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, 2021

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 July 27, 2021 was 60,717,176.

TRAVERE THERAPEUTICS, INC.

Form 10-Q
For the Fiscal Quarter Ended June 30, 2021

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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, 2020 (the "2020 10-K"), 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.
- We may not be able to reach alignment with the FDA regarding a pathway for potential accelerated approval submissions in the U.S.
- 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 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 preliminary 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 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 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 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.
- 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.
- We are dependent on information technology systems, infrastructure and data, which exposes us to data security risks.
- 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 $276.0 million aggregate principal amount of 2.50% Convertible Senior Notes due 2025 ("2025 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 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, 2021	December 31, 2020
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 83,288	$ 84,772
Available-for-sale debt securities, at fair value (amortized cost $439,411, allowance for credit losses of $0 as of June 30, 2021; amortized cost $276,111, allowance for credit losses of $0 as of December 31, 2020)	439,502	276,817
Accounts receivable, net	11,860	15,925
Inventory, net	7,409	7,608
Prepaid expenses and other current assets	7,339	8,143
Tax receivable	400	17,142
Total current assets	549,798	410,407
Property and equipment, net	11,720	9,418
Other non-current assets	34,361	33,489
Intangible assets, net	149,951	153,189
Goodwill	936	936
Total assets	$ 746,766	$ 607,439
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 8,343	$ 12,133
Accrued expenses	62,465	56,793
Other current liabilities	8,869	6,334
Business combination-related contingent consideration, current portion	17,300	17,400
Total current liabilities	96,977	92,660
Convertible debt	220,861	215,339
Other non-current liabilities	43,725	40,527
Business combination-related contingent consideration, less current portion	52,900	47,700
Total liabilities	414,463	396,226
Stockholders' Equity:		
Preferred stock $0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of June 30, 2021 and December 31, 2020	—	—
Common stock $0.0001 par value; 200,000,000 and 100,000,000 shares authorized; 60,710,876 and 52,248,431 issued and outstanding as of June 30, 2021 and December 31, 2020, respectively	6	5
Additional paid-in capital	1,011,692	797,985
Accumulated deficit	(678,754)	(585,875)
Accumulated other comprehensive loss	(641)	(902)
Total stockholders' equity	332,303	211,213
Total liabilities and stockholders' equity	$ 746,766	$ 607,439

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,	
	2021	2020	2021	2020
Net product sales	$ 54,617	$ 48,430	$ 102,024	$ 96,199
Operating expenses:				
Cost of goods sold	1,651	1,494	3,296	2,864
Research and development	51,807	30,790	99,753	61,038
Selling, general and administrative	34,965	34,971	71,743	68,110
Change in fair value of contingent consideration	1,509	4,286	10,096	2,363
Total operating expenses	89,932	71,541	184,888	134,375
Operating loss	(35,315)	(23,111)	(82,864)	(38,176)
Other income (expenses), net:				
Other income (expense), net	216	426	(877)	235
Interest income	988	1,316	1,397	3,291
Interest expense	(4,852)	(4,634)	(10,173)	(9,521)
Total other expense, net	(3,648)	(2,892)	(9,653)	(5,995)
Loss before income taxes	(38,963)	(26,003)	(92,517)	(44,171)
Income tax (expense) benefit	(49)	(65)	(362)	18,911
Net loss	$ (39,012)	$ (26,068)	$ (92,879)	$ (25,260)
Basic and diluted net loss per common share	$ (0.64)	$ (0.58)	$ (1.59)	$ (0.57)
Basic and diluted weighted average common shares outstanding	60,571,259	44,763,843	58,431,770	43,943,370
Comprehensive loss:				
Net loss	$ (39,012)	$ (26,068)	$ (92,879)	$ (25,260)
Foreign currency translation	(227)	(247)	875	(56)
Unrealized gain (loss) on debt securities	(152)	3,146	(614)	729
Comprehensive loss	$ (39,391)	$ (23,169)	$ (92,618)	$ (24,587)

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,	
	2021	**2020**
Cash Flows From Operating Activities:		
Net loss	$ (92,879)	$ (25,260)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	12,694	11,507
Non-cash interest expense	759	893
Amortization of discounts/premiums on investments, net	660	337
Amortization of debt discount and issuance costs	5,522	5,147
Provision for inventory	962	662
Share-based compensation	14,767	11,474
ESPP expense	437	390
Change in fair value of contingent consideration	10,096	2,363
Payments related to change in fair value of contingent consideration	(3,602)	(8,674)
Other	1,807	274
Changes in operating assets and liabilities:		
Accounts receivable	4,056	3,979
Inventory	(764)	(875)
Tax receivable	17,011	(18,714)
Other current and non-current operating assets	(1,224)	(2,508)
Change in lease assets and liabilities, net	5,492	—
Accounts payable and accrued expenses	4,681	(16,407)
Other current and non-current operating liabilities	(453)	(196)
Net cash used in operating activities	(19,978)	(35,608)
Cash Flows From Investing Activities:		
Purchase of fixed assets	(4,598)	(518)
Cash paid for intangible assets	(8,979)	(8,532)
Proceeds from the sale/maturity of debt securities	242,064	153,146
Purchase of debt securities	(406,000)	(36,743)
Net cash provided by (used in) investing activities	(177,513)	107,353
Cash Flows From Financing Activities:		
Payment of acquisition-related contingent consideration	(1,399)	(6,101)
Payment of guaranteed minimum royalty	(1,050)	(1,050)
Proceeds from exercise of stock options	3,074	431
Proceeds from the issuance of common stock in At-the-Market equity offering	4,878	—
Proceeds from the issuance of common stock, net of issuance costs	189,278	108,644
Proceeds from issuances under employee stock purchase plan	1,275	1,098
Net cash provided by financing activities	196,056	103,022
Effect of exchange rate changes on cash	(49)	(33)
Net change in cash and cash equivalents	(1,484)	174,734
Cash and cash equivalents, beginning of year	84,772	62,436
Cash and cash equivalents, end of period	$ 83,288	$ 237,170

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, 2021						Three Months Ended June 30, 2020					
	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount					Shares	Amount				
Balance - March 31	**60,435,730**	**$ 6**	**$1,002,687**	**$ (262)**	**$ (639,742)**	**$ 362,689**	**43,153,215**	**$ 4**	**$ 642,880**	**$ (1,500)**	**$ (415,636)**	**$ 225,748**
Share based compensation			7,288			7,288			5,760			5,760
Issuance of common stock under the equity incentive plan and proceeds from exercise	176,259		319			319	177,115		371			371
Equity offering, net of issuance costs			(98)			(98)	7,475,000	1	108,643			108,644
Unrealized gain (loss) on debt securities				(152)		(152)				3,146		3,146
Foreign currency translation adjustments				(227)		(227)				(247)		(247)
ESPP stock purchase and expense	98,887		1,496			1,496	97,544		1,291			1,291
Net loss					(39,012)	(39,012)					(26,068)	(26,068)
Balance - June 30	**60,710,876**	**$ 6**	**$1,011,692**	**$ (641)**	**$ (678,754)**	**$ 332,303**	**50,902,874**	**$ 5**	**$ 758,945**	**$ 1,399**	**$ (441,704)**	**$ 318,645**

	Six Months Ended June 30, 2021						Six Months Ended June 30, 2020					
	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount					Shares	Amount				
Balance - December 31	**52,248,431**	**$ 5**	**$ 797,985**	**$ (902)**	**$ (585,875)**	**$ 211,213**	**43,088,921**	**$ 4**	**$ 636,910**	**$ 726**	**$ (416,444)**	**$ 221,196**
Share based compensation			14,767			14,767			11,474			11,474
Issuance of common stock under the equity incentive plan and proceeds from exercise	646,872		3,074			3,074	241,409		431			431
Equity offering, net of issuance costs	7,716,686	1	194,156			194,157	7,475,000	1	108,643			108,644
Unrealized gain (loss) on debt securities				(614)		(614)				729		729
Foreign currency translation adjustments				875		875				(56)		(56)
ESPP stock purchase and expense	98,887		1,710			1,710	97,544		1,487			1,487
Net loss					(92,879)	(92,879)					(25,260)	(25,260)
Balance - June 30	**60,710,876**	**$ 6**	**$1,011,692**	**$ (641)**	**$ (678,754)**	**$ 332,303**	**50,902,874**	**$ 5**	**$ 758,945**	**$ 1,399**	**$ (441,704)**	**$ 318,645**

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, 2021, 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, also known as RE-021, is an investigational product candidate with a dual mechanism of action, selective endothelin receptor antagonist (“ERA”), with in vitro selectivity toward endothelin receptor type A, and a potent angiotensin receptor blocker (“ARB”). Sparsentan is currently being evaluated in two pivotal Phase 3 clinical studies in the following indications:

- **Focal segmental glomerulosclerosis ("FSGS")** is a rare kidney disease characterized by proteinuria where the glomeruli become progressively scarred. FSGS is a leading cause of end-stage renal disease.
- **Immunoglobulin A nephropathy ("IgAN")** is an immune-complex-mediated glomerulonephritis characterized by hematuria, proteinuria, and variable rates of progressive renal failure. IgAN is the most common primary glomerular disease.

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 issues. Pegtibatinase (TVT-058) is currently being tested in a Phase 1/2 double blind, randomized, placebo-controlled dose escalation study to assess its safety, tolerability, pharmacokinetics, pharmacodynamics and clinical effects in patients with classical HCU. Pegtibatinase (TVT-058) has been granted Rare Pediatric Disease and Fast Track designations by the FDA, as well as orphan drug designation in the United States and Europe. We acquired pegtibatinase (TVT-058) 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. 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:

The Company is 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, 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 the 2020 10-K filed with the SEC on March 1, 2021. 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, 2020 balance sheet information was derived from the audited financial statements as of that date.

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 Topic 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 for further discussion.

Research and Development Expenses

Research and development expenses are comprised of salaries and bonuses, benefits, non-cash share-based compensation, license fees, costs paid to third-party contractors to perform research, conduct clinical trials and pre/non-clinical trials, develop drug materials, and associated overhead expenses and facilities. We also incur 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

Our clinical trials are conducted pursuant to 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, initiation activities, enrollment, 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), we adjust our accruals accordingly on a prospective basis. Revisions to our contractual payment obligations are charged to expense in the period in which the facts that give rise to the revision become reasonably certain.

We currently have 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 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 royalties in excess of the annual contractual minimum at June 30, 2021, as such royalties are not yet probable and estimable.

Recently Issued Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the FASB or other standard setting bodies. Unless otherwise discussed, the Company believes that the impact of recently issued standards that are not yet effective will not have a material impact on its consolidated financial position or results of operations upon adoption.

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 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 Company's assessment of the impact of the new standard on the Company's financial statements is ongoing.

NOTE 3. REVENUE RECOGNITION

Product Revenue, 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 more than 99% of the revenue generated in North America.

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, payors 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 payors 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 payors. 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 revenues for the three and six months ended June 30, 2021 and 2020 (*in thousands*):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Bile acid products	$ 24,974	$ 21,573	$ 46,938	$ 43,854
Tiopronin products	29,643	26,857	55,086	52,345
Total net product revenue	$ 54,617	$ 48,430	$ 102,024	$ 96,199

NOTE 4. DEBT SECURITIES

The Company's debt securities as of June 30, 2021 and December 31, 2020 were comprised 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 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, 2021, investment activity for the Company included $242.1 million in maturities and $406.0 million in purchases, all relating to debt based marketable securities.

Debt securities consisted of the following (*in thousands*):

	June 30, 2021	December 31, 2020
Commercial paper	$ 178,837	$ 135,145
Corporate debt securities	226,351	98,646
Securities of government sponsored entities	34,314	43,026
Total debt securities	$ 439,502	$ 276,817

The following is a summary of short-term debt securities classified as available-for-sale as of June 30, 2021 (*in thousands*):

	Remaining Contractual Maturity (in years)	Amortized Cost	Unrealized Gains	Unrealized Losses	Aggregate Estimated Fair Value
Commercial paper	Less than 1	$ 178,865	$ 3	$ (31)	$ 178,837
Corporate debt securities	Less than 1	117,411	212	(29)	117,594
Securities of government-sponsored entities	Less than 1	31,824	—	(5)	31,819
Total maturity less than 1 year		328,100	215	(65)	328,250
Corporate debt securities	1 to 2	108,811	6	(60)	108,757
Securities of government-sponsored entities	1 to 2	2,500	—	(5)	2,495
Total maturity 1 to 2 years		111,311	6	(65)	111,252
Total available-for-sale securities		$ 439,411	$ 221	$ (130)	$ 439,502

The following is a summary of short-term debt securities classified as available-for-sale as of December 31, 2020 (*in thousands*):

	Remaining Contractual Maturity (in years)	Amortized Cost	Unrealized Gains	Unrealized Losses	Aggregate Estimated Fair Value
Commercial paper	Less than 1	$ 135,161	$ 1	$ (17)	$ 135,145
Corporate debt securities	Less than 1	92,906	723	—	93,629
Securities of government-sponsored entities	Less than 1	43,031	—	(5)	43,026
Total maturity less than 1 year		271,098	724	(22)	271,800
Corporate debt securities	1 to 2	5,013	4	—	5,017
Total available-for-sale securities		$ 276,111	$ 728	$ (22)	$ 276,817

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 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 debt securities in an unrealized loss position with no credit losses reported as of June 30, 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	$ 132,356	$ 31	$ —	$ —	$ 132,356	$ 31
Corporate debt securities	142,673	89	—	—	142,673	89
Securities of government-sponsored entities	7,565	10	—	—	7,565	10
Total	$ 282,594	$ 130	$ —	$ —	$ 282,594	$ 130

The following is a summary of available-for-sale debt securities in an unrealized loss position with no credit losses reported as of December 31, 2020 (*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	$ 112,148	$ 17	$ —	$ —	$ 112,148	$ 17
Corporate debt securities	—	—	—	—	—	—
Securities of government-sponsored entities	43,026	5	—	—	43,026	5
Total	$ 155,174	$ 22	$ —	$ —	$ 155,174	$ 22

As of June 30, 2021 and December 31, 2020, 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 Company does not believe the unrealized losses incurred during the period are due to credit-related factors. Liquidity issues that arose from economic circumstances surrounding the COVID-19 pandemic have continued to ease and unrealized losses observed in early 2020 have been substantially recovered. The credit ratings of the securities held remain of the highest quality, and while certain securities in the portfolio may be downgraded momentarily, the Federal Reserve has allowed institutions to continue to issue debt where there is need, with the government itself purchasing such securities. 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 5. ACQUISITIONS AND DISPOSITIONS

Acquisition of Orphan Technologies Limited

In November 2020, the Company completed the acquisition of Orphan Technologies Limited ("Orphan"), including Orphan's rare metabolic disorder drug OT-58, renamed pegtibatinase (TVT-058). The Company acquired Orphan by purchasing all of its outstanding shares. In exchange for the shares, the Company made an upfront cash payment at closing of $90.0 million plus closing adjustments, net liabilities assumed, and transaction expenses of $1.2 million, $1.8 million, and $4.2 million, respectively. Under the Agreement, the Company has also agreed to make contingent cash payments up to an aggregate of $427.0 million based on the achievement of certain development, regulatory and commercialization events as set forth in the Agreement, as well as additional tiered mid-single digit royalty payments based upon future net sales of any pegtibatinase (TVT-058) products in the US and Europe, subject to certain reductions as set forth in the Agreement, and a contingent payment in the event a pediatric rare disease voucher for any pegtibatinase (TVT-058) product is granted.

The Company has applied the principles of ASC 805 in determining the proper accounting treatment for the acquisition. Substantially all of the value of the assets acquired is concentrated within pegtibatinase (TVT-058), and as of the acquisition date, the Company does not anticipate any economic benefit to be derived from pegtibatinase (TVT-058) other than the primary indication. Accordingly, the transaction is treated as an asset acquisition with amounts charged to expense for the acquired in-process research and development on the date of acquisition.

In accordance with ASC 450, contingent cash payments will be accrued for when it is probable that a liability has been incurred and the amount can be reasonably estimated. As of June 30, 2021, no contingent cash payments have been accrued.

NOTE 6. LEASES

As of June 30, 2021, 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, 2021 (*in thousands*):

	June 30, 2021
2021	$ 1,842
2022	6,020
2023	6,200
2024	6,386
2025	6,578
Thereafter	18,535
Total undiscounted future minimum payments	45,561
Lease incentives payable by lessor	(566)
Present value discount	(9,456)
Total lease liability	35,539
Unamortized lease incentives, less incentives payable by lessor	(6,485)
Cash payments in excess of straight-line lease expense	(4,638)
Total ROU asset	$ 24,416

As of June 30, 2021, the ROU asset of $24.4 million was recorded to the Condensed Consolidated Balance Sheets as non-current Other Assets.

As of June 30, 2021, the current and non-current portions of the lease liability were recorded to the Condensed Consolidated Balance Sheets as follows (*in thousands*):

	June 30, 2021
Other current liabilities	$ 2,623
Other non-current liabilities	32,916
Total lease liabilities	$ 35,539

For the three and six months ended June 30, 2021, the Company recorded $1.2 million and $2.4 million in expense related to operating leases, including amortized tenant improvement allowances. For the three and six months ended June 30, 2020, the Company recorded a credit to expense of $0.2 million and zero in expense related to operating leases, including amortized tenant improvement allowances.

NOTE 7. 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, 2021 and December 31, 2020 are as follows:

	Revenue Discount		Payment Discount
	Cholbam	Chenodal	
June 30, 2021	6.5%	7.5%	7.85%
December 31, 2020	6.5%	8.5%	7.45%

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, 2021, the fair value of the Company's 2.5% Convertible Senior Notes due 2025 was $234.7 million, which was 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, 2021 (*in thousands*):

	As of June 30, 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	$ 83,288	$ 83,288	$ —	$ —
Debt securities, available-for-sale	439,502	—	439,502	—
Total	$ 522,790	$ 83,288	$ 439,502	$ —
Liabilities:				
Business combination-related contingent consideration	$ 70,200	$ —	$ —	$ 70,200
Total	$ 70,200	$ —	$ —	$ 70,200

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, 2020 (*in thousands*):

	As of December 31, 2020			
	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	$ 84,772	$ 84,772	$ —	$ —
Debt securities, available-for-sale	276,817	—	276,817	—
Total	$ 361,589	$ 84,772	$ 276,817	$ —
Liabilities:				
Business combination-related contingent consideration	65,100	—	—	65,100
Total	$ 65,100	$ —	$ —	$ 65,100

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, 2021 (*in thousands*):

	Fair Value Measurements of Acquisition-Related Contingent Consideration (Level 3)
Balance at January 1, 2021	$ 65,100
Changes in the fair value of contingent consideration	10,096
Contractual payments	(2,276)
Contractual payments included in accrued liabilities at June 30, 2021	(2,606)
Foreign currency impact	(114)
Balance at June 30, 2021	$ 70,200

For the three and six months ended June 30, 2021, the Company incurred charges of $1.5 million and $10.1 million in operating expenses on the Condensed Consolidated Statements of Operations and Comprehensive Loss for the change in fair value of the contingent consideration liabilities.

For the three and six months ended June 30, 2021, the change in fair value of contingent consideration is due to the timing of future payments and changes in market driven discount rates.

For the three and six months ended June 30, 2020, the Company incurred charges of $4.3 million and $2.4 million in operating expenses on the Condensed Consolidated Statements of Operations and Comprehensive Loss for the change in fair value of the contingent consideration liabilities. The value changed due to the timing of future payments and changes in market driven discount rates.

NOTE 8. INTANGIBLE ASSETS

As of June 30, 2021, the net book value of amortizable intangible assets was approximately $150.0 million.

The following table sets forth amortizable intangible assets as of June 30, 2021 and December 31, 2020 (*in thousands*):

	June 30, 2021	December 31, 2020
Finite-lived intangible assets	$ 273,332	$ 264,676
Less: accumulated amortization	(123,381)	(111,487)
Net carrying value	$ 149,951	$ 153,189

The following table summarizes amortization expense for the three and six months ended June 30, 2021 and 2020 (*in thousands*):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Research and development	$ 288	$ 289	$ 574	$ 578
Selling, general and administrative	5,848	4,996	11,514	9,881
Total amortization expense	$ 6,136	$ 5,285	$ 12,088	$ 10,459

NOTE 9. CONVERTIBLE NOTES PAYABLE

Convertible Senior Notes Due 2025

On September 10, 2018, the Company completed its 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 ("Maturity Date"), 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 composition of the Company's 2025 Notes are as follows (*in thousands*):

	June 30, 2021	December 31, 2020
2.50% convertible senior notes due 2025	$ 276,000	$ 276,000
Unamortized debt discount	(51,314)	(56,384)
Unamortized debt issuance costs	(3,825)	(4,277)
Total 2025 Notes, net of unamortized debt discount and debt issuance costs	$ 220,861	$ 215,339

The net proceeds from the issuance of the 2025 Notes were approximately $267.2 million, after deducting commissions and the offering expenses payable by the Company. A portion of the net proceeds from the 2025 Notes was used by the Company to repurchase $23.4 million aggregate principal amount of its then-outstanding 4.5% senior convertible notes due 2019 in privately-negotiated transactions.

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.

As of June 30, 2021, the 2025 Notes had a market price of $851 per $1,000 or $234.7 million principal amount. 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 $276.0 million 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 2025 Notes are the Company's general unsecured obligations that rank senior in right of payment to all of its indebtedness that is expressly subordinated in right of payment to the 2025 Notes, and equal in right of payment to the Company's unsecured indebtedness.

The 2025 Notes are classified on the Company's Condensed Consolidated Balance Sheets at June 30, 2021 as long-term convertible debt.

Under ASC 470-20, Debt with Conversion and Other Options, an entity must separately account for the liability and equity components of convertible debt instruments (such as the 2025 Notes) that may be settled entirely or partially in cash upon conversion, in a manner that reflects the issuer's economic interest cost. The liability component of the instrument is valued in a manner that reflects the market interest rate for a similar nonconvertible instrument at the date of issuance. The initial carrying value of the liability component was $198.6 million. The equity component of $77.4 million, representing the conversion option, was determined by deducting the fair value of the liability component from the par value of the 2025 Notes and was recorded in additional paid-in capital on the consolidated balance sheet at the issuance date. That equity component is treated as a discount on the liability component of the 2025 Notes, which is amortized over the seven-year term of the 2025 Notes using the effective interest rate method. The equity component is not re-measured as long as it continues to meet the conditions for equity classification. The Company allocated the total transaction costs of approximately $8.8 million related to the issuance of the 2025 Notes to the liability and equity components of the 2025 Notes based on their relative values. Transaction costs attributable to the liability component are amortized to interest expense over the seven-year term of the 2025 Notes, and transaction costs attributable to the equity component are netted with the equity component in stockholders' equity.

The effective interest rate on the liability components of the 2025 Notes for the period from the date of issuance through June 30, 2021 was 7.7%. The following table sets forth total interest expense recognized related to the 2025 Notes (*in thousands*):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Contractual interest expense	$ 1,725	$ 1,725	$ 3,450	$ 3,450
Amortization of debt discount	2,560	2,371	5,071	4,698
Amortization of debt issuance costs	226	225	451	449
Total interest expense for the 2025 Notes	$ 4,511	$ 4,321	$ 8,972	$ 8,597

The 2025 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. The 2025 Indenture contains customary events of default with respect to the 2025 Notes, including that upon certain events of default, 100% of the principal and accrued and unpaid interest on the 2025 Notes will automatically become due and payable.

NOTE 10. ACCRUED EXPENSES

Accrued expenses at June 30, 2021 and December 31, 2020 consisted of the following (*in thousands*):

	June 30, 2021	December 31, 2020
Government rebates payable	$ 11,756	$ 10,707
Compensation related costs	12,656	17,912
Accrued royalties and contingent consideration	8,340	7,857
Research and development	21,534	10,166
Selling, general and administrative	4,541	3,944
Miscellaneous accrued	3,638	6,207
Total accrued expenses	$ 62,465	$ 56,793

NOTE 11. 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, 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,					
	2021			2020		
	Shares	Net Loss	Loss per common share	Shares	Net Loss	Loss per common share
Basic and diluted loss per share	60,571,259	$ (39,012)	$ (0.64)	44,763,843	$ (26,068)	$ (0.58)

	Six Months Ended June 30,					
	2021			2020		
	Shares	Net Loss	Loss per common share	Shares	Net Loss	Loss per common share
Basic and diluted loss per share	58,431,770	$ (92,879)	$ (1.59)	43,943,370	$ (25,260)	$ (0.57)

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Restricted stock	1,591,426	1,517,338	1,614,123	1,437,383
Convertible debt	7,113,402	7,113,402	7,113,402	7,113,402
Options	9,367,565	8,479,710	9,298,480	8,327,753
Total anti-dilutive shares	18,072,393	17,110,450	18,026,005	16,878,538

NOTE 12. COMMITMENTS AND CONTINGENCIES

Research Collaboration and Licensing Agreements

As part of the Company's research and development efforts, the Company enters into research collaboration and licensing agreements with unrelated companies, scientific collaborators, universities, and consultants. These agreements contain varying terms and provisions which include fees and milestones to be paid by the Company, services to be provided, and ownership rights to certain proprietary technology developed under the agreements. Some of these agreements contain provisions which require the Company to pay royalties, in the event the Company sells or licenses any proprietary products developed under the respective agreements.

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 13. SHARE-BASED COMPENSATION

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, 2021:

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Unvested December 31, 2020	1,109,942	$ 17.84
Granted	800,797	25.49
Vested	(365,492)	17.96
Forfeited/canceled	(77,784)	21.42
Unvested June 30, 2021	1,467,463	$ 21.80

At June 30, 2021, unamortized stock compensation for service based restricted stock units was $28.0 million, with a weighted-average recognition period of 2.8 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, 2021:

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Unvested December 31, 2020	167,500	$ 16.48
Granted	—	—
Vested	(75,000)	15.46
Forfeited/canceled	—	—
Unvested June 30, 2021	92,500	$ 17.31

At June 30, 2021, unamortized stock compensation for performance based restricted stock units was less than $0.1 million, with a weighted-average recognition period of 0.7 years.

Stock Options

The following table summarizes stock option activity during the six months ended June 30, 2021:

	Shares Underlying Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2020	8,242,996	$18.97	6.43	$ 71,641
Granted	1,518,307	26.36		
Exercised	(206,380)	14.90		
Forfeited/canceled	(225,803)	22.98		
Outstanding at June 30, 2021	9,329,120	$20.17	6.42	$ 4,922

At June 30, 2021, unamortized stock compensation for stock options was $37.2 million, with a weighted-average recognition period of 2.7 years.

At June 30, 2021, outstanding options to purchase 6.2 million shares of common stock were exercisable with a weighted-average exercise price per share of $19.23.

Share-Based Compensation

The following table sets forth total share-based compensation for the three and six months ended June 30, 2021 and 2020 (*in thousands*):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Research and development	$ 2,845	$ 2,332	$ 5,847	$ 4,458
Selling, general & administrative	4,665	3,622	9,357	7,406
Total	$ 7,510	$ 5,954	$ 15,204	$ 11,864

NOTE 14. INCOME TAXES

For the six months ended June 30, 2021, we recognized an income tax expense of $0.4 million as compared to an income tax benefit of $18.9 million for the six months ended June 30, 2020. The change is primarily related to provisions of the Coronavirus Aid, Relief, and Economic Security Act ("CARES Act") concerning net operating loss carrybacks which were effected in 2020.

NOTE 15. INVENTORY

Inventory, net of reserves, consisted of the following at June 30, 2021 and December 31, 2020 (*in thousands*):

	June 30, 2021	December 31, 2020
Raw materials	$ 4,099	$ 3,219
Finished goods	3,310	4,389
Total inventory	$ 7,409	$ 7,608

The inventory reserve was $3.5 million and $3.6 million at June 30, 2021 and December 31, 2020, respectively.

NOTE 16. ACCOUNTS RECEIVABLE

Accounts receivable, net of reserves for prompt pay discounts and expected credit losses, was $11.9 million and $15.9 million at June 30, 2021 and December 31, 2020, 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 17. EQUITY OFFERINGS

Underwritten Public Offering of Common Stock

In June 2020, the Company sold an aggregate of 7.5 million shares of its common stock in an underwritten public offering, at a price of $15.50 per share. The net proceeds to the Company from the offering, after deducting the underwriting discounts and offering expenses, were $108.7 million.

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. Shares will be sold pursuant to the Company's effective registration statement on Form S-3 (Registration Statement No. 333-227182), as previously filed with the Securities and Exchange Commission. Through June 30, 2021, the Company has sold a total of 1,051,992 shares under the ATM Agreement, resulting in net proceeds of $28.6 million. $4.9 million of this total relates to the settlement of 184,186 shares in the first quarter of 2021. As of June 30, 2021, an aggregate of $71.4 million remained eligible for sale under the facility.

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, 2020 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, 2020, filed with the Securities and Exchange Commission (SEC) on March 1, 2021. 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 three and six months ended June 30, 2021, we have been monitoring the developments and assessing areas where there is potential for our business to be impacted. As of June 30, 2021, the majority of our labor force is still working remotely, 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. 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 could impact our ability to enter into, modify, and negotiate favorable terms and conditions relative to equity and debt financing activities. We had $522.8 million in cash and cash equivalents and available-for-sale securities as of June 30, 2021, 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, also known as RE-021, is a novel investigational product candidate designed with a dual mechanism of action, as a selective endothelin receptor antagonist (“ERA”), that has shown in vitro selectivity toward endothelin receptor type A and as a potent angiotensin receptor blocker, in a single molecule. Sparsentan is currently being evaluated in two pivotal Phase 3 clinical studies in rare kidney diseases, including:

- **Focal segmental glomerulosclerosis ("FSGS")**, a leading cause of end-stage kidney disease ("ESKD") and nephrotic syndrome. There are currently no United States Food and Drug Administration ("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. Sparsentan has orphan drug designation for FSGS in the United States and European Union. 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 to date in the study, sparsentan has been generally well-tolerated and the overall safety results in the study to date 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 the available data from the previously announced interim assessment of the DUPLEX Study would not be adequate to support an accelerated approval in the U.S. at this time. The FDA has indicated that it may be possible to submit an application for accelerated approval in FSGS after additional data accrue in the ongoing DUPLEX Study. We are in the process of continuing our engagement with the FDA with the objective of identifying a future path to providing sufficient additional estimated glomerular filtration (eGFR) data from the DUPLEX Study for a potential accelerated approval submission in the U.S. for sparsentan in FSGS in 2022. We intend to provide a further regulatory update around the FSGS program in the U.S. following these regulatory engagements.

  Based on recent interactions with the European Medicines Agency ("EMA"), we believe we have identified a path for submitting an application for conditional marketing authorization ("CMA") of sparsentan for the treatment of FSGS in Europe, utilizing the existing interim dataset to be supplemented with additional eGFR data during a clock-stop procedure available in the regulatory review process.

- **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 FDA approved treatments for IgAN. 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 patients with IgAN, and the study is fully enrolled. The PROTECT Study protocol provides 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. The interim assessment is designed to support potential submissions under the Subpart H pathway for accelerated approval in the United States and potential CMA consideration in Europe. 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. At this time, top-line data from the 36-week proteinuria endpoint analysis are expected to become available in August 2021 and we are monitoring the potential impact the evolving COVID-19 pandemic may have on this timing. Sparsentan has orphan drug designation for IgAN in the United States and European Union.

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 issues. Pegtibatinase (TVT-058) is currently being tested in a Phase 1/2 double blind, randomized, placebo-controlled dose escalation study to assess its safety, tolerability, pharmacokinetics, pharmacodynamics and clinical effects in patients with classical HCU. At this time, preliminary data from the ongoing Phase 1/2 study are expected to become available in 2021 and we are monitoring the potential impact the evolving COVID-19 pandemic may have on this timing. Pegtibatinase (TVT-058) has been granted Rare Pediatric Disease and Fast Track designations by the FDA, as well as orphan drug designation in the United States and European Union. It is estimated that there are at least 3,500 people living with HCU in the United States with similar numbers in Europe. We acquired pegtibatinase (TVT-058) as part of the November 2020 acquisition of Orphan Technologies Limited ("Orphan").

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, a new 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. We are not able to estimate the impact upon our business at this time.

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, 2021 compared to the three and six months ended June 30, 2020

Net Product Sales

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

	Three Months Ended June 30,			Six Months Ended June 30,		
	2021	2020	Change	2021	2020	Change
Net product revenues by product:						
Bile acid products	$ 24,974	$ 21,573	$ 3,401	$ 46,938	$ 43,854	$ 3,084
Tiopronin products	29,643	26,857	2,786	55,086	52,345	2,741
Total net product revenues	$ 54,617	$ 48,430	$ 6,187	$ 102,024	$ 96,199	$ 5,825

The sales increase for the three and six months ended June 30, 2021 compared to the three and six months ended June 30, 2020 was due to increased patient counts.

Operating Expenses

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

	Three Months Ended June 30,			Six Months Ended June 30,		
	2021	2020	Change	2021	2020	Change
Cost of goods sold	$ 1,651	$ 1,494	$ 157	$ 3,296	$ 2,864	$ 432
Research and development	51,807	30,790	21,017	99,753	61,038	38,715
Selling, general and administrative	34,965	34,971	(6)	71,743	68,110	3,633
Change in fair value of contingent consideration	1,509	4,286	(2,777)	10,096	2,363	7,733
	$ 89,932	$ 71,541	$ 18,391	$ 184,888	$ 134,375	$ 50,513

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, 2021 as compared to the three and six months ended June 30, 2020, our research and development expenses increased by $21.0 million and $38.7 million, respectively, due primarily to increased clinical trial expenses, including those in relation to TVT-058 and the acquisition of Orphan Technologies Limited, as well as increased personnel expenses arising from increased headcount.

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, 2021 as compared to the three and six months ended June 30, 2020, our selling, general and administrative expenses remained flat and increased by $3.6 million, respectively. The increase observed is primarily due to increased personnel expenses arising from increased headcount, and additional professional fees.

Change in the valuation of contingent consideration

For the three months ended June 30, 2021 as compared to the three months ended June 30, 2020, the change in fair value of contingent consideration is due to the passage of time and changes in market driven discount rates.

For the six months ended June 30, 2021 as compared to the six months ended June 30, 2020, the change in fair value of contingent consideration is due to refinements in revenue projections along with the passage of time 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,		
	2021	2020	Change	2021	2020	Change
Other income (expense), net	$ 216	$ 426	$ (210)	$ (877)	$ 235	$ (1,112)
Interest income	988	1,316	(328)	1,397	3,291	(1,894)
Interest expense	(4,852)	(4,634)	(218)	(10,173)	(9,521)	(652)
	$ (3,648)	$ (2,892)	$ (756)	$ (9,653)	$ (5,995)	$ (3,658)

The change in our other income (expenses) for the three and six months ended June 30, 2021 as compared to the three and six months ended June 30, 2020 of $0.8 million and $3.7 million, respectively, is primarily due to lower interest rate yields as compared to prior year, along with changes in foreign exchange rates.

Income Tax Benefit (Provision)

For the six months ended June 30, 2021, we recognized an income tax expense of $0.4 million as compared to an income tax benefit of $18.9 million for the six months ended June 30, 2020. The change is primarily related to provisions of the CARES Act concerning net operating loss carrybacks which were effected in 2020. Under GAAP, quarterly effective tax rates may vary significantly depending on the actual operating results in the various tax jurisdictions, and significant transactions, as well as changes in the valuation allowance related to the expected recovery of deferred tax assets.

At June 30, 2021, we had no unrecognized tax benefits. We did not recognize any interest or penalties related to unrecognized tax benefits during the three and six months ended June 30, 2021.

Liquidity and Capital Resources

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, 2021 and December 31, 2020 (*in thousands*):

	June 30, 2021	December 31, 2020
Cash & Cash Equivalents	$ 83,288	$ 84,772
Debt securities	439,502	276,817
Accumulated Deficit	(678,754)	(585,875)
Stockholders' Equity	332,303	211,213
Net Working Capital*	$ 452,821	$ 317,747
Net Working Capital Ratio**	5.67	4.43

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

Operating Leases

Future Minimum Rental Commitments

We have future minimum rental commitments totaling $45.6 million arising from our operating leases. These commitments consist of $46.6 million in aggregate base rent through August 2028, less rent abatement totaling $1.0 million.

Equity Offerings

2020 Underwritten Public Offering of Common Stock

In June 2020, the Company sold an aggregate of 7.5 million shares of its common stock in an underwritten public offering, at a price to the public of $15.50 per share. The net proceeds to the Company from the offering, after deducting the underwriting discounts and offering expenses, were $108.7 million.

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. Shares will be sold pursuant to the Company's effective registration statement on Form S-3 (Registration Statement No. 333-227182), as previously filed with the Securities and Exchange Commission. Through June 30, 2021, the Company has sold a total of 1,051,992 shares under the ATM Agreement, resulting in net proceeds of $28.6 million. $4.9 million of this total relates to the settlement of 184,186 shares in the first quarter of 2021. As of June 30, 2021, an aggregate of $71.4 million remained eligible for sale under the facility.

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.

Borrowings

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 ("Maturity Date”), unless earlier repurchased, redeemed, or converted. The 2025 Notes are senior unsecured obligations of ours 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 composition of our 2025 Notes are as follows (*in thousands*):

	June 30, 2021	December 31, 2020
2.50% convertible senior notes due 2025	$ 276,000	$ 276,000
Unamortized debt discount	(51,314)	(56,384)
Unamortized debt issuance costs	(3,825)	(4,277)
Total 2025 Notes, net of unamortized debt discount and debt issuance costs	$ 220,861	$ 215,339

The net proceeds from the issuance of the 2025 Notes were approximately $267.2 million, after deducting commissions and the offering expenses payable by us. A portion of the net proceeds from the 2025 Notes were used by us to repurchase $23.4 million aggregate principal amount of our then-outstanding 4.50% Senior Convertible Notes due 2019 ("2019 Notes") in privately-negotiated transactions.

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 (such 10 consecutive trading day period, the “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 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 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 $276.0 million in principal value and any conversion premium in any combination of cash and shares of its common stock (at the Company’s option).

Interest Expense

Total interest expense recognized for the six months ended June 30, 2021 and 2020 was $10.2 million and $9.5 million, respectively.

Cash Flows

Cash Flows from Operating Activities

Cash used in operating activities was $20.0 million for the six months ended June 30, 2021 compared to cash used of $35.6 million for the six months ended June 30, 2020. The change in cash used is attributable to changes in working capital, including a decrease in the tax receivable, and contingent consideration-related payments.

Cash Flows from Investing Activities

Cash used in investing activities for the six months ended June 30, 2021 was $177.5 million, compared to cash provided of $107.4 million for the six months ended June 30, 2020. The change is due to the purchase of available-for-sale investments, net of maturities. Approximately $140.0 million of the funds received in the February 2021 underwritten public offering of common stock were invested in available-for-sale investments.

Cash Flows from Financing Activities

Cash provided by financing activities for the six months ended June 30, 2021 was $196.1 million compared to cash provided by $103.0 million for the six months ended June 30, 2020. The change is due to the underwritten public offering of our common stock in February 2021.

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, if any;
- debt service obligations on the 2025 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.

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.

Off Balance Sheet Arrangements

None.

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, 2021, we had cash equivalents and marketable securities of approximately $447.4 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 $1.9 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, 2021, 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 12 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, 2020, 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. Pursuant to the DUPLEX Study protocol, patients are to continue in a blinded manner to assess the treatment effect on eGFR slope over 108 weeks in the confirmatory endpoint analysis of the study. Given that interim results from the study have been publicly announced, it is possible that we may see a higher than anticipated attrition rate in the study. To the extent that an insufficient number of patients choose to remain in the study for the full 108 weeks, it could jeopardize our ability to complete the study and submit for full regulatory approval for sparsentan in FSGS.

We may not be able to initiate or continue clinical trials in the rare diseases in 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.

*** We may not be able to reach alignment with the FDA regarding a pathway for potential accelerated approval submissions in the U.S.**

In February 2021, we announced that our ongoing pivotal Phase 3 DUPLEX Study of sparsentan in FSGS achieved its pre-specified interim FSGS partial remission of proteinuria endpoint (“FPRE”) after 36 weeks of treatment. 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. In May 2021, we provided a regulatory update regarding the sparsentan FSGS program, including feedback from the FDA that the available data from the previously announced interim assessment of the DUPLEX Study would not be adequate to support an accelerated approval in the U.S. at this time. The FDA has indicated that it may be possible to submit an application for accelerated approval in FSGS after additional data accrue

in the ongoing DUPLEX Study. We are in the process of continuing our engagement with the FDA with the objective of identifying a future path to providing sufficient additional estimated glomerular filtration (eGFR) data from the DUPLEX Study for a potential accelerated approval submission in the U.S. for sparsentan in FSGS in 2022.

In August 2021, we intend to conduct a pre-specified interim assessment of the primary proteinuria endpoint after 36 weeks of treatment in our ongoing pivotal Phase 3 PROTECT Study of sparsentan in IgAN. If the outcome of that assessment, coupled with the totality of the data available at the time, is favorable, then we intend to request a pre-NDA meeting with the FDA to review and discuss whether the data support an NDA submission for accelerated approval under the subpart H accelerated approval pathway.

It is possible that we may be unable to reach alignment with the FDA around a pathway for accelerated approval submissions in FSGS and/or IgAN. In that case, there would be a delay in our submission timeline as we would then instead await results from the confirmatory endpoint before determining whether to submit applications for regulatory approval for one or both of the indications. Also, even if FDA concurs with our plan to submit an NDA for accelerated approval, there is no guarantee that the FDA will accept the NDA for filing, as the FDA has the authority to refuse to file NDAs for a variety of reasons.

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, there can be no assurance that our clinical experience with sparsentan in FSGS will translate to favorable data in IgAN, which patient population has not previously been treated with sparsentan prior to the Phase 3 trial currently being conducted. Similarly, the positive pre-clinical data we have seen from pegtibatinase (TVT-058) being tested in a mouse model of homocystinuria may not be replicated in the ongoing Phase 1/2 clinical trial of pegtibatinase (TVT-058). 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.**

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.

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 are conducting the DUPLEX Study and the PROTECT Study under the Subpart H pathway for potential accelerated approval in the United States, and in Europe we plan to pursue potential Conditional Marketing Authorization, 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. For example, in the DUPLEX Study in conjunction with ongoing FDA dialogue to enable submission for potential approval on the subpart H pathway, in November 2020 we adopted an eGFR measurement over a 108-week period following randomized treatment, to support full approval. However, in May 2021, we received feedback from the FDA that the available data from the previously announced interim assessment of the DUPLEX Study would not be adequate to support an accelerated approval in the U.S. at this time. The FDA has indicated that it may be possible to submit an application for accelerated approval in FSGS after additional data accrue in the ongoing DUPLEX Study, and while we are in the process of continuing our engagement with the FDA with the objective of identifying a future path to providing sufficient additional eGFR data from the DUPLEX Study for a potential accelerated approval submission in the U.S. for sparsentan in FSGS in 2022, additional data from the DUPLEX Study may not be sufficient to support an NDA under Subpart H for accelerated approval.

If the FDA or EMA agree to review our regulatory submissions for accelerated approval/conditional marketing authorization, we expect that the FDA’s and EMA’s determination as to whether the sufficiency of the data from the DUPLEX and PROTECT Studies supports an accelerated approval/conditional marketing authorization 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 DUPLEX Study to be sufficient to grant accelerated approval or Conditional Marketing Authorization for sparsentan for the treatment of FSGS. Similarly, even if we achieve statistical significance on the interim or primary endpoints for the PROTECT Study, there can be no assurance that the FDA or EMA will deem that sufficient to grant accelerated approval or Conditional Marketing Authorization of sparsentan for the treatment of IgAN.

There can be no guarantee that the data generated from the DUPLEX Study will be sufficient to serve as the basis for an NDA filing, or that additional data from the DUPLEX Study will be sufficient to support an NDA under Subpart H for accelerated approval. In addition, our statistical modeling that supports proceeding with the DUPLEX Study on the Subpart H pathway is based on data from other FSGS studies. To the extent that the model population is not representative of the DUPLEX Study population, the FDA may not agree that the new results continue to support a Subpart H pathway. Furthermore, even if sparsentan is granted accelerated approval for FSGS, there can be no assurance that the post-marketing confirmatory data will support full approval of sparsentan as a treatment for FSGS.

Also, although we have reached agreement with the FDA regarding the initiation of the PROTECT Study and the trial began in December 2018, we continue to have regulatory interactions regarding certain details of the study. For example, in conjunction with ongoing FDA dialogue, in May 2020 we adopted a

measurement of the rate of change in eGFR over the 110-week period following the initiation of randomized treatment as the confirmatory endpoint of the study, and increased the total sample size from 280 patients to approximately 380 while maintaining the sample size for the primary endpoint at 280 patients. There can be no assurance that the study will proceed as planned and there can be no guarantee that the data generated from the study will be sufficient to serve as the basis for an NDA filing, including an NDA under Subpart H for accelerated approval or support Conditional Marketing Authorization in the EU. 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.

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.

*** 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 preliminary or future data from the study.**

While we have recently completed enrollment of the currently planned dose cohorts, patient enrollment may be extended in the future to additional dose cohorts in our 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 trial 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 we receive regulatory approval for any product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense.

Any regulatory approvals that we receive for any product candidates 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 the post marketing obligations and commercial acceptance of Cholbam, which was approved by the FDA on March 17, 2015. 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 independent 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 independent 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 independent 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 independent investigators and CROs may also have relationships with other commercial entities, some of which may compete with us. If independent 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 100mg version of the original formulation of Thiola (tiopronin tablets) was approved by the FDA and additional generic alternatives may be approved in the future.

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, 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. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our prospects for generating revenue outside of the United States, if any, could be adversely affected and our business may suffer.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates outside the United States, we may be unable to generate product revenue outside of the United States.

We may not be able to rely on orphan drug exclusivity for Cholbam or any of our products.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. We have obtained orphan designation for Cholbam in the United States, which expires 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 exclusivity for Cholbam in the United States, we

may not be able to maintain it. 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 successfully.

In order to successfully commercialize our products, 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.

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 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. Our efforts to educate patients, the medical community, and third-party payers on the benefits of our product 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 and IgAN, 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 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 in the study populations accurately reflect the prevalence of these diseases in the broader world population. If our estimates of the prevalence of FSGS or IgAN or of the number of patients who may benefit from treatment with sparsentan prove to be incorrect, 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.

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 or its use for treating cystinuria. 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 either 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 addition, we may be able to obtain up to five years patent term extension (to compensate for regulatory approval delay) for a patent covering such a product.

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 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. 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 intend to vigorously defend 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, 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, 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, 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, which began on February 15, 2021 and will remain open through August 15, 2021. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the 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, 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 December 31, 2021. 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 or 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.

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 also released a final rule, effective November 30, 2020, implementing a portion of the importation executive order providing guidance 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 implementation of the rule has been delayed by the Biden administration from January 1, 2022 to January 1, 2023 in response to ongoing litigation. 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 which have also been delayed until January 1, 2023. 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. On December 28, 2020, the United States District Court in Northern California issued a nationwide preliminary injunction against implementation of the interim final rule. On January 13, 2021, in a separate lawsuit brought by industry groups in the U.S. District of Maryland, the government defendants entered a joint motion to stay litigation on the condition that the government would not appeal the preliminary injunction granted in the U.S. District Court for the Northern District of California and that performance for any final regulation stemming from the MFN Model interim final rule shall not commence earlier than 60 days after publication of that regulation in the Federal Register. Based on a recent executive order, the Biden administration expressed its intent to pursue certain policy initiatives to reduce drug prices. 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 for Cholbam 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;
- 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 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. 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, a membrane used in pegtibatinase (TVT-058) drug substance manufacturing has recently become more difficult to acquire due to same or similar membranes being used in certain of the recently initiated COVID-19 vaccine manufacturing. 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.

Risks Related to Our Business

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

The ongoing COVID-19 pandemic is impacting domestic and worldwide economic activity, including global financial markets. The COVID-19 pandemic also poses the risk that we or our clinical trial subjects, employees, contractors, collaborators 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. 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 may 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. Clinical site initiation and patient enrollment may be further delayed due to prioritization of hospital resources toward the COVID-19 pandemic and patients’ ability or willingness to participate in clinical trials. For those patients who are enrolled and desire to continue in the clinical trials, some patients may not be able or willing to comply with clinical trial protocols if quarantines or governmental orders impede patient movement or interrupt healthcare services. Similarly, we may face increased challenges with the ability to recruit and retain patients and principal investigators and site staff who, as healthcare providers, may have heightened exposure to COVID-19, which could adversely impact our clinical trial operations, timelines and outcomes. In addition, we rely on independent clinical investigators, contract research organizations (CROs) and other third-party service providers to assist us in managing, monitoring and otherwise carrying out our clinical trials, and the pandemic may affect their ability to devote sufficient time and resources to our programs or to travel to sites to perform work for us. While we remain in close contact with our CROs,

clinical sites and suppliers to attempt to assess the impacts that COVID-19 may have on our clinical trials and projected timelines and we continue to implement appropriate mitigating measures in accordance with recent 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.

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. 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. 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, and the effectiveness of actions taken in the United States and other countries to contain and treat the disease.

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 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. 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 resulting from the ongoing issues arising from the COVID-19 pandemic, 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;
- debt service obligations on the 2025 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 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;
- 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 12 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.

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"). 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 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 antibribery 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) 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. Beginning in 2022, applicable manufacturers also will be required to report such information regarding payments and transfers of value provided during the previous year to physician assistants, nurse practitioners, clinical nurse specialists, anesthesiologist assistants, certified registered nurse anesthetists and certified nurse midwives.

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 addition, we may be subject to data privacy and security regulation by foreign governments, the federal government, and the states in which we conduct our business. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), and their respective implementing regulations, imposes specified 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. International data protection laws also impose strict obligations on the ability to process health related and other personal information of citizens of member states, including in relation to collection, analysis and transfer.

The EU General Data Protection Regulation ("GDPR") introduced new data protection requirements in the European Union ("EU"), as well as substantial fines for breaches of the data protection rules. The GDPR will increase our responsibility and liability in relation to personal data from the European Economic Area ("EAA") that we control and/or process, and we may be required to put in place additional mechanisms to ensure compliance with the changing EU data protection rules. The 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. Additionally, the GDPR imposes strict rules on the transfer of personal data to countries outside the EEA, including the United States and, in response, the EU and United States agreed in 2016 to a transfer framework for data transferred from the European Union to the United States, called the EU-US Privacy Shield. On July 16, 2020, however, the Court of Justice of the European Union issued a decision that declared the Privacy Shield framework, one of the primary mechanisms U.S. companies used to import personal information from Europe, invalid, and raised questions about whether the European Commission's Standard Contractual Clauses ("SCCs"), an alternative to the Privacy Shield, can lawfully be used for cross-border data transfers. On June 4, 2021, the European Commission adopted new SCCs under the GDPR for personal data transfers outside the EEA. Under this legal mechanism, we may have obligations to conduct transfer impact assessments for such cross-border data transfers and implement additional security measures. 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.

If we are unable to implement a valid compliance mechanism for cross-border personal information transfers, we may face increased exposure to regulatory actions, substantial fines and injunctions against processing or transferring personal information from Europe. Inability to import personal information from Europe to the United States may significantly and negatively impact our business operations, including by limiting our ability to conduct clinical trials activities in Europe; limiting our ability to collaborate with CROs, service providers, contractors and other companies subject to European data protection laws; or requiring us to increase our data processing capabilities in Europe at significant expense.

Further, the vote in the United Kingdom in favor of exiting the European Union, referred to as Brexit, has complicated data protection regulation in the United Kingdom. In particular, as of January 1,2021, the GDPR has been converted into United Kingdom law and the United Kingdom is now a "third country" under the GDPR. On June 28, 2021, the European Commission announced a decision of "adequacy" concluding that the United Kingdom ensures an equivalent level of data protection to the GDPR, which provides some relief regarding the legality of continued personal data flows from the EEA to the United Kingdom. Some uncertainty remains, however, as this adequacy determination must be renewed after four years and may be modified or revoked in the interim. Furthermore, we cannot fully predict how United Kingdom data protection laws or regulations may develop in the medium to longer term nor the effects of divergent laws and guidance regarding how data transfers to and from the United Kingdom will be regulated.

Additionally, in the United States, states have enacted data breach notification laws, personal data privacy laws, health information privacy laws, and consumer protection laws. For example, California enacted legislation known as the California Consumer Privacy Act (the "CCPA") in June 2018, which creates new individual privacy rights for consumers (as that word is broadly defined in the law) and places increased privacy and security obligations on entities handling personal data of consumers or households. The CCPA, which became effective on January 1, 2020, requires covered companies to provide new disclosures to California consumers, provides such consumers new ways to opt-out of certain sales of personal information, and allows for a new cause of action for data breaches. Although there are limited exemptions for clinical trial data under the CCPA, the CCPA will likely impact (possibly significantly) our business activities and exemplifies the vulnerability of our business to not only cyber threats but also the evolving regulatory environment related to personal data and protected health information. Additionally, California voters approved a new privacy law, the California Privacy Rights Act ("CPRA") in the November 3, 2020 election. Effective starting on January 1, 2023, the CPRA will significantly modify the CCPA, including by expanding consumers' rights with respect to certain sensitive personal information. The CPRA also creates a new state agency that will be vested with authority to implement and enforce the CCPA and the CPRA.

Other states have also enacted or proposed data privacy laws, which could further complicate the legal landscape and our domestic compliance efforts. For example, Virginia recently passed its Consumer Data Protection Act, and Colorado recently passed the Colorado Privacy Act, both of which differ from the CPRA and go into effect on January 1, 2023 and July 1, 2023 respectively. Additional state privacy legislation is expected to be enacted in the future, which, along with existing state measures, could increase our potential liability, increase compliance costs, or adversely affect our business.

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 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.

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 Tax Act enacted many significant changes to the U.S. tax laws and significantly revised the Internal Revenue Code of 1986, as amended. Future guidance from the Internal Revenue Service and other tax authorities with respect to the Tax Act may affect us, and certain aspects of the Tax Act could be repealed or modified in future legislation. For example, the CARES Act modified certain provisions of the Tax Act. In addition, it is uncertain if and to what extent various states will conform to the Tax Act, the CARES Act, or any newly enacted federal tax legislation. Changes in corporate tax rates, the realization of net deferred tax assets relating to our operations, the taxation of foreign earnings, and the deductibility of expenses under the Tax Act or 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 passage of the Tax Act, 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 the Tax Act, as modified by the CARES Act, 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. In addition, under the CARES Act, NOLs generated in tax years beginning after December 31, 2017, and before January 1, 2021, may be carried back to each of the five tax years preceding the tax years of such loss. The Company has recorded an income tax benefit of $18.1 million related to this legislation. As of December 31, 2020, we had federal net operating loss ("NOL") of $28.5 million. It is uncertain if and to what extent various states will conform to the Tax Act or the CARES Act. 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. Furthermore, while we believe based on input from our Independent Registered Public Accounting Firm that we are entitled to a refund from the carryback of post-2017 federal NOLs, there is no guarantee that the IRS will agree or that the refund will be received rapidly. 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. For example, California imposed limits on the usability of California state net operating losses to offset taxable income in tax years beginning after 2019 and before 2023. 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.

*** Our internal computer systems, or those of our CROs or other contractors and vendors who host our applications or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs.**

Despite the implementation of security measures designed to protect against a security incident, our internal computer systems and those of our CROs and other contractors or vendors who host our applications and those of our consultants are vulnerable to damage or disruption from computer viruses, software bugs, malicious code, and other unauthorized access including through cyber-attacks, ransomware attacks, supply chain attacks, natural disasters, terrorism, war, and telecommunication, equipment and electrical failures. While we have not, to our knowledge, experienced any significant system failure, accident, vulnerability, or security breach to date, if such an event were to occur, it 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. To the extent that any disruption or security breach results in a loss of or damage to our data or applications, or inappropriate disclosure or theft of confidential or proprietary information, we could incur liability, the further development of our product candidates could be delayed, our competitive position could be compromised, or our business reputation could be harmed.

*** We are dependent on information technology systems, infrastructure and data, which exposes us to data security risks.**

We are dependent upon our own or third-party information technology systems, infrastructure and data, including mobile technologies, to operate our business. The multitude and complexity of our computer systems may make them vulnerable to service interruption or destruction, disruption of data integrity, malicious intrusion, or random attacks. Likewise, data privacy or security incidents or breaches by employees or others may pose a risk that sensitive data, including our intellectual property, trade secrets or personal information of our employees, patients, customers or other business partners may be exposed to unauthorized persons or to the public. Cyber-attacks are prevalent and increasing in their frequency, sophistication and intensity. Cyber-attacks could include the deployment of harmful malware, supply chain attacks, denial-of-service, social engineering, malicious code, software bugs, and other means carried out by traditional computer “hackers” or sophisticated nation-state and nation-state sponsored actors to affect service reliability and threaten data confidentiality,

integrity and availability. 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. Despite the security controls we have in place, such attacks are very difficult to avoid. Our business partners face similar risks and any security breach of their systems could adversely affect our security posture, operations, reputation, and business.

A security breach or privacy violation that leads to disclosure or modification of or prevents access to patient information, including personally identifiable information or protected health information, could harm our reputation, compel us to comply with federal and/or state breach notification laws and foreign law equivalents, subject us to mandatory corrective action, require us to verify the correctness of database contents and otherwise subject us to litigation or other liability under laws and regulations that protect personal data, any of which could disrupt our business (including our clinical trial activities) and/or result in increased costs or loss of revenue. Moreover, the prevalent use of mobile devices that access confidential information increases the risk of data security breaches, which could lead to the loss of confidential information, trade secrets or other intellectual property. While we have invested, and continue to invest, in the protection of our data and information technology infrastructure and detection of any vulnerabilities, there can be no assurance that our efforts will prevent service interruptions, or identify and remediate breaches or vulnerabilities in our systems, which could adversely affect our business and operations and/or result in the loss of critical or sensitive information, which could result in financial, legal, business, operational, or reputational harm to us, including but not limited to causing interruptions and outages in our operations and services, and preventing us from conducting clinical trials, tests or research and development activities. Our third-party partners could also experience a security incident, which may also result in financial, legal, business, operational, or reputational harm to us and our third-party partner.

Applicable data protection laws, privacy policies and data protection 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 material adverse impacts, including without limitation, negative publicity, a loss of customer confidence in our services or security measures or breach of contract claims.

In addition, our liability insurance may not be sufficient in type or amount to cover us against claims related to security breaches, cyber-attacks and other related breaches. The successful assertion of one or more large claims against us that exceeds our available insurance coverage, or results in changes to our insurance policies (including premium increases or the imposition of large deductible or co-insurance requirements) could have an adverse effect on our business. In addition, we cannot be sure that our existing insurance coverage and coverage for errors and omissions will continue to be available on acceptable terms or that our insurers will not deny coverage as to any future claim.

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 European Union, result in restrictions or imposition of taxes and duties for importing our product candidates into the European Union, and may require us to incur additional expenses in order to develop, manufacture and commercialize our product candidates in the European Union.

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.

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 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, and a separate marketing authorization will be required to marker our product candidates in Great Britain. It is currently unclear whether the Medicines & Healthcare products Regulatory Agency ("MHRA") in the U.K. is sufficiently prepared to handle the increased volume of marketing authorization applications that it is likely to receive.

While the Trade and Cooperation Agreement provides for the tariff-free trade of medicinal products between the UK and the EU there may be additional non-tariff costs to such trade which did not exist prior to the end of the Transition Period. Further, should the UK diverge from the EU from a regulatory

perspective in relation to medicinal products, tariffs could be put into place in the future. We could therefore, both now and in the future, face significant additional expenses (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 impacted nations and the UK It is also possible that Brexit may negatively affect our ability to attract and retain employees, particularly those from the EU. These developments, or the perception that any of them could occur, may significantly reduce global trade and, in particular, trade between the impacted nations 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 earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man-made disasters or business interruptions, for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses.

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 December 31, 2020, we had approximately $276 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 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 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, 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 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 or pay cash upon their conversion.

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

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

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

If an event of default under the 2025 Notes occurs, the principal amount of the 2025 Notes, 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 Notes;
- 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, unless cured or waived, may have a material adverse effect on our results of operations.

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

Certain provisions of the 2025 Notes could make it more difficult or more expensive for or prevent a third party to acquire us. Upon the occurrence of certain transactions constituting a fundamental change, holders of the 2025 Notes will have the right, at their option, to require us to repurchase all of their 2025 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.

To the extent we issue shares of common stock upon conversion of the 2025 Notes, the conversion of some or all of the 2025 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 may encourage short selling by market participants because the conversion of the 2025 Notes could depress the price of shares of 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).
4.1	Form of Note Purchase Agreement for principal senior convertible notes with an interest rate of 4.50% due 2019 ("2019 Notes"), dated May 29, 2014, by and among the Company and the investors identified therein (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on June 4, 2014).
4.2	Form of Indenture for 2019 Notes, dated May 30, 2014 (incorporated by reference to Exhibit 10.2 to the Company's Current Report on Form 8-K, filed with the SEC on June 4, 2014).
4.3	Form of Note for 2019 Notes, dated May 30, 2014 (incorporated by reference to Exhibit 10.3 to the Company's Current Report on Form 8-K, filed with the SEC on June 4, 2014).
4.4	Base Indenture, dated September 10, 2018, between the Company and U.S. Bank National Association, as Trustee (incorporated by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K, filed with the SEC on September 10, 2018).
4.5	First Supplemental Indenture, dated September 10, 2018, between the Company and U.S. Bank National Association, as Trustee (including the form of 2.50% Convertible Senior Note due 2025) (incorporated by reference to Exhibit 4.2 to the Company's Current Report on Form 8-K, filed with the SEC on September 10, 2018).
10.1	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 18, 2021).
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

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: July 29, 2021

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: July 29, 2021

/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: July 29, 2021

/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, 2021 (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: July 29, 2021

/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, 2021 (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: July 29, 2021

/s/ Laura Clague

Laura Clague
Chief Financial Officer
(Principal Financial Officer)