, of any change to our internal control over financial reporting that occurred during the quarter covered by this report and that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting. Our evaluation did not identify significant changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Securities Exchange Act of 1934) that occurred during the quarter 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

The information required by this Item is incorporated herein by reference to the Notes to the Unaudited Condensed Consolidated Financial Statements--Note 13 Commitments and Contingencies: Legal Proceedings in Part I, Item 1, of this Quarterly Report on Form 10-Q.

Item 1A. Risk Factors

The following risk factors do not reflect any material changes to the risk factors set forth in our Annual Report on Form 10-K for the fiscal year ended December 31, 2021, other than the revisions to the risk factors set forth below with an asterisk (*) next to the title. The following information sets forth risk factors that could 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. If any of the following risks actually occur, our business, operating results, prospects or financial condition could be harmed. Additional risks not presently known to us, or that we currently deem immaterial, may also affect our business operations.

Risks Related to the Development of our Product Candidates

Our clinical trials may fail to demonstrate the safety and efficacy of our product candidates, including sparsentan and pegtibatinase (TVT-058), which could prevent or significantly delay their regulatory approval.

Before obtaining regulatory approval for the sale of any of our current or future product candidates, including sparsentan and pegtibatinase (TVT-058), we must subject these product candidates to extensive preclinical and clinical testing to demonstrate their safety and efficacy for humans. Clinical trials are expensive, time-consuming and may take years to complete.

We may experience numerous unforeseen events during, or as a result of, preclinical or nonclinical testing and the clinical trial process that could delay or prevent our ability to obtain regulatory approval or commercialize our product candidates, including:

- our preclinical or nonclinical tests or clinical trials may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional preclinical testing or clinical trials or we may abandon projects that we expect to be promising;
- regulators may require us to conduct studies of the long-term effects associated with the use of our product candidates;
- regulators or institutional review boards may not authorize us to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- the FDA or any non-United States regulatory authority may impose conditions on us regarding the scope or design of our clinical trials or may require us to resubmit our clinical trial protocols to institutional review boards for re-inspection due to changes in the regulatory environment;
- the number of patients required for our clinical trials may be larger than we anticipate or participants may drop out of our clinical trials at a higher rate than we anticipate;

- our third-party contractors or clinical investigators may fail to comply with regulatory requirements or fail to meet their contractual obligations to us in a timely manner;
- we might have to suspend or terminate one or more of our clinical trials if we, regulators or institutional review boards determine that the participants are being exposed to unacceptable health risks;
- regulators or institutional review boards may require that we hold, suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements;
- the cost of our clinical trials may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct our clinical trials may be insufficient or inadequate or we may not be able to reach agreements on acceptable terms with prospective clinical research organizations; and
- the effects of our product candidates may not be the desired effects or may include undesirable side effects or the product candidates may have other unexpected characteristics.

These risks and uncertainties impact all of our clinical programs that we pursue and have been amplified by the ongoing COVID-19 pandemic, as described below. We will only obtain regulatory approval to commercialize a product candidate if we can demonstrate to the satisfaction of the FDA, and in the case of foreign commercialization, to the applicable foreign regulatory authorities, in well-designed and conducted clinical trials, that our product candidates are safe and effective and otherwise meet the appropriate standards required for approval for a particular indication.

Our product development costs will also increase if we experience delays in testing or approvals. We do not know whether any preclinical tests or clinical trials will be initiated as planned, will need to be restructured or will be completed on schedule, if at all. Significant preclinical or clinical trial delays also could shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. Such delays could allow our competitors to bring products to market before we do and impair our ability to commercialize our products or product candidates.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete our clinical trials or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining, or may not be able to obtain, marketing approval for one or more of our product candidates;
- obtain approval for indications that are not as broad as intended or entirely different than those indications for which we sought approval; and
- have the product removed from the market after obtaining marketing approval.

In February 2021, we announced that our ongoing pivotal Phase 3 DUPLEX Study of sparsentan in focal segmental glomerulosclerosis (“FSGS”) achieved its pre-specified interim FSGS partial remission of proteinuria endpoint (“FPRE”) after 36 weeks of treatment and in August 2021, we announced that our ongoing pivotal Phase 3 PROTECT Study of sparsentan in IGA Nephropathy (“IgAN”) achieved its pre-specified primary efficacy endpoint after 36 weeks of treatment. Pursuant to the DUPLEX and PROTECT Study protocols, patients are to continue in a blinded manner to assess the treatment effect on eGFR slope over two years in the confirmatory endpoint analyses of the studies. Given that interim results from the studies have been publicly announced, it is possible that we may see a higher than anticipated attrition rate in one or both of these studies. To the extent that an insufficient number of patients choose to remain in either study for the full two years, it could jeopardize our ability to complete the studies and submit for full regulatory approval for sparsentan in FSGS and/or IgAN.

We may not be able to initiate or continue clinical trials in the rare diseases on which we are focused if we are unable to locate a sufficient number of eligible patients willing and able to participate in the clinical trials required by the FDA or foreign regulatory agencies. In addition, as other companies and researchers may be concurrently developing therapies for the same or similar indications that we are focused on, we could face competition for a limited number of patients, investigators and clinical trial sites willing to participate in clinical trials. Our inability to enroll and maintain a sufficient number of patients for any of our current or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

In January 2020, we randomized the first patients in a Phase 3 clinical trial to evaluate the effects of Chenodal in adult and pediatric patients with CTX. The pivotal study, known as the RESTORE study, is intended to support an NDA submission for marketing authorization of Chenodal for CTX in the United States. While Chenodal has been used as the standard of care for CTX for over three decades, it is not labeled for CTX and as such we cannot market this drug candidate for the treatment of CTX unless and until it receives FDA approval for this indication. If we experience delays in obtaining approval or if we fail to obtain approval of Chenodal for the treatment of CTX, our business, financial condition and results of operations could be adversely affected.

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful.

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful. For example, although we observed favorable responses with the physician-initiated treatment of fosmetpantotenate in PKAN patients outside the United States, the Phase 3 FORT Study evaluating the safety and efficacy of fosmetpantotenate compared to placebo in patients with PKAN did not meet its primary endpoint, did not demonstrate a difference between treatment groups, and did not meet its secondary endpoint. In addition, there can be no assurance that the positive eGFR results from the open-label portion of the DUET study of sparsentan in FSGS will be repeated in the Phase 3 clinical trial. Similarly, the positive pre-clinical data we have seen from pegtibatinase (TVT-058) being tested in a mouse model of homocystinuria and the positive topline results we reported in December 2021 from the ongoing Phase 1/2 clinical trial of pegtibatinase (TVT-058) may not be replicated in future studies. We cannot assure that any current or future clinical trials of sparsentan or pegtibatinase (TVT-058) will ultimately be successful.

Before obtaining regulatory approval to conduct clinical trials of our product candidates, we must conduct extensive preclinical tests to demonstrate the safety of our product candidates in animals. Preclinical testing is expensive, difficult to design and implement, and can take many years to complete. In addition, during the clinical development process, additional nonclinical toxicology studies are routinely conducted concurrently with the clinical development of a product candidate. If any of our product candidates show unexpected findings in concurrent toxicology studies, we could experience potentially significant

delays in, or be required to abandon, development of that product candidate. A failure of one or more of our nonclinical studies can occur at any stage of testing.

*** Communications and/or feedback from the FDA or EMA related to our current or planned future clinical trials does not guarantee any particular outcome from regulatory review for such clinical trials, and expedited regulatory review pathways may not actually lead to a faster development or regulatory review or approval process.**

Communications and/or feedback from the FDA or EMA related to our current or future clinical trials does not guarantee any particular outcome from regulatory review for such clinical trials, and expedited regulatory review pathways may not actually lead to a faster development or regulatory review or approval process.

In 2018 we initiated the following Phase 3 clinical trials of sparsentan: 1) a single Phase 3 clinical trial designed to serve as the basis for an NDA and MAA filing for sparsentan for the treatment of FSGS (the "DUPLEX Study"), and 2) a single Phase 3 clinical trial designed to serve as the basis for an NDA and MAA filing for sparsentan for the treatment of IgAN (the "PROTECT Study"). We initiated the DUPLEX Study and the PROTECT Study under the Subpart H pathway for potential accelerated approval in the United States, and potential Conditional Marketing Authorization in Europe, in both jurisdictions based on change in proteinuria. Recognition of change in proteinuria as a surrogate endpoint in kidney disease is a relatively new regulatory development, and, as the field continues to evolve, new learnings may impact regulatory viewpoints. In March 2022, we submitted an NDA to the FDA under Subpart H for accelerated approval of sparsentan for the treatment of IgAN. In May 2022, we announced that the FDA had accepted and granted Priority Review of our NDA under Subpart H for accelerated approval of sparsentan for the treatment of IgAN. The FDA has assigned a Prescription Drug User Fee Act (PDUFA) target action date of November 17, 2022, however there is no guarantee that the FDA will provide a decision on the application by the target action date.

In August 2022, following engagements with the FDA around the potential to file an NDA under Subpart H for accelerated approval of sparsentan for the treatment of FSGS, and feedback from the FDA instead indicating support solely for filing for traditional approval pending completion of the DUPLEX Study, we announced our updated plan (i) to pursue traditional approval for FSGS in the United States following receipt of the full two-year eGFR data from the DUPLEX Study, which is expected in the first half of 2023 and (jj) that we and Vifor Pharma are submitting an application for CMA of sparsentan for the treatment of IgAN in Europe, and that pending completion of the DUPLEX Study and data supportive of approval, we and Vifor Pharma are targeting to submit by the end of 2023 a subsequent variation of sparsentan for the treatment of FSGS in Europe. There is no guarantee that the data from the DUPLEX Study will support a regulatory submission in the United States or Europe.

We expect that the FDA's and EMA's (if the EMA agrees to review a regulatory submission for conditional marketing authorization) determination as to whether the sufficiency of the data from the PROTECT Study supports an accelerated approval (FDA)/conditional marketing authorization (EMA) in either jurisdiction will be made during the application review process based on the totality of the data, including eGFR data available for review from the respective studies. There can be no assurance that the FDA or EMA will deem our achievement of any interim endpoint or measurement in the PROTECT Study to be sufficient to grant accelerated approval or Conditional Marketing Authorization for sparsentan for the treatment of IgAN, or that our timelines will not be delayed notwithstanding the availability of an expedited regulatory review pathway (such as the accelerated approval).

Although the FDA has accepted our submission for accelerated approval of sparsentan for IgAN, there can be no assurance that the study will proceed as planned and there can be no guarantee that the FDA will grant accelerated approval or that the EMA will grant Conditional Marketing Authorization in the EU for sparsentan for IgAN. Furthermore, even if sparsentan is granted accelerated approval for IgAN, there can be no assurance that the post-marketing confirmatory data will support full approval of sparsentan as a treatment for IgAN, or that our timelines will not be delayed notwithstanding the accelerated approval.

In addition, because both the DUPLEX Study and PROTECT Study are evaluating the same compound for the treatment of chronic kidney diseases and utilizing similar endpoints, the risk of success or failure for the two studies may, depending on the outcomes of the studies, end up being correlated.

Although the FDA has granted Fast Track and Breakthrough Therapy designations to pegtibatinase (TVT-058) for the treatment of HCU, there is no guarantee that we will be able to reach agreement with the FDA on the study design for a proposed Phase 3 trial of pegtibatinase (TVT-058) or that pegtibatinase (TVT-058) will be approved for HCU in the future, on an expedited timeline or at all. We intend to use a surrogate endpoint, change in total homocysteine (tHcy) level, as a biomarker to demonstrate efficacy in the future Phase 3 pivotal trial and to support a future marketing application for TVT-058 for the treatment of HCU. While we have commenced discussions with the FDA regarding the use of this biomarker to support a future approval under the traditional pathway, we will need to have further interactions with the FDA as part of the routine regulatory advancement of the program and will need to confirm with the FDA the use of total homocysteine as the pivotal endpoint for the study and align with the FDA on the details of the study, as well as on other elements of the program such as matters related to chemistry, manufacturing and controls.

Obtaining access to an expedited program (such as Fast Track and Breakthrough Therapy designations) may not in fact lead to faster development timelines or achieve faster review or approval than conventional FDA procedures. We may experience delays in approval timelines attributable to, among other things, acquiring sufficient supply of our product to conduct clinical trials, identifying and resolving issues relating to chemistry, manufacturing and controls, or conducting additional preclinical or clinical studies. In addition, the FDA may withdraw access to an expedited program if it believes the access or designation is no longer supported by the data from our program.

An extended delay in the rate of enrollment or data collection in our ongoing Phase 1/2 Study of pegtibatinase (TVT-058), as a result of the COVID-19 pandemic or otherwise, may delay our timelines for analyzing future data from the study.

While we have recently completed enrollment of the initially planned dose cohorts, we are currently enrolling an additional patient cohort in the clinical trial of pegtibatinase (TVT-058) for homocystinuria, a rare disease. Given that this development candidate is still undergoing required testing, we may not be able to initiate or continue clinical trials if we are unable to locate a sufficient number of eligible patients willing and able to participate in the clinical trial required by the FDA or foreign regulatory agencies. In addition, as other companies and researchers may be concurrently developing therapies for the same or similar indications that we are focused on, we could face competition for a limited number of patients, investigators and clinical trial sites willing to participate in

clinical trials. Our inability to enroll and maintain a sufficient number of patients for any of our current or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

If the rate of enrollment in the ongoing Phase 1/2 Study of pegtibatinase (TVT-058) is slower than we anticipate, due to the COVID-19 pandemic or otherwise, or if there are barriers to data collection or monitoring activities due to the COVID-19 pandemic, our timelines for analyzing results from the Phase 1/2 Study of pegtibatinase (TVT-058) could be delayed.

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

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

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure, and any information we determine not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular drug, drug candidate or our business. If the topline data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our product candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

Even if any of our product candidates receives regulatory approval, we and/or a collaborative partner will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense.

Any regulatory approvals that any of our product candidates receives may be subject to significant 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 Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate.

In addition, if the FDA or a comparable foreign regulatory authority approves any product candidates, those products will be subject to extensive and ongoing regulatory requirements, including for the manufacturing, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export, recordkeeping, conduct of potential post-marketing studies and post-market submission requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with current good manufacturing practices and good clinical practices, for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, undesirable side effects caused by the product, problems encountered by our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, either before or after product approval, may result in, among other things:

- restrictions on the marketing, manufacturing, or distribution of the product;
- requirements to include additional warnings on the label;
- requirements to create or enhance a medication guide outlining the risks to patients;
- withdrawal of the product from the market;
- voluntary or mandatory product recalls;
- requirements to change the way the product is administered or for us to conduct additional clinical trials;
- fines, warning or untitled letters or holds on clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us or our strategic partners, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import or export of products;
- injunctions or the imposition of civil or criminal penalties; and
- harm to our reputation.

For example, we have certain post-marketing requirements and commitments associated with Cholbam. Further, we face risks relating to those post-marketing obligations, as well as the commercial acceptance of Cholbam. If the regulatory approval for Chenodal, Cholbam and/or Thiola are withdrawn for any reason, it would have a material adverse impact on our sales and profitability.

The third-party clinical investigators and contract research organizations that we rely upon to conduct our clinical trials may not be diligent, careful or timely, and may make mistakes, in the conduct of our trials.

We depend on third-party clinical investigators and contract research organizations ("CROs") to conduct our clinical trials under agreements with us. The CROs play a significant role in the conduct of our clinical trials. Failure of the CROs to meet their obligations could adversely affect clinical development of our product candidates. The third-party clinical investigators are not our employees and we cannot control the timing or amount of resources they devote to our studies. If their performance is substandard, it could delay or prevent approval of our FDA applications. Moreover, these third-party investigators and CROs may also have relationships with other commercial entities, some of which may compete with us. If third-party investigators and CROs allocate their resources to assist our competitors at our expense, it could harm our competitive position. In response to the COVID-19 pandemic, we have engaged or intend to engage providers of home health and remote monitoring services to assist with the ongoing conduct of our clinical trials in an effort to mitigate disruption caused by COVID-19 related issues. The introduction of new third parties into our ongoing clinical trials increases the risks associated with our dependence on third parties, including the risk that substandard performance by, or competing interests of, such third parties could have a negative impact on our clinical trials. Furthermore, there is no guarantee that the utilization of such home health providers or remote monitoring services will be successful in mitigating disruptions to our clinical trials caused by the COVID-19 pandemic.

Risks Related to the Commercialization of Our Products

The commercial success of Chenodal, Cholbam and Thiola depends on them being considered to be effective drugs with advantages over other therapies.

The commercial success of our products Chenodal, Cholbam and Thiola depends on them being considered to be effective drugs with advantages over other therapies. A number of factors, as discussed in greater detail below, may adversely impact the degree of acceptance of these products, including their efficacy, safety, price and benefits over competing therapies, as well as the coverage and reimbursement policies of third-party payers, such as government and private insurance plans.

If unexpected adverse events are reported in connection with the use of any of these products, physician and patient acceptance of the product could deteriorate and the commercial success of such product could be adversely affected. We are required to report to the FDA events associated with our products relating to death or injury. Adverse events could result in additional regulatory controls, such as a requirement for costly post-approval clinical studies or revisions to our approved labeling which could limit the indications or patient population for a product or could even lead to the withdrawal of a product from the market.

* We are subject to generic competition, and recent developments relating to generic competition for pharmaceutical products could cause our product sales and business to be negatively impacted.

Under the Hatch-Waxman Amendments of the Federal Food, Drug, and Cosmetic Act, a pharmaceutical manufacturer may file an ANDA, seeking approval of a generic copy of an approved innovator product or an NDA under Section 505(b)(2) that relies on the FDA's prior findings of safety and effectiveness in approving the innovator product. A Section 505(b)(2) NDA may be for a new or improved version of the original innovator product. Certain of our products, including Thiola, are subject to immediate competition from compounded and generic entrants, as the ANDA and NDA for these drug products have no remaining or current patent or non-patent exclusivity. In May 2021, a generic option for the 100 mg version of the original formulation of Thiola (tiopronin tablets) was approved by the FDA and additional generic alternatives may be approved in the future. During the three and six months ended June 30, 2022, we experienced a decrease in total net product revenues compared to the three and six months ended June 30, 2021, which was due in part to competition from generic tiopronin tablets. Our future net product revenues from Thiola may continue to be impacted by competition from existing or additional generic alternatives to Thiola.

In addition, there have been a number of recent regulatory and legislative initiatives designed to encourage generic competition for pharmaceutical products, including expedited review procedures for generic manufacturers and incentives designed to spur generic competition of branded drugs. In particular, the FDA and the U.S. Federal Trade Commission ("FTC") have been focused on brand companies' denial of drug supply to potential generic competitors for testing. In December 2019, the CREATES Act was enacted, which provides a legislatively defined private right of action under which generic companies can bring suit against companies who refuse access to product for the bioequivalence testing needed to support approval of a generic product.

We have completed our response to a civil investigative demand from the FTC related to the marketing, sale, distribution and pricing of our products, including Thiola. While the investigation remains open, at this time the FTC has not indicated that it has additional questions for us, and has not initiated any claim or proceeding against us relating to these matters.

We cannot currently predict the specific outcome or impact on our business of such regulatory and legislative initiatives, litigation or investigation. However, it is our policy, which is in compliance with the CREATES Act, to evaluate requests for samples of our branded products, and to provide samples in response to bona fide requests from qualified third parties, including generic manufacturers, subject to specified conditions. We have provided and are in the process of providing samples to certain generic manufacturers.

If additional generic versions of Thiola, or generic versions of Cholbam or Chenodal or any of our other current or future products are approved, sales of that product likely would be negatively impacted, which could have a material adverse impact on our sales and profitability. Both the original formulation of Thiola and Thiola EC are subject to generic competition, and a generic version of either formulation could have a material adverse impact on sales of Thiola EC. In addition, the defense of litigation and response to investigation requests could result in substantial costs, reputational impact, and the diversion of management attention and resources.

Changes in reimbursement practices of third-party payers, or patients' access to insurance coverage, could affect the demand for our products and/or the prices at which they are sold.

The business and financial condition of healthcare-related businesses will continue to be affected by efforts of governments and third-party payers to contain or reduce the cost of healthcare through various means. In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval for sparsentan, pegtibatinase (TVT-058), or any other product candidate that we develop, restrict or regulate post-approval activities and affect our ability to profitably sell sparsentan, pegtibatinase (TVT-058), or any other product candidate for which we obtain marketing approval.

Our products are sold to patients whose healthcare costs are met by third-party payers, such as government programs, private insurance plans and managed-care programs. These third-party payers are increasingly attempting to contain healthcare costs by limiting both coverage and the level of reimbursement for medical products and services. Levels of reimbursement, if any, may be decreased in the future, and future healthcare reform legislation, regulations or changes to reimbursement policies of third-party payers may otherwise adversely affect the demand for and price levels of our products, which could have a material adverse effect on our sales and profitability.

Economic, social, and congressional pressure may result in individuals and government entities increasingly seeking to achieve cost savings through mechanisms that limit coverage or payment for our products. For example, state Medicaid programs are increasingly requesting manufacturers to pay supplemental rebates and requiring prior authorization for use of drugs. Managed care organizations continue to seek price discounts and, in some cases, to impose restrictions on the coverage of particular drugs. Government efforts to reduce Medicaid expenses may lead to increased use of managed care organizations by Medicaid programs. This may result in managed care organizations influencing prescription decisions for a larger segment of the population and a corresponding constraint on prices and reimbursement for our products.

In addition, patients' access to employer sponsored insurance coverage may be negatively impacted by the COVID-19 pandemic or other economic factors that result in increased rates of unemployment. To the extent patients taking our approved therapies become unemployed and experience a reduction to, or increased costs associated with, their insurance coverage, demand for our products could decline, which could have a material adverse effect on our sales and profitability, either as a result of decreased sales of our products and/or increased provision by us of free product to uninsured or commercially insured patients. The extent and duration of this potential impact on our business is currently unknown.

We are dependent on third parties to manufacture and distribute our pharmaceutical products who may not fulfill their obligations.

We have no manufacturing capabilities and rely on third party manufacturers who are sole source suppliers for manufacturing of Chenodal, Cholbam and Thiola. The facilities used by our third-party manufacturers must be approved by the FDA. Our dependence on third parties for the manufacture of our products may harm our profit margin on the sale of products and our ability to deliver products on a timely and competitive basis. If our third-party manufacturers are unable to manufacture to specifications or in compliance with applicable regulatory requirements, our ability to commercialize our products will be adversely impacted and could affect our ability to gain market acceptance for our products and negatively impact our revenues.

We currently have no in-house distribution channels for Chenodal, Cholbam or Thiola and we are dependent on a third-party distributor, Eversana, to distribute such products. We rely on this distributor for all of our proceeds from sales of Chenodal, Cholbam and Thiola in the United States. The outsourcing of our distribution function is complex, and we may experience difficulties that could reduce, delay or stop shipments of such products. If we encounter such distribution problems, and we are unable to quickly enter into a similar agreement with another distributor on substantially similar terms, distribution of Chenodal, Cholbam and/or Thiola could become disrupted, resulting in lost revenues, provider dissatisfaction, and/or patient dissatisfaction.

Governments outside the United States tend to impose strict price controls and reimbursement approval policies, which may adversely affect our prospects for generating revenue.

In some countries, particularly EU countries and EFTA member states, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time (6 to 12 months or longer) after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost effectiveness of our product candidate to other available therapies. In addition, certain governmental authorities may conduct reviews of reimbursement previously provided and assert for various reasons that amounts need to be repaid. For example, in October 2021 our distributor/exploitant in France for our previously marketed product Kolbam informed us that they had received a notice that the price previously paid for Kolbam during its period on the market in France had been recalculated by the agency responsible for pharmaceutical pricing in France, with such notice asserting amounts owed for repayment. While we cannot currently estimate the likelihood that any of such asserted amount will ultimately need to be repaid following the currently ongoing review process and any applicable appeal procedures, we may ultimately determine the need to repay all or a portion of the amounts being asserted. From 2015 through 2020, the period during which we had sales of Kolbam in France, our aggregate revenues from sales of Kolbam in France attributable to all purchasers/payers were approximately $8 million. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels or subject to re-assessment and recoupment procedures, our prospects for generating revenue outside of the United States, if any, could be adversely affected and our business may suffer.

*** We are dependent on Vifor Pharma for the successful commercialization of sparsentan in certain key territories outside of the United States, if approved. We may not be able to establish additional collaborations or other arrangements for sparsentan in other territories, which may adversely impact our ability to generate product revenue in additional jurisdictions.**

Pursuant to the terms of the License Agreement, we granted an exclusive license to Vifor Pharma for the commercialization of sparsentan in the Licensed Territories, which consist of Europe, Australia and New Zealand. Consequently, the commercial success of sparsentan in the Licensed Territories will depend in significant part on the efforts of Vifor Pharma, over which we will have limited control. In December 2021, it was announced that CSL Limited, parent company to CSL Behring, intended to initiate a tender offer to acquire Vifor Pharma. In March 2022, CSL Behring announced the successful outcome of this offer and that the closing of the acquisition now remains subject only to regulatory clearance. We do not currently know what effect, if any, this acquisition, if consummated, will have on our relationship with Vifor Pharma. While our agreement with Vifor Pharma provides that it will remain in place following any

acquisition, there is no guarantee that our collaboration with Vifor Pharma will not be affected, adversely or otherwise, by a change in ownership of Vifor Pharma. Moreover, in connection with CSL Limited's acquisition of Vifor Pharma, if consummated, and any related restructuring, substantially less resources could be devoted to the commercialization of sparsentan in the Licensed Territories, or such efforts could be discontinued entirely. If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell sparsentan, if approved, in additional territories, our ability to generate product revenue outside of the United States and the Licensed Territories may be limited.

We may not be able to rely on orphan drug exclusivity for our products.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs, providing eligibility for orphan drug exclusivity upon regulatory approval if certain jurisdictional-specific conditions are met. For example, Cholbam was granted orphan drug designation in the United States and upon FDA approval of the marketing application in March 2015 was awarded seven years of orphan drug exclusivity, which expired in March 2022. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, that product is entitled to a period of marketing exclusivity, which precludes the applicable regulatory authority from approving another marketing application for the same drug for the same indication for that time period. The applicable period is seven years in the United States and ten years in Europe or, in the case of orphan drugs for which a pediatric investigation plan has been completed, 12 years. Even though we have been awarded orphan drug designation in the United States and Europe for sparsentan for the treatment of FSGS and IgAN and for the pegtibatinase for the treatment of HCU, we may not be able to maintain it in Europe and the orphan drug designation may not result in orphan drug exclusivity in the United States or Europe upon approval. For example, if a competitive product that contains the same active moiety and treats the same disease as our product is shown to be clinically superior to our product, any orphan drug exclusivity we have obtained will not block the approval of such competitive product and we may effectively lose orphan drug exclusivity. Similarly, if a competitive product that contains the same active moiety and treats the same disease as our product candidate is approved for orphan drug exclusivity before our product candidate, we may not be able to obtain approval for our product candidate until the expiration of the competitive product’s orphan drug exclusivity unless our product candidate is shown to be clinically superior to the competitive product.

If we are unable to maintain an effective and specialized sales force, we will not be able to commercialize our products in the United States successfully.

In order to successfully commercialize our products in the United States, we have built a specialized sales force. Factors that may hinder our ability to successfully market and commercially distribute our products include:

- inability of sales personnel to obtain access to or educate adequate numbers of physicians on the benefits and safety of prescribing our products;
- inability to recruit, retain and effectively manage adequate numbers of effective sales personnel;
- lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies that have more extensive product lines; and
- unforeseen delays, costs and expenses associated with maintaining our sales organization.

If we are unable to maintain our sales force for our products, we may not be able to generate sufficient product revenue in the United States. Similarly, if Vifor does not effectively engage or maintain its sales force for sparsentan, our ability to recognize milestone payments and royalties from the Licensed Territories will be adversely affected.

We will need to continue to expend significant time and resources to train our sales forces to be credible, persuasive and compliant in discussing our products with the specialists treating the patients indicated under the product’s label. In addition, if we are unable to effectively train our sales force and equip them with effective marketing materials our ability to successfully commercialize our products could be diminished, which would have a material adverse effect on our business, results of operations and financial condition

Risks Related to our Products and Product Candidates

Our products may not achieve or maintain expected levels of market acceptance or commercial success.

The success of our products is dependent upon achieving and maintaining market acceptance. Commercializing products is time consuming, expensive and unpredictable. There can be no assurance that we will be able to, either by ourselves or in collaboration with our partners or through our licensees, successfully commercialize new products or current products or gain market acceptance for such products. New product candidates that appear promising in development may fail to reach the market or may have only limited or no commercial success.

Further, the discovery of significant problems with a product similar to one of our products that implicate (or are perceived to implicate) an entire class of products could have an adverse effect on sales of the affected products. Accordingly, new data about our products, or products similar to our products, could negatively impact demand for our products due to real or perceived side effects or uncertainty regarding efficacy and, in some cases, could result in product withdrawal.

Our current products and any products that we or a collaboration partner bring to the market, including sparsentan and pegtibatinase (TVT-058), if they receive marketing approval, may not gain market acceptance by physicians, patients, third-party payers, and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and we may not become profitable. The degree of market acceptance of our current products and product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the prevalence and severity of any side effects, including any limitations or warnings contained in a product’s approved labeling;
- the efficacy and potential advantages over alternative treatments;
- the pricing of our product candidates;

- relative convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party insurance coverage and reimbursement.

Even if a potential or current product displays a favorable efficacy and safety profile in preclinical and clinical trials, market acceptance of the product will not be known until after it is launched. The efforts by us or any applicable collaboration partner to educate patients, the medical community, and third-party payers on the benefits of our products may require significant resources and may never be successful. Such efforts to educate the marketplace may require more resources than are required by the conventional marketing technologies employed by our competitors.

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

Certain of the diseases that our current and future product candidates are being developed to address, such as FSGS, IgAN and HCU, are relatively rare. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, may not be accurate.

Currently, most reported estimates of the prevalence of FSGS, IgAN and HCU are based on studies of small subsets of the population of specific geographic areas, which are then extrapolated to estimate the prevalence of the diseases in the broader world population. As new studies are performed the estimated prevalence of these diseases may change. There can be no assurance that the prevalence of FSGS, IgAN or HCU in the study populations accurately reflect the prevalence of these diseases in the broader world population.

If our estimates of the prevalence of FSGS, IgAN or HCU or of the number of patients who may benefit from treatment with sparsentan or pegtibatinase prove to be incorrect or if regulatory approval is conditioned on label restrictions that limit the approved patient population, the market opportunities for our product candidates may be smaller than we believe they are, our prospects for generating revenue may be adversely affected and our business may suffer.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval or commercialization.

Undesirable side effects caused by our product candidates could interrupt, delay or halt clinical trials and could result in the denial of regulatory approval by the FDA or other regulatory authorities for any or all targeted indications, and in turn prevent us from commercializing our product candidates and generating revenues from their sale.

Additionally, if sparsentan receives marketing approval, we expect the FDA will require us to include a REMS and mandatory birth control for women of child-bearing age regarding risk of embryo-fetal toxicity, as has been required for other approved endothelin antagonists.

In addition, if any of our product candidates receive marketing approval and we or others later identify undesirable side effects caused by the product:

- regulatory authorities may require the addition of restrictive labeling statements;
- regulatory authorities may withdraw their approval of the product; and
- we may be required to change the way the product is administered or conduct additional clinical trials.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product or could substantially increase the costs and expenses of commercializing the product candidate, which in turn could delay or prevent us from generating significant revenues from its sale or adversely affect our reputation.

*** We do not currently have patent protection for our commercial products. If we are unable to obtain and maintain protection for the intellectual property relating to our technology and products, the value of our technology and products will be adversely affected.**

Our success will depend in large part on our ability to obtain and maintain protection in the United States and other countries for the intellectual property covering, or incorporated into, our technology and products. The patent situation in the field of biotechnology and pharmaceuticals generally is highly uncertain and involves complex legal, technical, scientific and factual questions. We do not have, and do not expect to obtain, patent protection for Thiola, Chenodal or Cholbam. Additionally, although we have a pending U.S. patent application directed to Thiola EC and/or its use for treating cystinuria, we do not know whether this or any future patent applications will result in a granted patent covering Thiola EC. More generally, we may not be able to obtain additional issued patents relating to our technology or products. Even if issued, patents issued to us or our licensors may be challenged, narrowed, invalidated, held to be unenforceable or circumvented, which could limit our ability to stop competitors from marketing similar products or reduce the term of patent protection we may have for our products. Changes in either patent laws or in interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property or narrow the scope of our patent protection.

Our product candidate sparsentan is covered by U.S. Patent No. 6,638,937, which expired in 2019 and to which we have an exclusive license. In addition, U.S. Patent No. 9,662,312, to which we also have an exclusive license and which was granted on May 30, 2017 and expires in 2030, covers the use of sparsentan for treating glomerulosclerosis, including FSGS. And U.S. Patent No. 9,993,461, to which we also have an exclusive license and which was granted on June 12, 2018 and expires in 2030, covers the use of sparsentan for treating IgA nephropathy as well as glomerulosclerosis, including FSGS.

For products we develop based on a new chemical entity not previously approved by the FDA, we expect that in addition to the protection afforded by our patent filings that we will be able to obtain five years regulatory exclusivity via the provisions of the Food, Drug, and Cosmetic ("FDC") Act and possibly seven years regulatory exclusivity via the orphan drug provisions of the FDC Act. In the case of sparsentan, the periods of regulatory exclusivity may, if certain

conditions are satisfied, be extended by six months on the basis of pediatric exclusivity, thereby resulting in exclusivity periods of 5.5 years and 7.5 years, respectively. In addition, we may be able to obtain up to five years patent term extension (to compensate for regulatory approval delay) for one patent covering such a product for its FDA-approved use. Such a patent, like the periods of regulatory exclusivity, also may be extended by a further six months on the basis of pediatric exclusivity if certain conditions are satisfied.

The degree of future protection for our proprietary rights is uncertain, and we cannot ensure that:

- we or our licensors were the first to make the inventions covered by each of our pending patent applications;
- we or our licensors were the first to file patent applications for these inventions;
- others will not independently develop similar or alternative technologies or duplicate any of our technologies;
- any patents issued to us or our licensors that provide a basis for commercially viable products will provide us with any competitive advantages or will not be challenged by third parties;
- we will develop additional proprietary technologies that are patentable;
- we will file patent applications for new proprietary technologies promptly or at all;
- the claims we make in our patents will be upheld by patent offices in the United States and elsewhere;
- our patents will not expire prior to or shortly after commencing commercialization of a product; and
- the patents of others will not have a negative effect on our ability to do business.

We have negotiated a license agreement with Ligand Pharmaceuticals for the rights to sparsentan which we are initially developing for the treatment of FSGS and IgAN. This license subjects us to various commercialization, reporting and other obligations. If we were to default on our obligations, we and Vifor Pharma could lose our rights to sparsentan. We have obtained a U.S. patent and European patent each covering the use of sparsentan for treating glomerulosclerosis, including FSGS, as well as a second U.S. patent and a second European patent each covering both the use of sparsentan for treating IgAN and the use of sparsentan for treating glomerulosclerosis, including FSGS. In addition, in 2020 we obtained a U.S. patent covering the use of sparsentan for the treatment of Alport syndrome. However, we cannot be certain that we will be able to obtain patent protection for various other potential indications for sparsentan, or whether, if granted, we would be able to enforce such patents. Additionally, in November 2020, a third party filed an opposition to our second European patent (European Patent No. EP3222277, “the ‘277 EP Patent”), in the European Patent Office ("EPO"). While we are vigorously defending the ‘277 EP Patent against the opposition, there is no guarantee that we will be successful in doing so.

Our patents also may not afford us protection against competitors with similar technology. Because patent applications in the United States and many other jurisdictions are typically not published until 18 months after filing, or in some cases not at all, and because publications of discoveries in the scientific literature often lag behind the actual discoveries, neither we nor our licensors can be certain that we or they were the first to make the inventions claimed in our or their issued patents or pending patent applications, or that we or they were the first to file for protection of the inventions set forth in these patent applications. If a third party has also filed a United States patent application prior to the effective date of the relevant provisions of the America Invents Act (i.e. before March 16, 2013) covering our product candidates or a similar invention, we may have to participate in an adversarial proceeding, known as an interference, 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 our efforts could be unsuccessful, resulting in a loss of our United States patent position.

We cannot assure you that third parties will not assert patent or other intellectual property infringement claims against us with respect to technologies used in our products. If patent infringement suits were brought against us, we may be unable to commercialize some of our products which could severely harm our business. Litigation proceedings, even if not successful, could result in substantial costs and harm our business.

We expect to rely on orphan drug status to develop and commercialize certain of our product candidates, but our orphan drug designations may not confer marketing exclusivity or other expected commercial benefits.

We expect to rely on orphan drug exclusivity for sparsentan and potential future product candidates that we may develop. Orphan drug status currently confers seven years of marketing exclusivity in the United States under the FDC Act, and up to ten years of marketing exclusivity in Europe for a particular product in a specified indication or, in the case of orphan drugs for which a pediatric investigation plan has been completed, 12 years. The FDA and EMA have granted orphan designation for Chenodal, sparsentan, and pegtibatinase (TVT-058) for the treatment of CTX, FSGS, IgAN and homocystinuria, respectively. While we have been granted these orphan designations, we will not be able to rely on these designations to exclude other companies from manufacturing or selling these molecules for the same indication beyond these time frames. Furthermore, in Europe, orphan drug status is re-evaluated in connection with the marketing authorization review process and a product candidate must re-qualify as of such time in order to maintain orphan drug status. In addition, any marketing exclusivity in Europe can be reduced from ten years to six years if the initial designation criteria have significantly changed since the market authorization of the orphan product.

For any product candidate for which we have been granted orphan drug designation in a particular indication, it is possible that another company also holding orphan drug designation for the same product candidate will receive marketing approval for the same indication before we do. If that were to happen, our applications for that indication may not be approved until the competing company's period of exclusivity expires. Even if we are the first to obtain marketing authorization for an orphan drug indication in the United States, there are circumstances under which a competing product may be approved for the same indication during the seven-year period of marketing exclusivity, such as if the later product is shown to be clinically superior to our orphan product, or if the later product is deemed a different product than ours. Further, the seven-year marketing exclusivity would not prevent competitors from obtaining approval of the same product candidate as ours for indications other than those in which we have been granted orphan drug designation, or for the use of other types of products in the same indications as our orphan product.

*** Any drugs we develop may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, thereby harming our business.**

In March 2010, President Obama signed the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the "PPACA"), a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The PPACA revised the definition of “average manufacturer price” for reporting purposes, which could increase the amount of Medicaid drug rebates to states. The PPACA also increased the mandated Medicaid rebate from 15.1% to 23.1% of the average manufacturer price, expanded the rebate to Medicaid managed care utilization and increased the types of entities eligible for the federal 340B drug discount program. Further, the law imposed a significant annual fee on companies that manufacture or import certain branded prescription drug products. There have been executive, judicial, Congressional, and political challenges to certain aspects of the PPACA. For example, former President Trump signed several Executive Orders and other directives designed to delay the implementation of certain provisions of the PPACA or otherwise circumvent some of the requirements for health insurance mandated by the PPACA. Concurrently, Congress considered legislation to repeal or repeal and replace all or part of the PPACA. While Congress has not passed comprehensive repeal legislation, several bills affecting the implementation of certain taxes under the PPACA have been signed into law. For example, legislation enacted in 2017 informally titled the Tax Cuts and Jobs Act ("Tax Act") includes a provision which repealed, effective January 1, 2019, the tax-based shared responsibility payment imposed by the PPACA 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”. In addition, the 2020 federal spending package eliminated, effective January 1, 2020, the PPACA-mandated “Cadillac” tax on high-cost employer-sponsored health coverage and medical device tax and, effective January 1, 2021, also eliminated the health insurer tax. The Bipartisan Budget Act of 2018 ("BBA"), among other things, amended the PPACA, effective January 1, 2019, to increase the discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D from 50 percent to 70 percent, and closed the coverage gap in most Medicare drug plans, commonly referred to as the “donut hole”. On June 17, 2021 the U.S. Supreme Court dismissed a challenge on procedural grounds that argued the PPACA is unconstitutional in its entirety because the “individual mandate” was repealed by Congress. Thus, the PPACA will remain in effect in its current form. Further, prior to the U.S. Supreme Court ruling, on January 28, 2021, President Biden issued an executive order that initiated a special enrollment period for purposes of obtaining health insurance coverage through the PPACA marketplace. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the PPACA. It is possible that the PPACA will be subject to judicial or Congressional challenges in the future. It is unclear how any such challenges and the healthcare reform measures of the Biden administration will impact the PPACA and our business.

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. For example, in August 2011, the President signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee on Deficit Reduction did not achieve a targeted deficit reduction of at least $1.2 trillion for fiscal years 2012 through 2021, triggering the legislation’s automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect beginning on April 1, 2013 and, due to subsequent legislative amendments, including the BBA and the Infrastructure Investment and Jobs Act, will stay in effect through 2030 unless additional Congressional action is taken. The COVID-19 relief support 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. Additionally, in January 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 imaging centers.

If we are unable to obtain and maintain coverage and adequate reimbursement from governments or third-party payers for any products that we may develop or if we are unable to obtain acceptable prices for those products, our prospects for generating revenue and achieving profitability will suffer.

Our prospects for generating revenue and achieving profitability will depend heavily upon the availability of coverage and adequate reimbursement for the use of our approved product candidates from governmental and other third-party payers, both in the United States and in other markets. Reimbursement by a third-party payer may depend upon a number of factors, including the third-party payer’s determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

Obtaining reimbursement approval for a product from each government or other third-party payer is a time consuming and costly process that could require us to provide supporting scientific, clinical and cost effectiveness data for the use of our products to each payer. We may not be able to provide data sufficient to gain acceptance with respect to reimbursement. Additionally, we might need to conduct post-marketing studies in order to demonstrate the cost-effectiveness of any future products to such payers’ satisfaction. Such studies might require us to commit a significant amount of management time and financial and other resources. Even when a payer determines that a product is eligible for reimbursement, the payer may impose coverage limitations that preclude payment for some uses that are approved by the FDA or non-United States regulatory authorities. Also, prior authorization for a product may be required. In addition, there is a risk that full reimbursement may not be available for high-priced products. Moreover, eligibility for coverage does not imply that any product will be reimbursed in all cases or at a rate that allows us to make a profit or even cover our costs. Interim payments for new products, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Further, coverage policies and third-party payer reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained, less favorable coverage policies and reimbursement rates may be implemented in the future.

A primary trend in the United States healthcare industry and elsewhere is toward cost containment. We expect the changes made by PPACA, other legislation impacting the Medicare program and the 340B program, and the increasing emphasis on managed care to continue to put pressure on pharmaceutical product pricing. As these concerns continue to grow over the need for tighter oversight, there remains the possibility that the Heath Resources and Services Administration or another agency under the U.S. Department of Health and Human Services ("HHS") will propose regulations or that Congress will explore changes to the 340B program through legislation. For example, on November 30, 2018, the U.S. Health Resources & Services Administration published its final rule regarding the calculation of 340B ceiling price and imposition of civil monetary penalties on manufacturers for knowingly and intentionally overcharging covered entities, which became effective on January 1, 2019. Pursuant to the final rule, after January 1, 2019, manufacturers must calculate 340B program ceiling prices on a quarterly basis. Moreover, manufacturers could be subject to a $5,000 penalty for each instance where they knowingly and intentionally overcharge a covered entity under the 340B program.

There have also been a number of initiatives pending at the state and federal level that could negatively impact the reimbursement for products approved under the accelerated approval pathway in the United States.

Further, there has been increasing legislative and enforcement interest in the United States with respect to drug pricing practices, including several recent U.S. congressional inquiries and federal and state legislation designed to, among other things, increase drug pricing transparency, expedite generic competition, review relationships between pricing and manufacturer patient assistance programs, and reform government program drug reimbursement methodologies. At the federal level, the Trump 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, President Trump announced several executive orders related to prescription drug pricing that attempt to implement several of the Trump administration 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, 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, the Centers for Medicare & Medicaid Services ("CMS") issued an interim final rule implementing President Trump'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. Additionally, on March 11, 2021, President Biden signed the American Rescue Plan Act of 2021 into law, which eliminates the statutory Medicaid drug rebate cap, currently set at 100% of a drug's average manufacturer price, for single source and innovator multiple source drugs, beginning January 1, 2024. Further, in July 2021, the Biden administration released an executive order that included 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. In addition, Congress is considering drug pricing as part of other reform measures. It is unclear whether these or similar policy initiatives will be implemented in the future. At the state level, legislatures are increasingly passing legislation and implementing 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. Also, there have been reports that the U.S. government is considering targeted price controls and reference pricing based on foreign single-payer country access policies, which, if implemented, could adversely affect our revenues.

Any reduction in reimbursement from Medicare, Medicaid or other government-funded programs may result in a similar reduction in payments from private payers. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our drugs. Additionally, we are currently unable to predict what additional legislation or regulation, if any, relating to the healthcare industry may be enacted in the future or what effect recently enacted federal legislation or any such additional legislation or regulation would have on our business.

It is also possible that additional governmental action is taken in response to the COVID-19 pandemic.

We face potential product liability exposure far in excess of our limited insurance coverage.

The use of any of our potential products in clinical trials, and the sale of any approved products, may expose us to liability claims. These claims might be made directly by consumers, health care providers, pharmaceutical companies or others selling our products. We have obtained limited product liability insurance coverage for our clinical trials in the amount of $10 million per occurrence and $25 million in the aggregate. However, our insurance may not reimburse us or may not be sufficient to reimburse us for any expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive, and we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. We intend to expand our insurance coverage to include the sale of commercial products if we obtain marketing approval for product candidates in development, but we may be unable to obtain commercially reasonable product liability insurance for any products approved for marketing. On occasion, juries have awarded large judgments in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or series of claims brought against us would decrease our cash reserves and could cause our stock price to fall.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do. Our operating results will suffer if we fail to compete effectively.

Several of our competitors have substantially greater financial, research and development, distribution, manufacturing and marketing experience and resources than we do and represent substantial long-term competition for us. Other companies may succeed in developing and marketing products that are more effective and/or less costly than any products that may be developed and marketed by us, or that are commercially accepted before any of our products. Factors affecting competition in the pharmaceutical and drug industries vary, depending on the extent to which a competitor is able to achieve a competitive advantage based on its proprietary technology and ability to market and sell drugs. The industry in which we compete is characterized by extensive research and development efforts and rapid technological progress. Although we believe that our orphan drug status and proprietary position with respect to sparsentan may give us a competitive advantage, new developments are expected to continue and there can be no assurance that discoveries by others will not render such potential products noncompetitive. Furthermore, competitors could enter the market with generic versions of our products. For example, a generic option for the 100 mg version of the original formulation of Thola (tiopronin tablets) was approved by the FDA in May 2021.

Our competitive position also depends on our ability to enter into strategic alliances with one or more large pharmaceutical and contract manufacturing companies, attract and retain qualified personnel, develop effective proprietary products, implement development and marketing plans, obtain patent protection, secure adequate capital resources and successfully sell and market our approved products. There can be no assurance that we will be able to successfully achieve all of the foregoing objectives.

*** Use of third parties to manufacture our products and product candidates may increase the risk that we will not have sufficient quantities of our product and product candidates or such quantities at an acceptable cost, and clinical development and commercialization of our product and product candidates could be delayed, prevented or impaired.**

We do not own or operate manufacturing facilities for clinical or commercial production of our products or product candidates. We have limited personnel with experience in drug manufacturing and we lack the resources and the capabilities to manufacture any of our product candidates on a clinical or commercial scale. We outsource all manufacturing and packaging of our preclinical, clinical, and commercial products to third parties. The manufacture of pharmaceutical products requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up initial production and in maintaining required quality control. These problems include difficulties with production costs and yields and quality control, including stability of the product candidate.

We intend to rely on third-party manufacturers for the long-term commercial supply of our development stage product candidates, including sparsentan and pegtibatinase (TVT-058). We expect the manufacturers of each product candidate to, at least initially and potentially for a significant period of time, be single source suppliers to us. Reliance on third-party manufacturers entails risks to which we may not be subject if we manufactured our product candidates or products ourselves, including:

- reliance on the third party for regulatory compliance and quality assurance;
- limitations on supply availability resulting from capacity and scheduling constraints of the third parties, which has been exacerbated with the ongoing COVID-19 pandemic and the global supply chain disruptions;
- impact on our reputation in the marketplace if manufacturers of our products fail to meet the demands of our customers;
- the possible breach of the manufacturing agreement by the third party because of factors beyond our control; and
- the possible termination or nonrenewal of the agreement by the third party, based on its own business priorities, at a time that is costly or inconvenient for us.

The failure of any of our contract manufacturers to maintain high manufacturing standards could result in injury or death of clinical trial participants or patients using our products. Such failure could also result in product liability claims, product recalls, product seizures or withdrawals, delays or failures in testing or delivery, cost overruns or other problems that could seriously harm our business or profitability.

Our contract manufacturers will be required to adhere to FDA regulations setting forth cGMP. These regulations cover all aspects of the manufacturing, testing, quality control and recordkeeping relating to our product candidates and any products that we may commercialize. Our manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our manufacturers are subject to unannounced inspections by the FDA, state regulators and similar regulators outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, failure of regulatory authorities to grant marketing approval of our product candidates, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect regulatory approval and supplies of our product candidates.

Our product and any products that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that are both capable of manufacturing for us and willing to do so. The ongoing COVID-19 pandemic and associated vaccine development and manufacturing efforts have increased demand for the services supplied by many third party manufacturers, including some of those that we utilize for our products and product candidates, and there has recently been, and may continue to be, decreased availability of manufacturing slots at many such facilities. If the third parties that we engage to manufacture products for our developmental or commercial products should halt or cease to continue to do so for any reason, we likely would experience interruptions in cash flows and/or delays in advancing our clinical trials while we identify and qualify replacement suppliers, and we may be unable to obtain replacement supplies on terms that are favorable to us. Later relocation to another manufacturer will also require notification, review and other regulatory approvals from the FDA and other regulators and will subject our production to further cost and instability in the availability of our product candidates. In addition, if we are not able to obtain adequate supplies of our product candidates, or the drug substances used to manufacture them, it will be more difficult for us to sell our products and to develop our product candidates. This could greatly reduce our competitiveness. On March 27, 2020, President Trump signed into law the Coronavirus Aid, Relief, and Economic Security Act (the "CARES Act") in response to the COVID-19 pandemic. Throughout the COVID-19 pandemic, there has been public

concern over the availability and accessibility of critical medical products, and the Act enhances FDA's existing authority with respect to drug shortage measures. Under the Act, manufacturers must have in place a risk management plan that identifies and evaluates the risks to the supply of approved drugs for certain serious diseases or conditions for each establishment where the drug or active pharmaceutical ingredient is manufactured. The risk management plan will be subject to FDA review during an inspection. If we experience shortages in the supply of our marketed products, our results could be materially impacted.

Our current and anticipated future dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins and our ability to develop product candidates and commercialize any products that obtain regulatory approval on a timely and competitive basis.

* Materials necessary to manufacture our products and product candidates may not be available on commercially reasonable terms, or at all, which may delay the development and commercialization of our products and product candidates.

We rely on the manufacturers of our products and product candidates to purchase from third-party suppliers the materials necessary to produce the compounds for our preclinical and clinical studies and rely on these other manufacturers for commercial distribution if we obtain marketing approval for any of our product candidates. Suppliers may not sell these materials to our manufacturers at the time we need them or on commercially reasonable terms and all such prices are susceptible to fluctuations in price and availability due to transportation costs, government regulations, price controls, and changes in economic climate or other foreseen circumstances. We do not have any control over the process or timing of the acquisition of these materials by our manufacturers. In addition, significant increases in inflation and global supply chain disruptions, as well as past and potential future disruptions related to the COVID-19 pandemic and potential future disruptions related to Russia's invasion of Ukraine and global geopolitical tension, have had and may continue to have a negative impact on our manufacturers' ability to acquire the materials necessary for our business. Moreover, we currently do not have any agreements for the commercial production of these materials. If our manufacturers are unable to obtain these materials for our preclinical and clinical studies, product testing and potential regulatory approval of our product candidates would be delayed, significantly impacting our ability to develop our product candidates. If our manufacturers or we are unable to purchase these materials after regulatory approval has been obtained for our product candidates, the commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would materially affect our ability to generate revenues from the sale of our product candidates. For example, in 2021 a membrane used in pegtibatinase (TVT-058) drug substance manufacturing became more difficult to acquire due to the same or similar membranes being used in certain of the COVID-19 vaccine manufacturing and we continue to see challenges with securing materials used in the pegtibatinase manufacturing process that are in short supply as a result of the pandemic. While we believe our contingency plans will enable us to continue the ongoing clinical study of pegtibatinase (TVT-058) with the currently available clinical supplies, there is no guarantee that we will not face additional shortages of this membrane, or other materials necessary to manufacture pegtibatinase (TVT-058) or our other products and product candidates. If our risk mitigation plans are not successful in overcoming these challenges, our pegtibatinase program or other products and product candidates, could be delayed.

Risks Related to Our Business

Our limited operating history makes it difficult to evaluate our future prospects, and our profitability in the future is uncertain.

We face the problems, expenses, difficulties, complications and delays, many of which are beyond our control, associated with any business in its early stages and have a limited operating history on which an evaluation of our prospects can be made. Such prospects should be considered in light of the risks, expenses and difficulties frequently encountered in the establishment of a business in a new industry, characterized by a number of market entrants and intense competition, and in the shift from development to commercialization of new products based on innovative technologies.

We have experienced significant growth over the past five years in the number of our employees and the scope of our operations. We have expanded our sales and marketing, compliance and legal functions in addition to expansion of all functions to support a commercial organization. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. To succeed, we must recruit, retain, manage and motivate qualified clinical, scientific, technical, commercial and management personnel, and we face significant competition for experienced personnel.

Due to our limited resources, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability on the part of our management to manage growth could delay the execution of our business plans or disrupt our operations.

Factors that may inhibit our efforts to commercialize our products without strategic partners or licensees include:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to or educate adequate numbers of physicians to prescribe our products;
- the lack of complementary products to be offered by our sales personnel, which may put us at a competitive disadvantage against companies with broader product lines;
- unforeseen costs associated with expanding our own sales and marketing team for new products or with entering into a partnering agreement with an independent sales and marketing organization; and
- efforts by our competitors to commercialize competitive products.

Moreover, though we generate revenues from product sales arrangements, we may incur significant operating losses over the next several years. Our ability to achieve profitable operations in the future will depend in large part upon successful in-licensing of products approved by the FDA, selling and manufacturing these products, completing development of our products, obtaining regulatory approvals for these products, and bringing these products to market. The likelihood of the long-term success of our company must be considered in light of the expenses, difficulties and delays frequently encountered in the development and commercialization of new drug products, competitive factors in the marketplace, as well as the regulatory environment in which we operate.

In addition, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors.

*** We depend on a highly experienced and skilled workforce to grow and operate our business. If we are unable to attract, retain and engage our employees, we may not be able to grow effectively.**

The execution of our strategic objectives and future success will depend upon our continued ability to identify, hire, develop, motivate and retain a highly qualified workforce. We depend on contributions from our employees, and, in particular, our senior management team, to execute efficiently and effectively. Our success further depends on our ability to attract, retain and motivate highly skilled mid-level and senior managers as well as team members at various levels in the scientific, development, medical and commercial areas of the business.

Our headquarters are based in San Diego, California. This region is home to many other biopharmaceutical companies and many academic and research institutions. Competition for qualified key talent in our market is intense and may limit our ability to hire and retain employees on acceptable terms, or at all. As a result, we may not be able to retain our existing employees or hire new employees quickly enough to meet our needs.

To induce valuable employees to remain at our company, in addition to salary, cash incentives and other employee benefits, we have provided stock options and restricted stock unit ("RSU") awards that vest over time. The value to employees of stock options and RSU 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. Current market conditions and the potential for extreme stock price volatility exacerbates this risk. Despite our efforts to retain valuable employees, members of our management, scientific, development and commercial teams may terminate their employment with us on short notice. All of our employees have at-will employment, which means that they could leave our employment at any time, with or without notice. We do not maintain “key person” insurance policies on the lives of any of our employees.

If we fail to effectively manage our hiring and retention needs, our ability to meet our strategic objectives and our business and operating results may be adversely impacted.

*** The ongoing impacts of the COVID-19 pandemic could materially adversely affect our business, results of operations and financial condition.**

The ongoing COVID-19 pandemic continues to impact domestic and worldwide economic activity, including global supply and financial markets. The COVID-19 pandemic also poses the risk that we or our clinical trial subjects, employees, contractors, collaborators, suppliers and vendors may be prevented from conducting certain clinical trials or other business activities for an indefinite period of time, including due to travel restrictions, quarantines, “stay-at-home” and "shelter-in-place" orders or shutdowns that have been or may be requested or mandated by governmental authorities, or that our or their ability to conduct operations will be negatively impacted by staffing shortages while employees quarantine as a result of exposure to or transmission of the virus. In addition, the COVID-19 pandemic could impact personnel at third-party manufacturing facilities in the United States and other countries, including China, or the availability or cost of materials, which could potentially disrupt the supply chain for our commercial products, our product candidates or the comparator products in our ongoing clinical trials.

The timelines and conduct of our ongoing clinical trials previously have been, and may in the future be affected by the COVID-19 pandemic. For example, in 2020 we experienced a reduction in the rates of patient enrollment in our ongoing clinical trials as a result of the pandemic. While we remain in close contact with our CROs, clinical sites and suppliers in an attempt to manage and mitigate the impacts that the ongoing COVID-19 pandemic may have on our clinical trials and projected timelines and we have implemented certain mitigating measures in accordance with COVID-19 related FDA guidance in an effort to ensure the ongoing safety of the patients in our clinical trials and the continued collection of high quality data, there is no guarantee that such efforts will be successful. As challenging as conducting clinical trials is during normal times, the risks, operational challenges and costs of conducting clinical trials has increased substantially during the pandemic. In addition, restrictions caused by the ongoing COVID-19 pandemic may result in impediments to obtaining biopsies, which could impact the ability to timely obtain diagnoses of IgAN or FSGS.

Beginning in March 2020, substantially all of our workforce began working remotely either all or substantially all of the time as a result of applicable stay-at-home and shelter-in-place orders. As of the date of this report, the majority of our workforce is still working remotely, at least part of the time. The effects of these orders and our related remote-work policies may negatively impact productivity, disrupt our business and delay our development programs, regulatory and commercialization timelines, the magnitude of which will depend, in part, on the length and severity of the restrictions and other limitations on our ability to conduct our business in the ordinary course. Remote work operations also heighten the risk of cyber-attacks and make it more difficult for us to protect our confidential information. In addition, as the applicable orders have recently begun to be lifted and certain of our employees begin to return to the office, we cannot guarantee that our workforce will not face an outbreak that could adversely impact our operations.

While the long-term economic impact and the duration of the COVID-19 pandemic may be difficult to predict, the widespread pandemic has resulted in, and may continue to result in, significant disruption of global financial markets, which could reduce our ability to access capital and could negatively affect our liquidity and the liquidity and stability of markets for our common stock and convertible notes. In addition, a further market correction, recession or depression resulting from the spread of COVID-19 could materially adversely affect our business and the value of our common stock and convertible notes.

Moreover, the COVID-19 pandemic continues to evolve, and the extent to which the COVID-19 pandemic may impact our business, results of operations and financial position will depend on future developments, which are highly uncertain and cannot be predicted with confidence, such as the ultimate geographic spread of the disease, the duration of the pandemic, travel restrictions and social distancing in the United States and other countries, business closures or business disruptions, global supply challenges, and the effectiveness of actions taken in the United States and other countries to contain and treat the disease.

We will likely experience fluctuations in operating results and could incur substantial losses.

We expect that our operating results will vary significantly from quarter-to-quarter and year-to-year as a result of investments in research and development, specifically our clinical and preclinical development activities. We have not completed development of any drugs and we anticipate that our expenses will increase substantially as we:

- continue the open label portion of DUET and conduct the Phase 3 trials of sparsentan;
- continue the research and development of additional product candidates, including pegtibatinase (TVT-058);
- expand our sales and marketing infrastructure to commercialize our current products and any new products for which we may obtain regulatory approval; and
- expand operational, financial, and management information systems and personnel, including personnel to support product development efforts and our obligations as a public company.

Furthermore, the extent of the ultimate impact of the COVID- 19 pandemic on our operational and financial performance will depend on various developments, including the duration and spread of the pandemic, and its impact on potential customers, employees, and vendors, all of which cannot be reasonably predicted at this time.

To attain and sustain profitability, we must succeed in developing and commercializing drugs with significant market potential. This will require us to be successful in a range of challenging activities, including the discovery of product candidates, successful completion of preclinical testing and clinical trials of our product candidates, obtaining regulatory approval for these product candidates and manufacturing, marketing and selling those products for which we may obtain regulatory approval. We are only in the preliminary stages of these activities. We may not be successful enough in these activities to generate revenues that are substantial enough to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become or remain profitable could depress the market price of our common stock and could impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations. A decline in the market price of our common stock may also cause a loss of a part or all of your investment.

Negative publicity regarding any of our products could impair our ability to market any such product and may require us to spend time and money to address these issues.

If any of our products or any similar products distributed by other companies prove to be, or are asserted to be, harmful to consumers and/or subject to FDA enforcement action, our ability to successfully market and sell our products could be impaired. Because of our dependence on patient and physician perceptions, any adverse publicity associated with illness or other adverse effects resulting from the use or misuse of our products or any similar products distributed by other companies could limit the commercial potential of our products and expose us to potential liabilities.

We may not have sufficient insurance to cover our liability in any current or future litigation claims either due to coverage limits or as a result of insurance carriers seeking to deny coverage of such claims.

We face a variety of litigation-related liability risks. Our certificate of incorporation, bylaws, other applicable agreements, and/or Delaware law require us to indemnify (and advance expenses to) our current and past directors and officers and employees from reasonable expenses related to the defense of any action arising from their service to us, including circumstances under which indemnification is otherwise discretionary. While our directors and officers are included in a director and officer liability insurance policy, which covers all our directors and officers in some circumstances, our insurance coverage does not cover all of our indemnification obligations and may not be adequate to cover any indemnification or other claims against us. In addition, the underwriters of our present coverage may seek to avoid coverage in certain circumstances based upon the terms of the respective policies. If we incur liabilities that exceed our coverage under our directors and officers insurance policy or incur liabilities not covered by our insurance, we would have to self-fund any indemnification amounts owed to our directors and officers and employees in which case our results of operations and financial condition could be materially adversely affected. Further, if D&O insurance becomes prohibitively expensive to maintain in the future, we may be unable to renew such insurance on economic terms or unable renew such insurance at all. The lack of D&O insurance may make it difficult for us to retain and attract talented and skilled directors and officers to serve our company, which could adversely affect our business.

*** We may need substantial funding and may be unable to raise capital when needed, which would force us to delay, reduce or eliminate our product development programs or commercialization efforts.**

We expect our general and research and development expenses to increase in connection with our ongoing and planned activities, particularly as we conduct Phase 3 clinical trials of sparsentan, and conduct any other later-stage clinical trials of our product candidates. In addition, subject to obtaining regulatory approval of any of our product candidates, we expect to incur significant commercialization expenses for product sales and marketing, securing commercial quantities of product from our manufacturers, and product distribution. Our expenses have and may continue to increase as a result of increasing inflation in the Unites States and abroad. We currently have no additional commitments or arrangements for any additional financing to fund the research and development and commercial launch of our product candidates. General market conditions, including increases in interest rates and stock price volatility, and ongoing issues arising from the COVID-19 pandemic, Russia's invasion of Ukraine and global geopolitical tensions, as well as market conditions affecting companies in the life sciences industry in general, may make it difficult for us to seek financing from the capital markets on attractive terms, or at all.

Management believes our ability to continue our operations depends on our ability to sustain and grow revenue, results of operations and our ability to access capital markets when necessary to accomplish our strategic objectives. Management believes that we may incur losses in the immediate future. We expect that our operating results will vary significantly from quarter-to-quarter and year-to-year as a result of investments in research and development, specifically our clinical and preclinical development activities. We expect to finance our cash needs from cash on hand and results of operations, and depending on results of operations we may either need additional equity or debt financing, or need to enter into strategic alliances on products in development to continue our operations until we can achieve sustained profitability and positive cash flows from operating activities. Additional funds may not be available to us when we need them on terms that are acceptable to us, or at all. If adequate funds are not available to us on a timely basis, we may be required to reduce or eliminate research development programs or commercial efforts.

Our future capital requirements will depend on many factors, including:

- the progress and results of our pre-clinical and clinical studies of sparsentan for FSGS and IgAN, pegtibatinase (TVT-058) for HCU, Chenodal for CTX, and any other drug candidates;

- the costs, timing and outcome of regulatory review of our product candidates;
- the impacts of inflation and resulting cost increases;
- debt service obligations on the 2025 Notes and 2029 Notes;
- the number and development requirements of other product candidates that we pursue;
- the costs of commercialization activities, including product marketing, sales and distribution;
- the emergence of competing products and technologies and other adverse market developments;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property related claims;
- the extent to which we acquire or invest in businesses, products and technologies; and
- our ability to establish collaborations and obtain milestone, royalty or other payments from any such collaborators.

*** The market price for shares of our common stock may be volatile and purchasers of our common stock could incur substantial losses.**

The price of our stock is likely to be volatile. The stock market in general, and the market for biotechnology companies in particular, have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by many factors, including:

- results of clinical trials of our product candidates or those of our competitors;
- our entry into or the loss of a significant collaboration;
- regulatory or legal developments in the United States and other countries, including changes in the health care payment systems;
- our ability to obtain and maintain marketing approvals from the FDA or similar regulatory authorities outside the United States;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors and issuance of new or changed securities analysts' reports or recommendations;
- general economic, industry and market conditions, including the impacts thereon of rising inflation and interest rates, the COVID19 pandemic, Russia's invasion of Ukraine and global geopolitical tensions;
- results of clinical trials conducted by others on drugs that would compete with our product candidates;
- developments or disputes concerning patents or other proprietary rights;
- public concern over our product candidates or any products approved in the future;
- litigation;
- communications from government officials regarding health care costs or pharmaceutical pricing;
- future sales or anticipated sales of our common stock by us or our stockholders; and
- the other factors described in this "Risk Factors" section.

In addition, the stock markets, and in particular, the Nasdaq Global Market, have experienced extreme price and volume fluctuations that have affected and continue to affect the market prices of equity securities of many pharmaceutical companies. The realization of any of the above risks or any of a broad range of other risks, including those described in these "Risk Factors" could have a dramatic and material adverse impact on the market price of our common stock.

We may be unable to successfully integrate new products or businesses we may acquire.

We intend to expand our product pipeline by pursuing acquisition of pharmaceutical products. If an acquisition is consummated, the integration of the acquired business, product or other assets into our company may also be complex and time- consuming and, if such businesses, products and assets are not successfully integrated, we may not achieve the anticipated benefits, cost-savings or growth opportunities. Potential difficulties that may be encountered in the integration process include the following:

- integrating personnel, operations and systems, while maintaining focus on producing and delivering consistent, high quality products;
- coordinating geographically dispersed organizations;
- distracting employees from operations;
- retaining existing customers and attracting new customers; and
- managing inefficiencies associated with integrating the operations of the Company.

Furthermore, these acquisitions and other arrangements, even if successfully integrated, may fail to further our business strategy as anticipated, expose us to increased competition or challenges with respect to our products or geographic markets, and expose us to additional liabilities associated with an acquired

business, product, technology or other asset or arrangement. Any one of these challenges or risks could impair our ability to realize any benefit from our acquisitions or arrangements after we have expended resources on them.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.

Our business exposes us to potential liability risks inherent in the research, development, manufacturing and marketing of pharmaceutical products. If any of our product candidates in clinical trials or commercialized products harm people we may be subject to costly and damaging product liability claims. We have clinical trial insurance and commercial product liability coverage. However, this insurance may not be adequate to cover all claims. We may be exposed to product liability claims and product recalls, including those which may arise from misuse or malfunction of, or design flaws in, such products, whether or not such problems directly relate to the products and services we have provided. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products that we may develop;
- damage to our reputation;
- regulatory investigations that could require costly recalls or product modifications;
- withdrawal of clinical trial participants;
- costs to defend the related litigation;
- substantial monetary awards to trial participants or patients, including awards that substantially exceed our product liability insurance, which we would then be required to pay from other sources, if available, and would damage our ability to obtain liability insurance at reasonable costs, or at all, in the future;
- loss of revenue;
- the diversion of management’s attention from managing our business; and
- the inability to commercialize any products that we may develop.

A successful product liability claim or a series of claims brought against us could cause our stock price to fall and, if judgments exceed our insurance coverage, could decrease our available cash and adversely affect our business.

We may become involved in certain litigation matters, any of which could result in substantial costs, divert management's attention and otherwise have a material adverse effect on our business, operating results or financial condition.

From time to time we may become involved in certain litigation matters, including those described in Note 13 of the Condensed Consolidated Financial Statements included in this report. Although we intend to vigorously defend our interests in each matter, there is no guarantee that we will be successful and we may have to pay damages awards or otherwise may enter into settlement arrangements in connection with such matters. Any such payments or settlement arrangements could have material adverse effects on our business, operating results or financial condition. Even if we are successful in defending our interests in each matter, litigation with respect to such matters could result in substantial costs and significant adverse impact on our reputation and divert management's attention and resources, which could have a material adverse effect on our business, operating results or financial condition.

* We are subject to significant ongoing regulatory obligations and oversight, which may result in significant additional expense and may limit our commercial success.

We are subject to significant ongoing regulatory obligations, such as safety reporting requirements and additional post-marketing obligations, including regulatory oversight of the promotion and marketing of our products. In addition, the manufacture, quality control, labeling, packaging, safety surveillance, adverse event reporting, storage and recordkeeping for our products are subject to extensive and ongoing regulatory requirements. If we become aware of previously unknown problems with any of our products, a regulatory agency may impose restrictions on our products, our contract manufacturers or us. If we, our products and product candidates, or the manufacturing facilities for our products and product candidates fail to comply with applicable regulatory requirements, a regulatory agency, including the FDA, may send enforcement letters, mandate labeling changes, suspend or withdraw regulatory approval, suspend any ongoing clinical trials, refuse to approve pending applications or supplements filed by us, suspend or impose restrictions on manufacturing operations, request a recall of, seize or detain a product, seek criminal prosecution or an injunction, or impose civil or criminal penalties or monetary fines. In such instances, we could experience a significant drop in the sales of the affected products, our product revenues and reputation in the marketplace may suffer, and we could become the target of lawsuits.

We are also subject to regulation by national, regional, state and local agencies, including but not limited to the FDA, CMS, Department of Justice, the Federal Trade Commission, the HHS Office of Inspector General and other regulatory bodies. The FDC Act, Social Security Act, Public Health Service Act and other federal and state statutes and regulations govern to varying degrees the research, development, manufacturing and commercial activities relating to prescription pharmaceutical products, including preclinical testing, clinical research, approval, production, labeling, sale, distribution, post-market surveillance, advertising, dissemination of information, promotion, marketing, and pricing to government purchasers and government health care programs. Our manufacturing partners are subject to many of the same requirements.

Companies may not promote drugs for “off-label” uses-that is, uses that are not described in the product’s labeling and that differ from those approved by the FDA or other applicable regulatory agencies. However, a company may share truthful and not misleading information that is otherwise consistent with the product’s labeling. A company that is found to have improperly promoted off-label uses may be subject to significant liability, including civil and administrative remedies as well as criminal sanctions. In addition, management’s attention could be diverted from our business operations and our reputation could be damaged.

The federal health care program Anti-Kickback statute prohibits, among other things, knowingly and willfully offering, paying, soliciting, or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any health care item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted broadly to apply to arrangements that pharmaceutical companies have with prescribers, purchasers and formulary managers, among others. Further, the PPACA, among other things, amends the intent requirement of the federal anti-kickback statute so that a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. In addition, the PPACA provides that the government may assert that a claim including items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act. Although there are a number of statutory exceptions and regulatory safe harbors under the federal anti-kickback statute protecting certain common manufacturer business arrangements and activities from prosecution, the exceptions and safe harbors are drawn narrowly and an arrangement must meet all of the conditions specified in order to be fully protected from scrutiny under the federal anti-kickback statute. We seek to comply with the exceptions and safe harbors whenever possible, but our practices, such as our patient assistance programs and prompt pay discounts with certain customers, may not in all cases meet all of the criteria for protection from anti-kickback liability and may be subject to scrutiny.

The federal false claims laws, including the federal False Claims Act, prohibit any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to get a false claim paid. Additionally, the civil monetary penalties statute imposes penalties against any person or entity that, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent. Many pharmaceutical and other health care companies have been investigated and have reached substantial financial settlements with the federal government under the federal False Claims Act for a variety of alleged marketing activities, including providing free product to customers with the expectation that the customers would bill federal programs for the product; providing consulting fees, grants, free travel, and other benefits to physicians to induce them to prescribe the company's products; and inflating prices reported to private price publication services, which may be used by states to set drug payment rates under government health care programs. Companies have been prosecuted for causing false claims to be submitted because of the marketing of their products for unapproved uses. Pharmaceutical and other health care companies have also been prosecuted on other legal theories of Medicare and Medicaid fraud.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. It is not clear whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of any Travere products, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

We also could become subject to government investigations and related subpoenas. Such subpoenas are often associated with previously filed qui tam actions, or lawsuits filed under seal under the federal False Claims Act. Qui tam actions are brought by private plaintiffs suing on behalf of the federal government for alleged violations of the federal False Claims Act. The time and expense associated with responding to such subpoenas, and any related qui tam or other actions, may be extensive, and we cannot predict the results of our review of the responsive documents and underlying facts or the results of such actions. Responding to government investigations, defending any claims raised, and any resulting fines, restitution, damages and penalties, settlement payments or administrative actions, as well as any related actions brought by stockholders or other third parties, could have a material impact on our reputation, business and financial condition and divert the attention of our management from operating our business.

The number and complexity of both federal and state laws continues to increase, and additional governmental resources are being added to enforce these laws and to prosecute companies and individuals who are believed to be violating them. In particular, the PPACA includes a number of provisions aimed at strengthening the government's ability to pursue anti-kickback and false claims cases against pharmaceutical manufacturers and other healthcare entities, including substantially increased funding for healthcare fraud enforcement activities, enhanced investigative powers, amendments to the federal False Claims Act that make it easier for the government and whistleblowers to pursue cases for alleged kickback and false claim violations and public reporting of certain payments and transfers of value by certain pharmaceutical manufacturers to physicians and teaching hospitals nationwide. We anticipate that government scrutiny of pharmaceutical sales and marketing practices will continue for the foreseeable future and subject us to the risk of further government investigations and enforcement actions. Responding to a government investigation or enforcement action would be expensive and time-consuming, and could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

The U.S. Foreign Corrupt Practices Act, and similar worldwide anti-bribery laws generally prohibit companies and their intermediaries from making improper payments to government officials for the purpose of obtaining or retaining business. Our policies mandate compliance with these anti-bribery laws. We operate in parts of the world that have experienced governmental corruption to some degree and in certain circumstances, strict compliance with anti-bribery laws may conflict with local customs and practices or may require us to interact with doctors and hospitals, some of which may be state controlled, in a manner that is different than in the United States. We cannot assure that our internal control policies and procedures will protect us from reckless or criminal acts committed by our employees or agents. Violations of these laws, or allegations of such violations, could disrupt our business and result in criminal or civil penalties or remedial measures, any of which could have a material adverse effect on our business, financial condition and results of operations and could cause the market value of our common stock to decline.

The federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), created new federal criminal statutes that prohibit, among other actions, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payers, and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Like the federal anti-kickback statute, the PPACA amended the intent standard for certain healthcare fraud provisions under HIPAA such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation.

Additionally, the federal Physician Payments Sunshine Act within the PPACA, and its implementing regulations, require that certain manufacturers of drugs, devices, biologicals and medical supplies to report annually information related to certain payments or other transfers of value made or distributed to physicians (defined to include doctors, dentists, optometrists, podiatrists, and chiropractors), other healthcare professionals (such as physician assistants and

nurse practitioners), and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, physicians and teaching hospitals and to report annually certain ownership and investment interests held by physicians and their immediate family members.

Moreover, the Drug Supply Chain Security Act imposes obligations on manufacturers of pharmaceutical products related to product tracking and tracing. We are not sure whether additional legislative changes will be enacted, or whether the current regulations, guidance or interpretations will be changed, or what the impact of such changes on our business, if any, may be.

Also, many states have similar fraud and abuse statutes or regulations, including state anti-kickback and false claims laws, that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payer. Further, certain states require implementation of commercial compliance programs and marketing codes, compliance with the pharmaceutical industry's voluntary compliance guidelines, and compliance with the applicable compliance guidance promulgated by the federal government. Other various state level requirements include restricting payments or the provision of other items of value that may be made to healthcare providers and other potential referral sources; restricting various marketing practices; requiring prescription drug companies to report expenses relating to the marketing and promotion of drug products; requiring the posting of information relating to clinical studies and their outcomes; requiring the registration of sales representatives; requiring the reporting of certain information related to drug pricing; and requiring drug manufacturers to track and report information related to payments, gifts, compensation, and other items of value to physicians and other healthcare providers.

In February 2021, we entered into a limited co-promotion arrangement with Albireo Pharma, Inc. ("Albireo"), providing for our Cholbam dedicated sales representatives to dedicate a portion of their efforts to promoting Albireo's product, Bylvay (odevixibat), in the United States following approval. In July 2021, Albireo announced that the U.S. Food & Drug Administration ("FDA") has approved Bylvay (odevixibat) for the treatment of pruritis in patients with Progressive Familial Intrahepatic Cholestasis ("PFIC"). In addition to our activities in connection with promoting our own products, if our or Albireo's sales representatives violate or are perceived to have violated any applicable regulatory requirement in promoting Bylvay (odevixibat), we could become subject to investigations, litigation, and/or penalties as described above, reputational harm, as well as contractual liabilities associated with the Albireo co-promotion agreement, any of which could have a material adverse effect on our business. The limited co-promotion agreement terminated in July 2022, in accordance with our mutual agreement with Albireo to terminate the agreement upon the one-year anniversary of the July 2021 launch.

If we are not able to obtain and maintain required regulatory approvals, we will not be able to commercialize our products, and our ability to generate revenue will be materially impaired.

Our product candidates, once approved, and the activities associated with their manufacture, marketing, distribution, and sales are subject to extensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries. Failure to adhere to regulations set out by these bodies for one or more of our commercial products could prevent us from commercializing the product candidate in the jurisdiction of the regulatory authority. We have only limited experience in meeting the regulatory requirements incumbent on the sale of drugs in the United States and elsewhere, and expect to rely on third-parties to assist us in these processes. If these third parties fail to adequately adhere to the regulations governing drug distribution and promotion we may be unable to sell our products, which could have a material effect on our ability to generate revenue.

Our product candidates and the activities associated with their development and commercialization, including testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries. Failure to obtain regulatory approval for a product candidate will prevent us from commercializing the product candidate in the jurisdiction of the regulatory authority. We have only limited experience in filing and prosecuting the applications necessary to obtain regulatory approvals and expect to rely on third-party contract research organizations to assist us in this process.

Securing FDA approval requires the submission of extensive preclinical and clinical data and supporting information to the FDA for each therapeutic indication to establish the product candidate's safety and efficacy. Securing FDA approval also requires the submission of information about the product manufacturing process to, and successful inspection of manufacturing facilities by, the FDA. Our future products may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining regulatory approval or prevent or limit commercial use.

Our product candidates may fail to obtain regulatory approval for many reasons, including:

- our failure to demonstrate to the satisfaction of the FDA or comparable regulatory authorities that a product candidate is safe and effective for a particular indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable regulatory authorities for approval;
- our inability to demonstrate that a product candidate's benefits outweigh its risks;
- our inability to demonstrate that the product candidate presents an advantage over existing therapies;
- the FDA's or comparable regulatory authorities' disagreement with the manner in which we interpret the data from preclinical studies or clinical trials;
- failure of the third-party manufacturers with which we contract for clinical or commercial supplies to satisfactorily complete an FDA pre-approval inspection of the facility or facilities at which the product is manufactured to assess compliance with the FDA's cGMP regulations to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity; and
- a change in the approval policies or regulations of the FDA or comparable regulatory authorities or a change in the laws governing the approval process.

The process of obtaining regulatory approvals is expensive, often takes many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in regulatory approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application

may cause delays in the approval or rejection of an application. The FDA and non-United States regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent regulatory approval of a product candidate. Any regulatory approval we ultimately obtain may be limited or subject to restrictions or post approval commitments that render the approved product not commercially viable. Any FDA or other regulatory approval of our product candidates, once obtained, may be withdrawn, including for failure to comply with regulatory requirements or if clinical or manufacturing problems follow initial marketing.

*** We are subject to stringent and changing obligations related to data privacy and security. Our actual or perceived failure to comply with such obligations could lead to regulatory investigations or actions; litigation; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; loss of customers or sales; and other adverse business consequences.**

In the ordinary course of business, we collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, process) personal data and other sensitive information, including proprietary and confidential business data, trade secrets, intellectual property, data we collect about trial participants in connection with clinical trials, and sensitive third-party data. Our data processing activities may 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. For example, HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), and their respective implementing regulations, imposes specific requirements relating to the privacy, security and transmission of individually identifiable health information. Among other things, HITECH, through its implementing regulations, makes certain of HIPAA's privacy and security standards directly applicable to business associates, defined as a person or organization, other than a member of a covered entity's workforce, that creates, receives, maintains or transmits protected health information for or on behalf of a covered entity for a function or activity regulated by HIPAA as well as their covered subcontractors. In addition, the California Consumer Privacy Act of 2018 ("CCPA") imposes obligations on businesses to which it applies. These obligations include, without limitation, providing specific disclosures in privacy notices, affording California residents certain rights related to their personal data, and requiring businesses subject to the CCPA to implement certain measures to effectuate California residents' personal data rights. The CCPA allows for statutory fines for noncompliance (up to $7,500 per violation). In addition, the California Privacy Rights Act of 2020 ("CPRA"), effective January 1, 2023, will expand the CCPA. For example, the CPRA establishes a new California Privacy Protection Agency to implement and enforce the CCPA (as amended), which could increase the risk of an enforcement action. Other states have enacted data privacy laws. For example, but not limited to, Virginia passed its Consumer Data Protection Act, and Colorado passed the Colorado Privacy Act, both of which differ from the CPRA and become effective in 2023. Additional data privacy and security legislation has been proposed at the federal, state, and local levels in recent years, which, along with existing laws, could increase our potential liability, increase compliance costs, or adversely affect our business.

Outside the United States, an increasing number of laws, regulations, and industry standards apply to data privacy and security. For example, the European Union's General Data Protection Regulation ("EU GDPR"), the United Kingdom's GDPR ("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. For example, the EU GDPR imposes significant and complex burdens on processing personal data, particularly for processing "special category personal data" (such as personal data related to health and genetic information), which could be relevant to our operations in the context of our conduct of clinical trials and is of interest to relevant regulators. Under the EU GDPR, government regulators may impose temporary or definitive bans on data processing, as well as fines of up to 20 million euros or 4% of annual global revenue, whichever is greater. Further, under the EU GDPR, individuals may initiate litigation related to processing of their personal data, as well as consumer protection organizations authorized at law to represent data subjects' interests.

In addition, privacy advocates and industry groups around the world have proposed, and may propose, standards with which we are legally or contractually bound to comply. We may also be bound by contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. Additionally, we may publish privacy policies, marketing materials and other statements, such as compliance with certain certifications, regarding data privacy and security. If these policies, materials or statements are found to be deficient, lacking in transparency, deceptive, unfair, or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators or other adverse consequences.

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 originates in the EU, the UK, Switzerland or in other foreign jurisdictions) or prevent us from conducting business in certain countries. Additionally, we are aware of regulatory decisions and actions that have prevented companies from transferring data across boarders. 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 European Economic Area ("EEA") that the European Commission does not consider to provide an adequate level of data privacy and security, such as the United States. The European Commission released a set of "Standard Contractual Clauses" ("SCCs") that are designed to be a valid mechanism to facilitate personal data transfers out of the EEA to these jurisdictions. Currently, these SCCs are a valid mechanism to transfer personal data outside of the EEA. Additionally, the SCCs impose additional compliance burdens, such as conducting transfer impact assessments to determine whether additional security measures are necessary to protect the at-issue personal data. As we incorporate the new SCCs into our contractual arrangements, we may be required to expend significant resources to update our contractual arrangements and to comply with such obligations. In addition, 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, and have also passed or are considering laws requiring local data residency or otherwise impeding the transfer of personal data across borders, any of which could increase the cost and complexity of doing business.

If we are unable to implement a valid compliance mechanism for cross-border personal data transfers, we may face increased exposure to regulatory actions, substantial fines and injunctions against processing or transferring personal data from Europe. Inability to import personal data from Europe to the United States may significantly and negatively impact our business operations, including by limiting our ability to conduct clinical trial activities in Europe and

elsewhere; limiting our ability to collaborate with CROs, service providers, contractors and other companies that are subject to such cross-border data transfer or localization laws; or requiring us to increase our personal data processing capabilities and infrastructure in foreign jurisdictions at significant expense.

Our obligations related to data privacy and security are quickly changing in an increasingly stringent fashion, creating some uncertainty as to the effective future legal framework. Additionally, these obligations may be subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions. Preparing for and complying with these obligations requires significant resources and may necessitate changes to our information technologies, systems, and practices and to those of any third parties that process personal data on our behalf. Although we endeavor to comply with all applicable data privacy and security obligations, we may at times fail (or be perceived to have failed) to do so. Moreover, despite our efforts, our personnel or third parties upon whom we rely may fail to comply with such obligations, which could negatively impact our business operations and compliance posture. For example, any failure by a third-party service provider to comply with applicable law, regulations, or contractual obligations could result in adverse effects, including proceedings against us by governmental entities or others. If we or any of our partners fail to comply or are perceived to have failed to comply with applicable regulatory requirements, we or they could be subject to a range of regulatory actions or litigation that could affect our or our partners' ability to commercialize our products and conduct necessary research and development, and could harm or prevent sales of the affected products, or could substantially increase the costs and expenses of commercializing and marketing our products. Any threatened or actual government enforcement action or litigation could also generate adverse publicity and require that we devote substantial resources that could otherwise be used in other aspects of our business. Compliance with applicable federal, state, and foreign laws is difficult and time consuming, and companies that violate them may face substantial penalties. The potential sanctions include significant criminal fines, civil monetary penalties, administrative penalties, disgorgement, exclusion from participation in federal health care programs, individual imprisonment, injunctions, recall or seizure of products, total or partial suspension of production, reputational harm, administrative burdens, interruption or cessation of clinical trials, additional oversight and reporting obligations if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, diminished profits and future earnings, and the curtailment or restructuring of our operations, and other sanctions. Because of the breadth of these laws, it is possible that some of our business activities could be subject to challenge under one or more of these laws. Such a challenge, irrespective of the underlying merits of the challenge or the ultimate outcome of the matter, could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

*** If our information technology systems or data, or those of third parties upon which we rely, are or were compromised, we could experience adverse impacts resulting from such compromise, including, but not limited to, regulatory investigations or actions; litigation; fines and penalties; interruptions to our operations such as clinical trials; harm to our reputation; loss of revenue or profits; loss of sales and other adverse consequences.**

In the ordinary course of our business, we and our third-party service providers may process proprietary, confidential, and sensitive data, 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. We may share or receive sensitive information with or from third parties.

Cyberattacks, malicious internet-based activity, and online and offline fraud are prevalent and continue to increase. These threats are becoming increasingly difficult to detect. These threats come from a variety of sources, including traditional computer "hackers," threat actors, personnel (such as through theft or misuse), "hacktivists", organized criminal threat actors, sophisticated nation-states, and nation-state-supported actors. Some actors now engage and are expected to continue to engage in cyberattacks, including without limitation nation-state actors for geopolitical reasons and in conjunction with military conflicts and defense activities. During times of war and other major conflicts, including the war in Ukraine, we and the third parties upon which we rely may be vulnerable to a heightened risk of these attacks, including retaliatory cyberattacks that could materially disrupt our systems and operations, supply chain, and ability to produce, sell and distribute our goods and services. We and the third parties upon which we rely may be subject to a variety of other evolving threats, including, but not limited to, social-engineering attacks (including through phishing attacks), malicious code (such as viruses and worms), malware (including as a result of advanced persistent threat intrusions), denial-of-service attacks (such as credential stuffing), personnel misconduct or error, ransomware attacks, supply-chain attacks, software bugs, server malfunctions, software or hardware failures, loss of data or other information technology assets, adware, telecommunications failures, and other similar threats. In particular, ransomware attacks, including those from organized criminal threat actors, nation-states and nation-state supported actors, are becoming increasingly prevalent and severe and can lead to significant interruptions, delays, or outages in our operations, disruption of clinical trials, loss of data (including data related to clinical trials), loss of income, significant extra expenses to restore data or systems, reputational loss and the diversion of funds. To alleviate the financial, operational and reputational impact of a ransomware attack, it may be preferable to make extortion payments, but we may be unwilling or unable to do so (including, for example, if applicable laws prohibit such payments). Similarly, supply chain attacks have increased in frequency. Additionally, the COVID-19 pandemic and our remote workforce poses increased risks to our information technology systems and data, as more of our employees work from home, utilizing network connections outside our premises. 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. A security incident or other interruption could disrupt our ability (and that of third parties upon whom we rely) to provide our products and services. We may expend significant resources or modify our business activities (including our clinical trial activities) to try to protect against security incidents. Certain data privacy and security obligations may require us to implement and maintain specific security measures, industry-standard or reasonable security measures to protect our information technology systems and sensitive information.

While we have implemented security measures designed to protect against security incidents, there can be no assurance that these measures will be effective. We may be unable in the future to detect vulnerabilities in our information technology systems (including our products) because such threats and techniques change frequently, are often sophisticated in nature, and may not be detected until after a security incident has occurred. Despite our efforts to

identify and remediate vulnerabilities, if any, in our information technology systems, our efforts may not be successful and could result in a material disruption of our programs and operations. For example, the loss of clinical trial data from completed or ongoing clinical trials for any of our product candidates could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Further, we may experience delays in developing and deploying remedial measures designed to address any such identified vulnerabilities

Applicable data security obligations may require us to notify relevant stakeholders of any security incidents, including affected individuals, customers, and regulators. Such disclosures are costly, and the disclosures or the failure to comply with such requirements, could lead to adverse consequences. 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 cause customers to stop using our products or services, deter new customers from using our products or services, and negatively impact our ability to grow and operate our business.

Our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our data privacy and security obligations. In addition, our insurance coverage may not be adequate or sufficient to protect us from or to mitigate liabilities arising out of our privacy and security practices or that such coverage will continue to be available on commercially reasonable terms or at all, or that such coverage will pay future claims.

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

New income, sales, use or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could affect the tax treatment of our domestic and foreign earnings. Any new taxes could adversely affect our domestic and international business operations, and our business and financial performance. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us. For example, the Biden administration and Congress have proposed various U.S. federal tax law changes, which if enacted could have a material impact on our business, cash flow, financial condition or results of operations. In addition, it is uncertain if and to what extent various states will conform to federal tax laws. Future reform legislation could have a material impact on the value of our deferred tax assets, could result in significant one-time charges, and could increase our future U.S. tax expense.

Our effective tax rate may fluctuate, and we may incur obligations in tax jurisdictions in excess of accrued amounts.

Our effective tax rate is derived from a combination of applicable tax rates in the various places that we operate. In preparing our financial statements, we estimate the amount of tax that will become payable in each of such places. Nevertheless, our effective tax rate may be different than experienced in the past due to numerous factors, including changes in the mix of our profitability from state to state, the results of examinations and audits of our tax filings, our inability to secure or sustain acceptable agreements with tax authorities, changes in accounting for income taxes and changes in tax laws. Any of these factors could cause us to experience an effective tax rate significantly different from previous periods or our current expectations and may result in tax obligations in excess of amounts accrued in our financial statements.

Our ability to use net operating loss carryforwards and certain other tax attributes to offset future taxable income and taxes may be subject to limitations.

Under current law, our federal net operating losses ("NOLs") generated in tax years beginning after December 31, 2017, may be carried forward indefinitely, but the deductibility of such federal NOLs in tax years beginning after December 31, 2020, is limited to 80% of taxable income. As of December 31, 2021, we had federal net operating loss ("NOL") of $85.8 million. It is uncertain if and to what extent various states will conform to federal tax laws. In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, if a corporation undergoes an “ownership change,” which is generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation’s ability to use its pre-change NOL carryforwards and other pre-change U.S. tax attributes (such as research tax credits) to offset its post-change income or taxes may be limited. It is possible that we have experienced an ownership change in the past. In addition, we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control.

As a result, our federal NOL carryforwards may be subject to a percentage limitation if used to offset income in tax years following an ownership change. In addition, it is possible that we have in the past undergone, and in the future may undergo, additional ownership changes that could limit our ability to use all of our pre-change NOL carryforwards and other pre-change tax attributes (such as research tax credits) to offset our post-change income or taxes. Similar provisions of state tax law may also apply to limit our use of accumulated state tax attributes. In addition, at the state level, there may be periods during which the use of NOL carryforwards is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. As a result, we may be unable to use all or a material portion of our NOL carryforwards and other tax attributes, which would harm our future operating results by effectively increasing our future tax obligations.

Changes in funding for the FDA, the SEC and other government agencies could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal functions on which the operation of our business may rely, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept payment of user fees, and statutory, regulatory, and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical FDA, SEC and other government employees and stop critical activities. If a prolonged government shutdown occurs, or if resource constraints continue to arise from the COVID-19 pandemic, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

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

Following the result of a referendum in 2016, the United Kingdom left the European Union on January 31, 2020, commonly referred to as "Brexit." Pursuant to the formal withdrawal arrangements agreed between the United Kingdom and the European Union, the United Kingdom was subject to a transition period that ended December 31, 2020, or the Transition Period, during which EU rules continued to apply. A trade and cooperation agreement, or the Trade and Cooperation Agreement, that outlines the future trading relationship between the United Kingdom and the European Union was agreed in December 2020 and has been in effect since January 1, 2022.

Since a significant proportion of the regulatory framework in the United Kingdom applicable to our business and our product candidates is derived from EU directives and regulations, Brexit has had, and may 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 United Kingdom or, to the extent any development of our product candidates takes place in the United Kingdom, the European Union. For example, Great Britain is no longer covered by the centralized procedures for obtaining EU-wide marketing authorization from the EMA, and a separate marketing authorization will be required to market our product candidates in Great Britain. Centralized marketing authorizations continue to allow marketing in Northern Ireland.

While the Trade and Cooperation Agreement provides for the tariff-free trade of medicinal products between the United Kingdom and the European Union there are additional non-tariff costs which did not exist prior to the end of the Transition Period. Further, should the United Kingdom further diverge from the European Union 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 (when compared to the position prior to the end of the Transition Period) to operate our business, which could significantly and materially harm or delay our ability to generate revenues 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 European Union and the United Kingdom.

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

Our operations, and those of our third-party manufacturers, CROs and other contractors and consultants, could be subject to disruptions resulting from earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical pandemic or epidemics, wars and other geopolitical conflicts (including related to Russia's invasion of Ukraine), and other natural or man-made disasters or business interruptions, for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses.

Our corporate headquarters are located in San Diego, California, an area prone to wildfires and earthquakes. These and other natural disasters could severely disrupt our operations, and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as the manufacturing facilities of our third-party contract manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. Any disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which, could have a material adverse effect on our business.

In addition, we rely on third-party manufacturers, some of whom are located in China, to manufacture API for certain of our product candidates, including sparsentan. Any disruption in production or inability of our manufacturers in China to produce or ship adequate quantities to meet our needs, whether as a result of a natural disaster or other causes (such as the COVID-19 pandemic), could impair our ability to operate our business on a day-to-day basis and to continue our research and development of our product candidates. In addition, we are exposed to the possibility of product supply disruption and increased costs in the event of changes in the policies of the United States or Chinese governments (such as tariffs on chemical intermediates we use that are manufactured in China), political unrest or unstable economic conditions in China. Any recall of the manufacturing lots or similar action regarding our API used in clinical trials could delay the trials or detract from the integrity of the trial data and its potential use in future regulatory filings. In addition, manufacturing interruptions or failure to comply with regulatory requirements by any of these manufacturers could significantly delay clinical development of potential products and reduce third-party or clinical researcher interest and support of proposed trials. These interruptions or failures could also impede commercialization of our product candidates and impair our competitive position.

Risks Related to our Indebtedness and Investments

* Our indebtedness could adversely affect our financial condition.

As of March 31, 2022, we had approximately $385 million of total debt outstanding, classified as long term. As a result of our indebtedness, a portion of our cash flow will be required to pay interest and principal on the 2025 Notes and 2029 Notes if the notes are not converted to shares of common stock prior to maturity. We may not generate sufficient cash flow from operations or have future borrowings available to enable us to repay our indebtedness or to fund other liquidity needs.

Our indebtedness pursuant to the 2025 Notes and 2029 Notes could have important consequences. For example, it could:

- make it more difficult for us to satisfy our obligations with respect to any other debt we may incur in the future;
- increase our vulnerability to general adverse economic and industry conditions;
- require us to dedicate a substantial portion of our cash flow from operations to payments on our indebtedness and related interest, thereby reducing the availability of our cash flow to fund working capital, capital expenditures and other general corporate purposes;
- limit our flexibility in planning for, or reacting to, changes in our business and the industry in which we operate;
- increase our cost of borrowing;
- place us at a competitive disadvantage compared to our competitors that may have less debt; and
- limit our ability to obtain additional financing for working capital, capital expenditures, acquisitions, debt service requirements or general corporate purposes.

We expect to use cash flow from operations and outside financings to meet our current and future financial obligations, including funding our operations, debt service and capital expenditures. Our ability to make these payments depends on our future performance, which will be affected by financial, business, economic and other factors, many of which we cannot control. Our business may not generate sufficient cash flow from operations in the future, which could result in our being unable to repay indebtedness, or to fund other liquidity needs. If we do not generate sufficient cash from operations, we may be forced to reduce or delay our business activities and capital expenditures, sell assets, obtain additional debt or equity capital or restructure or refinance all or a portion of our debt, including the 2025 Notes and 2029 Notes, on or before maturity. We cannot make any assurances that we will be able to accomplish any of these alternatives on terms acceptable to us, or at all. In addition, the terms of existing or future indebtedness may limit our ability to pursue any of these alternatives.

* We may be unable to raise the funds necessary to repurchase the 2025 Notes and 2029 Notes for cash following a fundamental change, or to pay any cash amounts due upon conversion, and our future indebtedness may limit our ability to repurchase the 2025 Notes and 2029 Notes or pay cash upon their conversion.

Noteholders may require us to repurchase their 2025 Notes and 2029 Notes following a fundamental change at a cash repurchase price generally equal to the principal amount of the 2025 Notes and 2029 Notes to be repurchased, plus accrued and unpaid interest to, but excluding, the fundamental change repurchase date. In addition, upon conversion, we would satisfy part or all of our conversion obligation in cash unless we elected to settle conversions solely in shares of our common stock.

We may not have enough available cash or be able to obtain financing at the time we are required to repurchase the 2025 Notes and 2029 Notes or pay the cash amounts due upon conversion of the 2025 Notes and 2029 Notes. In addition, applicable law, regulatory authorities and the agreements governing our future indebtedness may restrict our ability to repurchase the 2025 Notes and 2029 Notes or pay the cash amounts due upon conversion of the 2025 Notes and 2029 Notes. Our failure to repurchase the 2025 Notes and 2029 Notes or to pay the cash amounts due upon conversion of the 2025 Notes and 2029 Notes when required will constitute a default under the base and supplemental indentures that will govern the 2025 Notes and 2029 Notes, which we refer to collectively as the “indenture.” We may not have sufficient funds to satisfy all amounts due under the other indebtedness and the 2025 Notes and 2029 Notes.

* A default under the 2025 Notes or 2029 Notes may have a material adverse effect on our financial condition.

If an event of default under the 2025 Notes or 2029 Notes occurs, the principal amount of the 2025 Notes or the 2029 Notes, as applicable, plus accrued and unpaid interest (including additional interest, if any) may be declared immediately due and payable, subject to certain conditions set forth in the indenture governing such notes. Events of default include, but are not limited to:

- failure to pay (for more than 30 days) interest when due;
- failure to pay principal when due;
- failure to deliver shares of common stock upon conversion of a 2025 Note or 2029 Note;
- failure to provide notice of a fundamental change;
- acceleration on our other indebtedness in excess of $10 million (other than indebtedness that is non-recourse to us); or
- certain types of bankruptcy or insolvency involving us.

Accordingly, the occurrence of a default under the 2025 Notes or 2029 Notes, unless cured or waived, may have a material adverse effect on our results of operations.

*** Provisions of the 2025 Notes and 2029 Notes could discourage an acquisition of us by a third party.**

Certain provisions of the 2025 Notes and 2029 Notes could make it more difficult or more expensive for a third party to acquire us. Upon the occurrence of certain transactions constituting a fundamental change, holders of the 2025 Notes and 2029 Notes will have the right, at their option, to require us to repurchase all of their 2025 Notes and 2029 Notes or any portion of the principal amount of such Notes in integral multiples of $1,000. We may also be required to increase the conversion rate for conversions in connection with certain fundamental changes.

*** Conversion of the Notes may dilute the ownership interest of existing stockholders, including holders who had previously converted their 2025 Notes or 2029 Notes.**

To the extent we issue shares of common stock upon conversion of the 2025 Notes or 2029 Notes, the conversion of some or all of the 2025 Notes or 2029 Notes will dilute the ownership interests of existing stockholders. Any sales in the public market of shares of the common stock issuable upon such conversion could adversely affect prevailing market prices of shares of our common stock. In addition, the existence of the 2025 Notes and 2029 Notes may encourage short selling by market participants because the conversion of the 2025 Notes and 2029 Notes could depress the price of shares of our common stock.

General Risk Factors

*** Unstable market, economic and geopolitical conditions, including as a result of the ongoing COVID-19 pandemic, Russia's invasion of Ukraine and global geopolitical tension, may have serious adverse consequences on our business, financial condition and stock price.**

The global credit and financial markets have experienced extreme volatility and disruptions, including as a result of the ongoing COVID-19 pandemic, Russia's invasion of Ukraine and global geopolitical tension, and may experience disruptions in the future. These disruptions can result in severely diminished liquidity and credit availability, increase in inflation, declines in consumer confidence, declines in economic growth, increases in unemployment rates and uncertainty about economic stability. There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. Our general business strategy may be adversely affected by any such economic downturn, volatile business environment, higher inflation, or continued unpredictable and unstable market conditions. If the current equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our operations, growth strategy, financial performance and stock price and could require us to delay or abandon clinical development plans. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may not survive an economic downturn or rising inflation, which could directly affect our ability to attain our operating goals on schedule and on budget.

Other international and geopolitical events could also have a serious adverse impact on our business. For instance, in February 2022, Russia initiated military action against Ukraine. In response, the United States and certain other countries imposed significant sanctions and trade actions against Russia and could impose further sanctions, trade restrictions, and other retaliatory actions. While we cannot predict the broader consequences, the conflict and retaliatory and counter-retaliatory actions could materially adversely affect global trade, currency exchange rates, inflation, regional economies, and the global economy, which in turn may increase our costs, disrupt our supply chain, impair our ability to raise or access additional capital when needed on acceptable terms, if at all, or otherwise adversely affect our business, financial condition, and results of operations.

*** Our business could be negatively impacted by environmental, social and corporate governance (ESG) matters or our reporting of such matters.**

There is an increasing focus from certain investors, employees, partners, and other stakeholders concerning ESG matters. While we have internal efforts directed at ESG matters and preparations for increased future disclosures, we may be, or be perceived by certain stakeholders to be, as not acting responsibly in connection with these matters, which could negatively impact us. For instance, the SEC has recently proposed ESG reporting requirements, which, if approved, would significantly increase our costs. In addition, we currently do not report our environmental emissions, and lack of reporting or future reporting could result in certain investors declining to invest in our common stock.

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

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

None.

Item 6. Exhibits

(a) Exhibits

3.1	Certificate of Incorporation of the Company (incorporated by reference to Exhibit 3.1 to Amendment No. 2 to the Company's General Form for Registration of Securities on Form 10-12G, filed with the SEC on October 28, 2010).
3.2	Certificate of Amendment of Certificate of Incorporation of the Company (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K, filed with the SEC on June 11, 2015).
3.3	Certificate of Amendment of Certificate of Incorporation of the Company (incorporated by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K, filed with the SEC on November 16, 2020).
3.4	Certificate of Amendment of Certificate of Incorporation of the Company (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K, filed with the SEC on May 18, 2021).
3.5	Amended and Restated Bylaws of the Company (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K, filed with the SEC on November 16, 2020).
3.6	Certificate of Amendment of Bylaws of Travere Therapeutics, Inc., effective June 9, 2021 (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K, filed with the SEC on June 10, 2021).
10.1†	Retirement and Transition Agreement dated April 19, 2022, between the Company and Laura Clague (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on April 20, 2022).
10.2†	Travere Therapeutics, Inc. 2018 Equity Incentive Plan, as amended (incorporated by reference to Exhibit 99.1 to the Company's Current Report on Form 8-K, filed with the SEC on May 12, 2022).
31.1	Chief Executive Officer's Certification pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Chief Financial Officer's Certification pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Chief Executive Officer's Certification pursuant to Section 906 of the Sarbanes Oxley Act of 2002
32.2	Chief Financial Officer's Certification 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	Taxonomy Extension Presentation Linkbase Document
104	The cover page to this Quarterly Report on Form 10-Q has been formatted in Inline XBRL

† Indicates management contract or compensatory plan.

SIGNATURES

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

Date: August 4, 2022

TRAVERE THERAPEUTICS, INC.

By: /s/ Eric M. Dube
Name: Eric M. Dube
Title: Chief Executive Officer

By: /s/ Laura Clague
Name: Laura Clague
Title: Chief Financial Officer

Exhibit 31.1

CERTIFICATION OF CHIEF EXECUTIVE OFFICER

PURSUANT TO EXCHANGE ACT RULE 13a-14(a) OR 15d-14(a)

I, Eric M. Dube, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: August 4, 2022

/s/ Eric M. Dube
Eric M. Dube
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF CHIEF FINANCIAL OFFICER

PURSUANT TO EXCHANGE ACT RULE 13a-14(a) OR 15d-14(a)

I, Laura Clague, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: August 4, 2022

/s/ Laura Clague
Laura Clague
Chief Financial Officer
(Principle Financial Officer)

Exhibit 32.1

CERTIFICATION OF

CHIEF EXECUTIVE OFFICER

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 accompanying Quarterly Report on Form 10-Q of Travere Therapeutics, Inc. (the "Company"), for the period ending June 30, 2022 (the "Report"), the undersigned officer of the Company hereby certifies pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to such officer's knowledge:

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

/s/ Eric M. Dube
Eric M. Dube
Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION OF

CHIEF FINANCIAL OFFICER

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 accompanying Quarterly Report on Form 10-Q of Travere Therapeutics, Inc. (the "Company"), for the period ending June 30, 2022 (the "Report"), the undersigned officer of the Company hereby certifies pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to such officer's knowledge:

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

/s/ Laura Clague
Laura Clague
Chief Financial Officer
(Principal Financial Officer)