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 March 31, 2023

or

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

For the transition period from ___________ to ___________
Commission File Number: 001-36257

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 May 2, 2023 was 74,607,901.

TRAVERE THERAPEUTICS, INC.

Form 10-Q
For the Fiscal Quarter Ended March 31, 2023

TABLE OF CONTENTS

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, 2022, 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 future prospects are highly dependent upon our ability to successfully develop and execute commercialization strategies for our products, including FILSPARI (sparsentan) to reduce proteinuria in adults with primary Immunoglobulin A nephropathy (IgAN), and to attain market acceptance among physicians, patients and healthcare payers.
- In order to operate our business and increase adoption and sales of our products, we need to continue to develop our commercial organization, including maintaining and growing a highly experienced and skilled workforce with qualified sales representatives.
- Our clinical trials are expensive and time-consuming and may fail to demonstrate the safety and efficacy of our product candidates. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful.
- Communications and/or feedback from regulatory authorities related to our clinical trials does not guarantee any particular outcome from or timeline for regulatory review, and expedited regulatory review pathways may not actually lead to faster development or approval.
- Interim, topline and preliminary data from our clinical trials that we announce or publish may change materially as more patient data become available and audit and verification procedures are completed.
- We face substantial generic and other competition, and our operating results will suffer if we fail to compete effectively.
- Healthcare reform initiatives, unfavorable pricing regulations, and changes in reimbursement practices of third-party payers or patients' access to insurance coverage could affect the pricing of and demand for our products.
- We are dependent on third parties to manufacture and distribute our products.
- The market opportunities for our products and product candidates may be smaller than we believe they are.
- 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 certain of our commercial products. If we are unable to obtain and maintain intellectual property relating to our technology and products, their value may 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.
- We will likely experience fluctuations in operating results and could incur substantial losses, and the market price for shares of our common stock may be volatile.

- 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. Our indebtedness could adversely affect our financial condition.
- We may be unable to successfully integrate new products or businesses we may acquire.
- We may become involved in litigation matters, 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.

PART I - FINANCIAL INFORMATION

Item 1. Financial Statements

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

	March 31, 2023	December 31, 2022
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 161,376	$ 61,688
Marketable debt securities, at fair value	400,137	388,557
Accounts receivable, net	21,537	16,646
Inventory, net	6,712	6,922
Prepaid expenses and other current assets	15,142	12,624
Total current assets	604,904	486,437
Property and equipment, net	9,127	9,049
Operating lease right of use assets	20,289	21,000
Intangible assets, net	162,883	145,038
Other assets	11,028	11,061
Total assets	$ 808,231	$ 672,585
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 16,727	$ 17,290
Accrued expenses	85,766	95,742
Deferred revenue, current portion	10,975	11,976
Business combination-related contingent consideration, current portion	6,900	7,000
Operating lease liabilities, current portion	4,545	4,433
Other current liabilities	5,732	5,722
Total current liabilities	130,645	142,163
Convertible debt	375,974	375,545
Deferred revenue, less current portion	8,778	10,931
Business combination-related contingent consideration, less current portion	68,300	64,200
Operating lease liabilities, less current portion	26,326	27,510
Other non-current liabilities	9,068	9,385
Total liabilities	619,091	629,734
Commitments and Contingencies (See Note 13)		
Stockholders' Equity:		
Preferred stock $0.0001 par value; 20,000,000 shares authorized; no shares issued and outstanding as of March 31, 2023 and December 31, 2022	—	—
Common stock $0.0001 par value; 200,000,000 shares authorized; 74,586,806, and 64,290,570 issued and outstanding as of March 31, 2023 and December 31, 2022, respectively	7	6
Additional paid-in capital	1,291,863	1,059,975
Accumulated deficit	(1,100,554)	(1,014,223)
Accumulated other comprehensive loss	(2,176)	(2,907)
Total stockholders' equity	189,140	42,851
Total liabilities and stockholders' equity	$ 808,231	$ 672,585

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

TRAVERE THERAPEUTICS, INC. AND SUBSIDIARIES
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(in thousands, except share and per share amounts)
(unaudited)

	Three Months Ended March 31,	
	2023	2022
Net product sales	$ 50,283	$ 46,443
License and collaboration revenue	6,710	2,044
Total revenue	56,993	48,487
Operating expenses:		
Cost of goods sold	5,125	2,138
Research and development	59,913	56,611
Selling, general and administrative	72,245	46,788
Change in fair value of contingent consideration	6,756	9,080
Total operating expenses	144,039	114,617
Operating loss	(87,046)	(66,130)
Other income (expenses), net:		
Interest income	3,646	278
Interest expense	(2,940)	(2,515)
Other income, net	87	26
Loss on extinguishment of debt	—	(7,578)
Total other income (expense), net	793	(9,789)
Loss before income tax provision	(86,253)	(75,919)
Income tax provision	(78)	(52)
Net loss	$ (86,331)	$ (75,971)
Basic and diluted net loss per common share	$ (1.27)	$ (1.20)
Basic and diluted weighted average common shares outstanding	68,174,099	63,132,841
Comprehensive loss:		
Net loss	$ (86,331)	$ (75,971)
Foreign currency translation (loss) gain	(566)	71
Unrealized gain (loss) on marketable debt securities	1,297	(1,204)
Comprehensive loss	$ (85,600)	$ (77,104)

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

TRAVERE THERAPEUTICS, INC. AND SUBSIDIARIES
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(unaudited, in thousands, except share amounts)

	Three Months Ended March 31, 2023						Three Months Ended March 31, 2022					
	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount					Shares	Amount				
Balance - December 31	**64,290,570**	**$ 6**	**$ 1,059,975**	**$ (2,907)**	**$ (1,014,223)**	**$ 42,851**	**62,491,498**	**$ 6**	**$ 1,068,634**	**$ (562)**	**$ (765,966)**	**$ 302,112**
Cumulative-effect adjustment from adoption of ASU 2020-06	—	—	—	—	—	—	—	—	(74,945)	—	30,225	(44,720)
Share based compensation	—	—	13,325	—	—	13,325	—	—	7,935	—	—	7,935
Issuance of common stock under the equity incentive plan and proceeds from exercise	592,486	—	2,296	—	—	2,296	317,179	—	122	—	—	122
Employee stock purchase program purchase and expense	—	—	439	—	—	439	—	—	251	—	—	251
Equity offering, net of issuance costs of $12.6 million	9,703,750	1	191,198	—	—	191,199	—	—	—	—	—	—
Issuance of pre-funded common stock warrants, net of issuance costs of $1.6 million	—	—	24,630	—	—	24,630	—	—	—	—	—	—
Issuance of common stock under At-The-Market offering, net of issuance costs of $0.6 million	—	—	—	—	—	—	701,600	—	19,545	—	—	19,545
Foreign currency translation adjustments	—	—	—	(566)	—	(566)	—	—	—	71	—	71
Unrealized gain (loss) on marketable debt securities	—	—	—	1,297	—	1,297	—	—	—	(1,204)	—	(1,204)
Net loss	—	—	—	—	(86,331)	(86,331)	—	—	—	—	(75,971)	(75,971)
Balance - March 31	**74,586,806**	**$ 7**	**$ 1,291,863**	**$ (2,176)**	**$ (1,100,554)**	**$ 189,140**	**63,510,277**	**$ 6**	**$ 1,021,542**	**$ (1,695)**	**$ (811,712)**	**$ 208,141**

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

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

	For the Three Months Ended March 31,	
	2023	2022
Cash Flows From Operating Activities:		
Net loss	$ (86,331)	$ (75,971)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	9,545	7,092
Share based compensation	13,764	8,186
Change in estimated fair value of contingent consideration	6,756	9,080
Payments from change in fair value of contingent consideration	(2,289)	(2,075)
Loss on extinguishment of debt	—	7,578
Other	(488)	2,112
Changes in operating assets and liabilities:		
Accounts receivable	(4,891)	1,241
Inventory	(513)	(157)
Prepaid expenses and other current and non-current assets	(2,350)	648
Accounts payable	(627)	(4,606)
Accrued expenses	(9,611)	(4,413)
Deferred revenue, current and non-current	(3,844)	(2,146)
Other current and non-current liabilities	(238)	(1,889)
Net cash used in operating activities	(81,117)	(55,320)
Cash Flows From Investing Activities:		
Proceeds from the sale/maturity of marketable debt securities	94,118	64,715
Purchase of marketable debt securities	(103,190)	(26,283)
Purchase of fixed assets	(630)	(131)
Purchase of intangible assets	(27,337)	(5,136)
Net cash (used in) provided by investing activities	(37,039)	33,165
Cash Flows From Financing Activities:		
Payment of guaranteed minimum royalty	(525)	(525)
Payment of business combination-related contingent consideration	(604)	(667)
Proceeds from issuances of 2029 convertible senior notes	—	316,250
Payment of debt issuance costs	—	(9,488)
Repurchase of 2025 convertible senior notes including premium	—	(211,324)
Proceeds from exercise of stock options	2,296	122
Proceeds from the issuance of common stock, net of issuance costs	191,199	—
Proceeds from the issuance of pre-funded warrants, net of issuance costs	24,630	—
Proceeds from the issuance of common stock in At-the-Market equity offering, net of issuance costs	—	19,545
Net cash provided by financing activities	216,996	113,913
Effect of exchange rate changes on cash	848	(939)
Net increase in cash and cash equivalents	99,688	90,819
Cash and cash equivalents, beginning of year	61,688	165,753
Cash and cash equivalents, end of period	$ 161,376	$ 256,572

The accompanying notes are an integral part of these 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 its subsidiaries. Travere is a fully integrated 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. The Company regularly evaluates and, where appropriate, acts on opportunities to expand its product pipeline through licenses and acquisitions of products in areas that will serve patients with serious unmet medical need and that the Company believes offer attractive growth characteristics.

FILSPARI™ (sparsentan)

On February 17, 2023, the FDA granted accelerated approval of FILSPARI™ (sparsentan) to reduce proteinuria in adults with primary IgAN at risk of rapid disease progression, generally at UPCR ≥1.5 gram/gram. FILSPARI, a once-daily, oral medication is designed to selectively target two critical pathways (endothelin 1 and angiotensin-II) in the disease progression of IgAN.

Clinical-Stage Programs:

The continued approval of FILSPARI for IgAN may be contingent upon confirmation of a clinical benefit in the Company's ongoing Phase 3 clinical trial of sparsentan for the treatment of IgAN (the "PROTECT Study"), which is designed to demonstrate whether FILSPARI slows kidney function decline. Topline results from the two-year confirmatory endpoints in the PROTECT Study are expected in the fourth quarter of 2023 and are intended to support traditional approval of FILSPARI.

Sparsentan remains a novel investigational product candidate which has been granted Orphan Drug Designation for the treatment of focal segmental glomerulosclerosis (FSGS) in the U.S. and Europe. The double-blind portion of the Phase 3 study of sparsentan for FSGS has recently concluded and, following release of the top-line data from the study which showed that the study did not meet its primary endpoint, the Company is conducting further analyses of the data and is preparing to engage with regulators to explore a potential path forward toward a potential regulatory submission in FSGS.

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

Chenodal (chenodeoxycholic acid or CDCA) is a naturally occurring bile acid that is approved for the treatment of people with radiolucent stones in the gallbladder. In September 2022, the Company was granted Fast Track Designation by the FDA for the investigation of Chenodal for cerebrotendinous xanthomatosis (CTX). In January 2020, the Company randomized the first patients in its 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 a Cooperative Research and Development Agreement ("CRADA"), which forms a multi-stakeholder approach to pool resources with leading experts, and incorporate the patient perspective early in the therapeutic identification and development process. The Company is partnering with the National Institutes of Health’s National Center for Advancing Translational Sciences ("NCATS") and a leading patient advocacy organization, Alagille Syndrome Alliance, aimed at the identification of potential small molecule therapeutics for Alagille syndrome ("ALGS"). There are no treatment options currently approved for ALGS.

The Company is party to a collaboration agreement with PharmaKrysto Limited and their early-stage cystinuria discovery program, whereby the Company is responsible for funding all research and development expenses for the pre-clinical activities associated with the cystinuria program.

Other Commercial Products:

- 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.
- 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.
- 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.

NOTE 2. BASIS OF PRESENTATION AND SIGNIFICANT ACCOUNTING POLICIES

The accompanying unaudited consolidated financial statements should be read in conjunction with the audited consolidated financial statements and notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2022, filed with the SEC on February 23, 2023. The accompanying 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 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, 2022 balance sheet information was derived from the audited financial statements as of that date. Certain reclassifications have been made to the prior period consolidated financial statements to conform to the current period presentation.

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

Principles of Consolidation

The unaudited consolidated financial statements represent the consolidation of the accounts of the Company, its subsidiaries and variable interest entities for which the Company has been determined to be the primary beneficiary, in conformity with accounting principles generally accepted in the United States ("U.S. GAAP"). All intercompany accounts and transactions have been eliminated in consolidation. See Note 6 for further discussion of variable interest entities (“VIE”) that the Company consolidates.

Revenue Recognition

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

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

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

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

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

Cost of goods sold

Cost of goods sold includes the cost of inventory sold, third party manufacturing and supply chain costs, product shipping and handling costs, and provisions for excess and obsolete inventory. Cost of goods sold also includes the cost of goods sold under the Company's license and collaboration agreements, which currently consists of the sale of active pharmaceutical ingredient to the Company's collaboration partner, at cost plus a margin.

The following table summarizes cost of goods sold for the three months ended March 31, 2023 and 2022 (*in thousands*):

	Three Months Ended March 31,	
	2023	2022
Cost of goods sold - product sales	$ 2,088	$ 2,138
Cost of goods sold - license and collaboration	3,037	—
Total cost of goods sold	$ 5,125	$ 2,138

Capitalization of Inventory Costs

Prior to the regulatory approval of the Company's drug candidates, the Company incurs expenses for the manufacture of drug product supplies to support clinical development that could potentially be available to support the commercial launch of those drugs. The Company capitalizes inventory costs associated with its products after regulatory approval, when, based on management's judgment, future commercialization is considered probable and the future economic benefit is expected to be realized. Until the date at which regulatory approval has been received, costs related to the production of inventory are recorded as research and development expenses as incurred. Any eventual sale of previously expensed ("zero-cost") inventories may impact future margins, for any periods in which those inventories are sold.

Sales of FILSPARI for the three months ended March 31, 2023 primarily consisted of zero-cost inventories, which favorably impacted gross margin for related sales. Prior to the February 2023 FDA approval of FILSPARI (sparsentan), the Company recognized approximately $7.5 million in research and development expenses related to the production of active pharmaceutical ingredient to support the commercial launch of FILSPARI. The Company expects to continue to benefit from the sale of previously expensed inventories through at least 2024.

Research and Development Expenses

Research and development includes expenses related to sparsentan, pegtibatinase, and the Company's other pipeline programs. The Company expenses all research and development costs as they are incurred. The Company's research and development costs are composed of salaries and bonuses, benefits, share-based compensation, license fees, milestones under license agreements, costs paid to third-party contractors to perform research, conduct clinical trials, develop drug materials and delivery devices, manufacture drug product supplies to support clinical development, and associated overhead expenses and facilities costs. The Company charges direct internal and external program costs to the respective development programs. The Company also incurs indirect costs that are not allocated to specific programs because such costs benefit multiple development programs and allow us to increase our pharmaceutical development capabilities. These consist of internal shared resources related to the development and maintenance of systems and processes applicable to all of our programs.

Nonrefundable advance payments for goods and services to be received in the future for use in research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the related goods are delivered or the services are performed, or when it is no longer expected that the goods will be delivered or the services rendered.

Clinical Trial Expenses

The Company records expenses in connection with its clinical trials under contracts with contract research organizations ("CROs") that support conducting and managing clinical trials, as well as contract manufacturing organizations ("CMOs") for the manufacture of drug product supplies to support clinical development. 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, and in the case of CMOs, costs associated with the production of drug product supplied and the procurement of raw materials to be consumed in the manufacturing process.

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

The Company currently has one Phase 1/2 clinical trial and three Phase 3 clinical trials in process that are in varying stages of activity, with ongoing non-clinical support trials. As such, clinical trial expenses will vary depending on all the factors set forth above and may fluctuate significantly from quarter to quarter.

Intangible Assets with Cost Accumulation Model

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

Consistent with all prior periods since Thiola was acquired, the Company has not accrued any liability for future royalties in excess of the annual contractual minimum at March 31, 2023 as such royalties are not yet probable and estimable.

Variable Interest Entity

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

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

Recently Adopted Accounting Pronouncements

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

Recently Issued Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board ("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.

NOTE 3. REVENUE RECOGNITION

Product Sales, Net

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

The Company sells FILSPARI to three direct-to-patient specialty pharmacies. The Company sells its other products to patients and pharmacies, with distribution facilitated through a single direct-to-patient distributor. Revenues from product sales are recognized in satisfaction of a single performance obligation when the customer obtains control of the Company's product. For FILSPARI, sales are recognized upon delivery of the product to the specialty pharmacies. The Company receives payments from its FILSPARI sales based on terms that are generally 30 days from shipment of the product to the specialty pharmacy. For the Company's other products, product sales are recognized upon delivery to the patient. The Company receives payments from sales of its other products, primarily through third party payers, based on terms that generally are within 30 days of delivery of product to the patient. Contracts do not contain significant financing components based on the typical period of time between performance of services and collection of consideration.

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, payers and other indirect customers relating to the Company's sales of its products. These provisions are based on the estimates of the amounts earned or to be claimed on the related sales. These amounts are treated as variable consideration, estimated and recognized as a reduction of the transaction price at the time of the sale, using the most likely amount method, 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). The Company includes these estimated amounts in the transaction price to the extent it is probable that a significant reversal of cumulative revenue recognized for such transactions will not occur. 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 estimate, which would affect net product revenue and earnings in the period such variances become known. For the three months ended March 31, 2023 and 2022, the Company recorded a net product revenue increase of $0.4 million and $0.1 million, respectively, related to performance obligations satisfied in previous periods.

Government Rebates: The Company calculates the rebates that it will be obligated to provide to government programs and deducts these estimated amounts from its gross product sales at the time the revenues are recognized. Allowances for government rebates and discounts are established based on an estimated allocation of payers 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: The Company calculates the rebates it incurs according to any contracts with certain commercial payers and deducts these amounts from its 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 for applicable products. Rebate discounts are included in other current liabilities in the accompanying consolidated balance sheets.

Prompt Pay Discounts: The Company offers discounts to certain customers for prompt payments. The Company accrues 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, the Company offers its customers a limited right to return product purchased directly from the Company, which is principally based upon the product's expiration date. Historically, returns have been immaterial.

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

The following table summarizes net product sales for the three months ended March 31, 2023 and 2022 (*in thousands*):

	Three Months Ended March 31,	
	2023	2022
Bile acid products	$ 26,105	$ 25,075
Tiopronin products	21,174	21,368
FILSPARI	3,004	—
Total net product sales	$ 50,283	$ 46,443

NOTE 4. COLLABORATION AND LICENSE AGREEMENTS

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

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

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

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

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

For the three months ended March 31, 2023, the Company recognized $6.7 million in license and collaboration revenue, which consisted of $3.3 million from the sale of active pharmaceutical ingredient to CSL Vifor at cost plus a margin, and $3.4 million for clinical development activities, based upon the ratio of costs incurred to total estimated costs. For the three months ended March 31, 2022, the Company recognized $2.0 million in license and collaboration revenue for clinical development activities, based upon the ratio of costs incurred to total estimated costs.

Deferred revenue related to the clinical development activities as of March 31, 2023 was $19.8 million. Of this amount, $11.0 million was classified as current as of March 31, 2023, based upon amounts expected to be realized within the next year.

NOTE 5. MARKETABLE DEBT SECURITIES

The Company's marketable debt securities as of March 31, 2023 and December 31, 2022 were composed of available-for-sale commercial paper and corporate and government debt securities. 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.

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

	March 31, 2023	December 31, 2022
Marketable debt securities:		
Commercial paper	$ 133,869	$ 123,647
Corporate debt securities	185,474	224,055
Securities of government sponsored entities	80,794	40,855
Total marketable debt securities	$ 400,137	$ 388,557

The following is a summary of short-term marketable debt securities classified as available-for-sale as of March 31, 2023 (*in thousands*):

	Remaining Contractual Maturity (in years)	Amortized Cost	Unrealized Gains	Unrealized Losses	Aggregate Estimated Fair Value
Marketable debt securities:					
Commercial paper	Less than 1	$ 134,218	$ —	$ (349)	$ 133,869
Corporate debt securities	Less than 1	118,324	—	(883)	117,441
Securities of government-sponsored entities	Less than 1	27,845	9	(107)	27,747
Total maturity less than 1 year		280,387	9	(1,339)	279,057
Corporate debt securities	1 to 2	68,268	109	(344)	68,033
Securities of government-sponsored entities	1 to 2	53,140	117	(210)	53,047
Total maturity 1 to 2 years		121,408	226	(554)	121,080
Total available-for-sale marketable debt securities		$ 401,795	$ 235	$ (1,893)	$ 400,137

The following is a summary of short-term marketable debt securities classified as available-for-sale as of December 31, 2022 (*in thousands*):

	Remaining Contractual Maturity (in years)	Amortized Cost	Unrealized Gains	Unrealized Losses	Aggregate Estimated Fair Value
Marketable debt securities:					
Commercial paper	Less than 1	$ 124,301	$ 2	$ (656)	$ 123,647
Corporate debt securities	Less than 1	155,841	—	(1,355)	154,486
Securities of government-sponsored entities	Less than 1	7,473	—	(80)	7,393
Total maturity less than 1 year		287,615	2	(2,091)	285,526
Corporate debt securities	1 to 2	70,195	33	(659)	69,569
Securities of government-sponsored entities	1 to 2	33,702	6	(246)	33,462
Total maturity 1 to 2 years		103,897	39	(905)	103,031
Total available-for-sale securities		$ 391,512	$ 41	$ (2,996)	$ 388,557

During the three months ended March 31, 2023, investment activity for the Company included $94.1 million in maturities and $103.2 million in purchases, all relating to debt-based marketable securities. During the three months ended March 31, 2022, investment activity for the Company included $64.7 million in maturities and $26.3 million in purchases, all relating to debt-based marketable securities. As of March 31, 2023 and December 31, 2022, the accrued interest receivable related to the Company's marketable debt securities was $2.0 million and $1.9 million and was recorded in prepaid expenses and other current assets on the Consolidated Balance Sheets.

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

The following is a summary of available-for-sale marketable debt securities in an unrealized loss position with no credit losses reported as of March 31, 2023 (*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	$ 133,869	$ 349	$ —	$ —	$ 133,869	$ 349
Corporate debt securities	79,397	826	70,900	401	150,297	1,227
Securities of government-sponsored entities	51,225	292	2,486	25	53,711	317
Total	$ 264,491	$ 1,467	$ 73,386	$ 426	$ 337,877	$ 1,893

The following is a summary of available-for-sale marketable debt securities in an unrealized loss position with no credit losses reported as of December 31, 2022 (*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	$ 117,853	$ 656	$ —	$ —	$ 117,853	$ 656
Corporate debt securities	99,066	1,041	107,964	973	207,030	2,014
Securities of government-sponsored entities	31,402	263	4,456	63	35,858	326
Total	$ 248,321	$ 1,960	$ 112,420	$ 1,036	$ 360,741	$ 2,996

As of March 31, 2023 and December 31, 2022, the amortized cost of the available-for-sale marketable debt securities in an unrealized loss position was $339.8 million and $363.7 million, respectively.

As of March 31, 2023 and December 31, 2022, 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 decrease in unrealized losses for the three months ended March 31, 2023 was primarily due to fluctuations in short-term interest rates. The Company does not believe the unrealized losses incurred during the period are due to credit-related factors. The credit ratings of the securities held remain of the highest quality. Moreover, the Company continues to receive payments of interest and principal as they become due, and our expectation is that those payments will continue to be received timely. Factors unknown to us at this time may cause actual results to differ and require adjustments to the Company's estimates and assumptions in the future.

NOTE 6. VARIABLE INTEREST ENTITIES

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

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

The upfront payments were expensed to research and development and other income (expense), net upon initial consolidation. The consolidated assets and liabilities as of March 31, 2023 and December 31, 2022 were immaterial. The results of operations were not significant for the three months ended March 31, 2023 and 2022. The Company is not required to provide additional funding other than the contractually required amounts disclosed above. The creditors and beneficial holders of PharmaKrysto have no recourse to the general credit or assets of the Company.

NOTE 7. LEASES

As of March 31, 2023, the Company had two operating leases, including 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 the Company's ability to direct the use of the office space, which occurred in phases over 2020, and utilizing a discount rate equal to the Company's estimated incremental 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 lease inception, it was not reasonably certain that the Company 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 Company has one operating lease with Esprit Investments Limited for office space located in Dublin, Ireland, which was entered into in October 2022. The initial term of the office lease ends in September 2027. The lease provides the option to extend the term of the lease by a period of 5 years, although at lease inception, it was not reasonably certain that the Company would elect this option and therefore the renewal period was excluded from the initial lease measurement. The aggregate base rent due over the initial term of the lease is approximately $0.5 million. Utilizing a discount rate equal to the Company's estimated incremental borrowing rate, the Company established an ROU asset and corresponding lease liability of $0.4 million.

Following is a schedule of the future minimum rental commitments for the Company's operating leases reconciled to the lease liability and ROU asset as of March 31, 2023 (*in thousands*):

	March 31, 2023
2023 (remaining nine months)	$ 4,754
2024	6,501
2025	6,673
2026	6,889
2027	7,064
Thereafter	4,781
Total undiscounted future minimum payments	36,662
Present value discount	(5,791)
Total lease liability	30,871
Unamortized lease incentives	(5,329)
Cash payments in excess of straight-line lease expense	(5,253)
Total ROU asset	$ 20,289

The weighted-average remaining lease term and weighted-average discount rate of the Company's operating leases are as follows:

	March 31, 2023	December 31, 2022
Weighted-average remaining lease term in years	5.4	5.7
Weighted-average discount rate	6.48 %	6.48 %

For the three months ended March 31, 2023, the Company recorded $1.2 million in expense related to operating leases, including amortized tenant improvement allowances. For the three months ended March 31, 2022, the Company recorded $1.2 million in expense related to operating leases, including amortized tenant improvement allowances.

NOTE 8. FAIR VALUE MEASUREMENTS

The Company utilizes 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 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 marketable debt securities within Level 2.

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 March 31, 2023, the fair value of the Company's 2.5% Convertible Senior Notes due 2025 was $65.2 million and the fair value of the Company's 2.25% Convertible Senior Notes due 2029 was $307.8 million. As of December 31, 2022, the fair value of the Company's 2.5% Convertible Senior Notes due 2025 was $62.9 million and the fair value of the Company's 2.25% Convertible Senior Notes due 2029 was $283.0 million. The fair values were estimated utilizing market quotations and are considered Level 2.

The following table presents the Company's assets and liabilities, measured and recognized at fair value on a recurring basis, classified under the appropriate level of the fair value hierarchy as of March 31, 2023 (*in thousands*):

	As of March 31, 2023			
	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	$ 161,376	$ 161,376	$ —	$ —
Marketable debt securities, available-for-sale	400,137	—	400,137	—
Total	$ 561,513	$ 161,376	$ 400,137	$ —
Liabilities:				
Business combination-related contingent consideration	$ 75,200	$ —	$ —	$ 75,200
Total	$ 75,200	$ —	$ —	$ 75,200

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, 2022 (*in thousands*):

	As of December 31, 2022			
	Total carrying and estimated fair value	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Assets:				
Cash and Cash Equivalents	$ 61,688	$ 61,688	$ —	$ —
Marketable debt securities, available-for-sale	388,557	—	388,557	—
Total	$ 450,245	$ 61,688	$ 388,557	$ —
Liabilities:				
Business combination-related contingent consideration	$ 71,200	$ —	$ —	$ 71,200
Total	$ 71,200	$ —	$ —	$ 71,200

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 March 31, 2023 and December 31, 2022 are as follows:

	Revenue Discount		Payment Discount
	Cholbam	Chenodal	
March 31, 2023	6.75%	6.75%	7.65%
December 31, 2022	7.75%	8.00%	8.10%

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.

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 three months ended March 31, 2023 (*in thousands*):

	Fair Value Measurements of Acquisition-Related Contingent Consideration (Level 3)	
	2023	2022
Balance at January 1,	$ 71,200	$ 67,100
Changes in the fair value of contingent consideration	6,756	9,080
Contractual payments included in accrued liabilities at March 31,	(2,756)	(2,680)
Balance at March 31,	$ 75,200	$ 73,500

NOTE 9. INTANGIBLE ASSETS

Ligand License Agreement

In 2012, the Company entered into an agreement with Ligand Pharmaceuticals, Inc. ("Ligand") for a worldwide sublicense to develop, manufacture and commercialize sparsentan (the "Ligand License Agreement"). As consideration for the license, the Company is required to make substantial payments upon the achievement of certain milestones, totaling up to $114.1 million. In March 2023, the Company capitalized a $23.0 million milestone payment to Ligand (and Bristol-Myers Squibb Company ("BMS")) that was triggered upon the accelerated approval of FILSPARI in February 2023. Pursuant to the Ligand License Agreement, the Company is obligated to pay to Ligand (and BMS) an escalating royalty between 15% and 17% of net sales of sparsentan, with payments due quarterly. The Company began incurring costs associated with such royalties following the February 2023 approval of FILSPARI (sparsentan). For the three months ended March 31, 2023, the Company capitalized $0.5 million to intangible assets for royalties owed on net sales of FILSPARI. The cost of the $23.0 million milestone payment and royalty payments are being amortized to selling, general and administration on a straight-line basis through April 30, 2033.

The following table sets forth amortizable intangible assets as of March 31, 2023 and December 31, 2022 (*in thousands*):

	March 31, 2023	December 31, 2022
Finite-lived intangible assets	$ 329,767	$ 302,935
Less: accumulated amortization	(167,820)	(158,833)
Net carrying value	$ 161,947	$ 144,102

As of March 31, 2023 and December 31, 2022, the Company had goodwill of $0.9 million.

The following table summarizes amortization expense for the three months ended March 31, 2023 and 2022 (*in thousands*):

	Three Months Ended March 31,	
	2023	2022
Research and development	$ 2,394	$ 286
Selling, general and administrative	6,593	6,270
Total amortization expense	$ 8,987	$ 6,556

NOTE 10. CONVERTIBLE NOTES PAYABLE

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

	March 31, 2023	December 31, 2022
2.25% convertible senior notes due 2029	$ 316,250	$ 316,250
2.50% convertible senior notes due 2025	68,904	68,904
Unamortized debt issuance costs - 2.25% convertible senior notes due 2029	(8,400)	(8,750)
Unamortized debt issuance costs - 2.50% convertible senior notes due 2025	(780)	(859)
Total convertible senior notes, net of unamortized debt discount and debt issuance costs	$ 375,974	$ 375,545

Convertible Senior Notes Due 2029

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

The Company received net proceeds from the issuance of the 2029 Notes of $306.4 million, after deducting commissions and offering expenses of $9.9 million. At March 31, 2023, accrued interest on the 2029 Notes of $0.6 million is included in accrued expenses in the accompanying Consolidated Balance Sheets. The 2029 Notes comprise the Company's senior, unsecured obligations and are (i) equal in right of payment with the Company's existing and future senior, unsecured indebtedness; (ii) senior in right of payment to the Company's existing and future indebtedness that is expressly subordinated to the 2029 Notes; (iii) effectively subordinated to the Company's existing and future secured indebtedness, to the extent of the value of the collateral securing that indebtedness; and (iv) structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables.

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

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

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

Convertible Senior Notes Due 2025

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

The net proceeds from the issuance of the 2025 Notes were approximately $267.2 million, after deducting commissions and the offering expenses of $8.8 million payable by the Company. At March 31, 2023, accrued interest of $0.1 million is included in accrued expenses in the accompanying Consolidated Balance Sheets. The 2025 Notes comprise the Company’s senior, unsecured obligations and are (i) equal in right of payment with the Company’s existing and future senior, unsecured indebtedness; (ii) senior in right of payment to the Company’s existing and future indebtedness that is expressly subordinated to the 2025 Notes; (iii) effectively subordinated to the Company’s existing and future secured indebtedness, to the extent of the value of the collateral securing that indebtedness; and (iv) structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables.

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

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

The 2025 Notes will be redeemable, in whole or in part, at the Company’s option at any time, and from time to time, on or after September 15, 2022 and, in the case of any partial redemption, on or before the 40th scheduled trading day before the maturity date, at a cash redemption price equal to the principal amount of the 2025 Notes to be redeemed, plus accrued and unpaid interest, if any, to, but excluding, the redemption date, but only if the last reported sale price per share of the Company’s common stock exceeds 130% of the conversion price on each of at least 20 trading days during the 30 consecutive trading days ending on, and including, the trading day immediately before the date the Company sends the related redemption notice. If a fundamental change (as defined in the 2025 Indenture) occurs, then, subject to certain exceptions, holders may require the Company to repurchase their 2025 Notes at a cash repurchase price equal to the principal amount of the 2025 Notes to be repurchased, plus accrued and unpaid interest, if any, to, but excluding, the fundamental change repurchase date. In the event of conversion, holders would forgo all future interest payments, any unpaid accrued interest and the possibility of further stock price appreciation. Upon the receipt of conversion requests, the settlement of the 2025 Notes will be paid pursuant to the terms of the 2025 Indenture. In the event that all of the 2025 Notes are converted, the Company would be required to repay the principal amount and any conversion premium in any combination of cash and shares of its common stock at the Company's option. In addition, calling the 2025 Notes for redemption will constitute a "make-whole fundamental change."

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

On March 11, 2022, the Company completed its repurchase of $207.1 million aggregate principal amount of 2025 Notes for cash, including accrued and unpaid interest, for a total of $213.8 million. This transaction involved a contemporaneous exchange of cash between the Company and holders of the 2025 Notes participating in the issuance of the 2029 Notes. Accordingly, we evaluated the transaction for modification or extinguishment accounting in accordance with ASC 470-50, *Debt – Modifications and Extinguishments* on a creditor-by creditor basis depending on whether the exchange was determined to have substantially different terms. The repurchase of the 2025 Notes and issuance of the 2029 Notes were deemed to have substantially different terms based on the present value of the cash flows or significant difference between the value of the conversion option immediately prior to and after the exchange. Therefore, the repurchase of the 2025 Notes was accounted for as a debt extinguishment. The Company recorded a $7.6 million loss on extinguishment of debt on its Consolidated Statements of Operations for the three months ended March 31, 2022, which includes the write-off of related deferred financing costs of $3.4 million. After giving effect to the repurchase, the total remaining principal amount outstanding under the 2025 Notes as of March 31, 2023 was $68.9 million.

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

The 2025 and 2029 Notes do not contain any financial or operating covenants or any restrictions on the payment of dividends, the issuance of other indebtedness or the issuance or repurchase of securities by the Company. There were no events of default for the 2025 Notes or 2029 Notes at March 31, 2023.

The 2025 and 2029 Notes are classified on the Company's Consolidated Balance Sheets at March 31, 2023 as long-tern convertible debt.

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

	Three Months Ended March 31,	
	2023	**2022**
Contractual interest expense	$ 2,209	$ 1,843
Amortization of debt issuance costs	429	337
Total interest expense for the 2025 and 2029 Notes	$ 2,638	$ 2,180

Total interest expense recognized for the three months ended March 31, 2023 was $2.9 million. Total interest expense recognized for the three months ended March 31, 2022 was $2.5 million.

NOTE 11. ACCRUED EXPENSES

Accrued expenses at March 31, 2023 and December 31, 2022 consisted of the following (*in thousands*):

	March 31, 2023	**December 31, 2022**
Compensation related costs	$ 26,839	$ 35,267
Research and development	24,909	26,070
Sales discounts, rebates, and allowances	11,451	13,486
Selling, general and administrative	9,064	8,791
Accrued royalties	7,218	7,755
Miscellaneous accrued expenses	6,285	4,373
Total accrued expenses	$ 85,766	$ 95,742

NOTE 12. NET LOSS PER COMMON SHARE

Basic and diluted net loss per common share is calculated by dividing net loss applicable to common stockholders by the weighted-average number of common shares outstanding during the period.

As discussed in Note 17, as part of its February 2023 underwritten public offering, the Company issued and sold pre-funded warrants to purchase 1.25 million shares of its common stock at a price to the public of $20.9999 per pre-funded warrant. The pre-funded warrants are exercisable immediately and are exercisable for one share of the Company's common stock. The exercise price of each pre-funded warrant is $0.0001 per share of common stock. Since the $0.0001 price per share represents little consideration and is non-substantive in relation to the $20.9999 price per pre-funded warrant and the $21.00 price per share of the common stock offered to the public, and as the warrants are immediately exercisable with no further vesting conditions or contingencies associated with them, the shares underlying the warrants are therefore included in the calculation of basic net loss per common share.

The Company’s potentially dilutive shares, which include outstanding stock options, restricted stock units, and shares issuable upon conversion of the 2025 Notes and 2029 Notes, are considered to be common stock equivalents and are not included in the calculation of diluted net loss per share because their effect is anti-dilutive.

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

	Three Months Ended March 31,					
	2023			2022		
	Shares	Net Loss	Loss per common share	Shares	Net Loss	Loss per common share
Basic and diluted loss per share	68,174,099	$ (86,331)	$ (1.27)	63,132,841	$ (75,971)	$ (1.20)

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

	Three Months Ended March 31,	
	2023	2022
Convertible debt	11,697,952	8,336,091
Options	10,546,780	9,864,889
Restricted stock	3,278,797	1,900,016
Total anti-dilutive shares	25,523,529	20,100,996

NOTE 13. COMMITMENTS AND CONTINGENCIES

Commitments

Certain of the Company's contractual arrangements with contract manufacturing organizations ("CMOs") require binding forecasts or commitments to purchase minimum amounts for the manufacture of drug product supply, which may be material to the Company's financial statements. As of March 31, 2023, we have commitments to purchase $36.3 million in active pharmaceutical ingredient, to be delivered in 2023, which is planned to support commercial sales of FILSPARI.

Contingencies

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

Legal Proceedings

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

NOTE 14. SHARE-BASED COMPENSATION

Stock Options

The following table summarizes stock option activity during the three months ended March 31, 2023:

	Shares Underlying Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2022	9,932,422	$ 21.56	5.79	$ 24,658
Granted	1,011,250	22.35	—	—
Exercised	(148,466)	15.47	—	995
Forfeited/canceled	(70,488)	27.62	—	—
Outstanding at March 31, 2023	10,724,718	$ 21.68	5.95	$ 30,730
Vested and expected to vest at March 31, 2023	10,724,718	$ 21.68	5.95	$ 30,730

At March 31, 2023, unamortized stock compensation for stock options was $38.8 million, with a weighted-average recognition period of 2.8 years.

At March 31, 2023, outstanding options to purchase 7.4 million shares of common stock were exercisable with a weighted-average exercise price per share of $20.65.

In connection with the retirement of the Company's former Chief Financial Officer, the Board of Directors approved a modification to extend the deadline to exercise each stock option held to the earlier of three months following the last vesting date or the original expiration date of the option, and to continue vesting on the original schedule of any underlying unvested stock options and restricted stock units. The modification resulted in incremental compensation cost of $2.6 million for the three months ended March 31, 2023.

Restricted Stock Units

Service Based Restricted Stock Units

The following table summarizes the Company’s service based restricted stock unit activity during the three months ended March 31, 2023:

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Unvested at December 31, 2022	2,343,709	$ 24.65
Granted	1,631,792	22.33
Vested	(444,020)	24.62
Forfeited/canceled	(26,180)	24.02
Unvested at March 31, 2023	3,505,301	$ 23.58

At March 31, 2023, unamortized stock compensation for service based restricted stock units was $73.8 million a weighted-average recognition period of 3.1 years.

Performance Based Restricted Stock Units

The following table summarizes the Company’s performance based restricted stock unit activity during the three months ended March 31, 2023:

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Unvested at December 31, 2022	157,048	$ 25.24
Granted	66,250	22.40
Vested	—	—
Forfeited/canceled	(22,840)	24.86
Unvested at March 31, 2023	200,458	$ 24.34

At March 31, 2023, unamortized stock compensation for performance based restricted stock units was $2.6 million, with a weighted-average recognition period of 1.1 years.

Share-Based Compensation

The following table sets forth total share-based compensation for the three months ended March 31, 2023 and 2022 (*in thousands*):

	Three Months Ended March 31,	
	2023	2022
Research and development	$ 4,481	$ 3,168
Selling, general and administrative	9,283	5,018
Total share-based compensation	$ 13,764	$ 8,186

NOTE 15. INVENTORY

Inventory consisted of the following at March 31, 2023 and December 31, 2022 (*in thousands*):

	March 31, 2023	December 31, 2022
Raw materials	$ 3,077	$ 3,627
Finished goods	3,635	3,295
Total inventory	$ 6,712	$ 6,922

NOTE 16. ACCOUNTS RECEIVABLE

Accounts receivable, net of reserves for prompt pay discounts and expected credit losses, was $21.5 million and $16.6 million at March 31, 2023 and December 31, 2022, respectively. The total reserves for both periods were immaterial.

The Company's evaluation and accounting for 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 February 2023, the Company sold an aggregate of approximately 9.7 million shares of its common stock and pre-funded warrants to purchase 1.25 million shares of its common stock in an underwritten public offering, at a price to the public of $21.00 per share of common stock and $20.9999 per pre-funded warrant. The pre-funded warrants are exercisable immediately, subject to certain beneficial ownership limitations which can be modified by the respective holders with at least 61 days' notice, and are exercisable for one share of the Company's common stock. The exercise price of each pre-funded warrant is $0.0001 per share of common stock. The net proceeds to the Company from the offering, after deducting the underwriting discounts and offering expenses, were approximately $215.8 million.

The pre-funded warrants were classified as a component of permanent stockholders' equity within additional paid-in capital and were recorded at the issuance date using a relative fair value allocation method. The pre-funded warrants are equity classified because they (i) are freestanding financial instruments that are legally detachable and separately exercisable from the equity instruments, (ii) are immediately exercisable, (iii) do not embody an obligation for the Company to repurchase its shares, (iv) permit the holders to receive a fixed number of shares of common stock upon exercise, (v) are indexed to the Company's common stock and (vi) meet the equity classification criteria. In addition, such pre-funded warrants do not provide any guarantee of value or return. The Company valued the pre-funded warrants at issuance, concluding that their sale price approximated their fair value, and allocated the aggregate net proceeds from the sale proportionately to the common stock and prefunded warrants, including approximately $24.6 million allocated to the pre-funded warrants and recorded as a component of additional paid-in capital.

At-the-Market Equity Offering

In February 2020, the Company entered into an Open Market Sale Agreement ("ATM Agreement") with Jefferies LLC, as agent (“Jefferies”), pursuant to which the Company may offer and sell, from time to time through Jefferies, shares of its common stock having an aggregate offering price of up to $100.0 million. Of the $100.0 million originally authorized for sale under the ATM Agreement, approximately $28.6 million were sold under the Company’s prior registration statement on Form S-3 (Registration No. 333-227182). An additional $51.9 million were sold under the Company's effective registration statement on Form S-3 (Registration Statement No. 333-259311), which included $20.1 million in the year ended December 31, 2022. The Company did not sell any shares under the ATM Agreement during the three months ended March 31, 2023. As of March 31, 2023, an aggregate of $19.5 million remained eligible for sale under the ATM Agreement.

NOTE 18. SUBSEQUENT EVENTS

On May 1, 2023, we announced that the pivotal Phase 3 DUPLEX Study did not achieve its two-year primary endpoint with statistical significance over the active control irbesartan. We are continuing to analyze the data to further evaluate the potential for sparsentan as a treatment for FSGS and plan to meet with the regulators to explore a potential path to a submission for sparsentan in FSGS.

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 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, 2022 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, 2022, filed with the Securities and Exchange Commission (SEC) on February 23, 2023. 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.

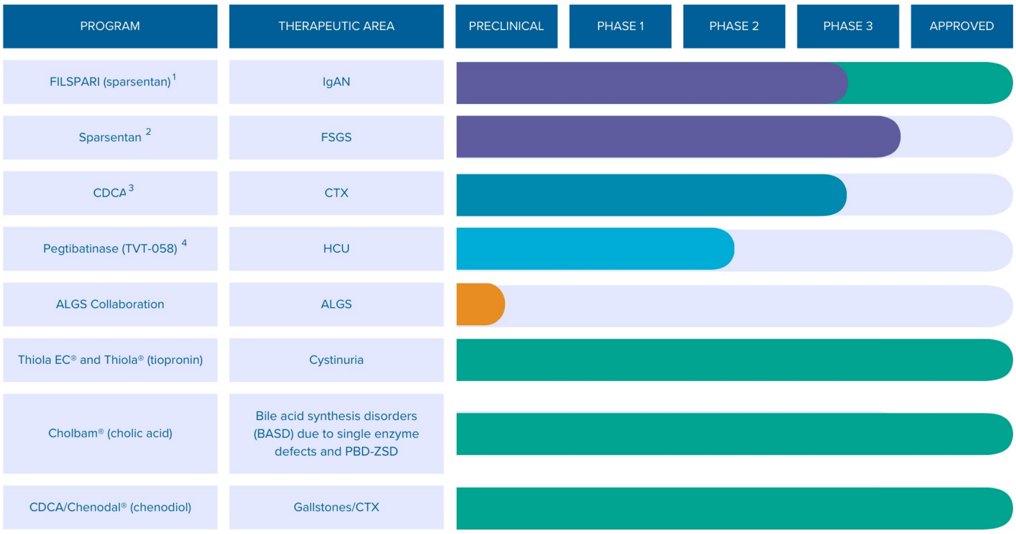
Overview

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. Our approach centers on advancing our innovative pipeline with multiple late-stage clinical programs targeting rare diseases with significant unmet medical needs. Upon approval of any of our late-stage programs, we intend to leverage the skills of our talented commercial organization which has successfully identified, supported and treated patients prescribed our approved products over the last nine years.

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 limited or 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.



CTX: cerebrotendinous xanthomatosis

1 On February 17, 2023, the FDA granted accelerated approval of FILSPARI™ (sparsentan) to reduce proteinuria in adults with primary IgAN at risk of rapid disease progression, generally a urine protein-to-creatinine ratio (UPCR) ≥1.5 gram/gram.

2 On May 1, 2023, we announced topline primary efficacy results from the pivotal Phase 3 DUPLEX Study of sparsentan, as described below.

3 CDCA is not indicated for CTX, but has received a medical necessity determination in the United States by the FDA for CTX. We are conducting a Phase 3 clinical trial to examine the safety and efficacy of CDCA (Chenodal®) for the treatment of CTX.

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

FILSPARI™ (sparsentan)

On February 17, 2023, the FDA granted accelerated approval of FILSPARI™ (sparsentan) to reduce proteinuria in adults with primary IgAN at risk of rapid disease progression, generally at UPCR ≥1.5 gram/gram. FILSPARI became commercially available in the U.S. beginning the week of February 27, 2023, and we are providing a comprehensive patient support program throughout the patient's treatment journey.

This indication is granted under accelerated approval based on reduction in proteinuria. It has not been established whether FILSPARI slows kidney function decline in patients with IgAN. The continued approval of FILSPARI may be contingent upon confirmation of a clinical benefit in the ongoing Phase 3 PROTECT Study, which is designed to demonstrate whether FILSPARI slows kidney function decline. Topline results from the two-year confirmatory endpoints in the PROTECT Study are expected in the fourth quarter of 2023 and are intended to support traditional approval of FILSPARI.

FILSPARI, a once-daily, oral medication is designed to selectively target two critical pathways in the disease progression of IgAN (endothelin 1 and angiotensin-II) and is the first and only non-immunosuppressive therapy approved for the treatment of this condition.

FILSPARI (sparsentan) is a dual endothelin angiotensin receptor antagonist (DEARA). Pre-clinical data have shown that blockade of both endothelin type A and angiotensin II type 1 pathways in forms of rare chronic kidney disease, reduces proteinuria, protects podocytes and prevents glomerulosclerosis and mesangial cell proliferation. Sparsentan has been granted Orphan Drug Designation for the treatment of IgAN in the U.S. and Europe and FILSPARI has been granted seven years of Orphan Drug Exclusivity for proteinuria in adults with primary IgAN at risk of rapid disease progression.

IgAN is characterized by hematuria, proteinuria, and variable rates of progressive renal failure. With an estimated prevalence of up to 150,000 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. FILSPARI is the first and only non-immunosuppressive therapy approved for this condition. We expect approximately 30,000 to 50,000 patients in the United States to be addressable under FILSPARI's accelerated approval indication statement.

The approval of FILSPARI, granted under the FDA's accelerated approval pathway, is based on clinically meaningful and statistically significant improvements in proteinuria compared to an active comparator in the pivotal and ongoing Phase 3 PROTECT Study, the largest head-to-head interventional study to date in IgAN. It is a global, randomized, multicenter, double-blind, parallel-arm, active-controlled clinical trial evaluating the safety and efficacy of 400mg of sparsentan, compared to 300mg of irbesartan, in 404 patients ages 18 years and up with IgAN and persistent proteinuria despite available ACE or ARB therapy.

The PROTECT Study protocol provided for an unblinded analysis of at least 280 patients to be performed after 36 weeks of treatment to evaluate the primary efficacy endpoint - the change in proteinuria (UPCR) at week 36 from baseline. Secondary efficacy endpoints include the rate of change in eGFR following the initiation of randomized treatment over 58-week and 110-week periods, as well as the rate of change in eGFR over 52-week and 104-week periods following the first six weeks of randomized treatment in approximately 380 patients. In August 2021, we announced positive topline interim results from the ongoing Phase 3 PROTECT Study. The PROTECT Study met its pre-specified interim primary efficacy endpoint with statistical significance. After 36 weeks of treatment, patients receiving FILSPARI achieved a mean reduction in proteinuria from baseline of 49.8 percent, compared to a mean reduction in proteinuria from baseline of 15.1 percent for irbesartan-treated patients ($p<0.0001$). We believe that preliminary eGFR data available at the time of the interim analysis are indicative of a potential clinically meaningful treatment effect after two years of treatment. Results from the interim assessment in the PROTECT Study showed that FILSPARI was well tolerated with a clearly defined safety profile that has been consistent across all clinical trials conducted to date. In PROTECT, the most common adverse reactions (≥ 5%) are peripheral edema, hypotension (including orthostatic hypotension), dizziness, hyperkalemia, and anemia. Because of the risks of liver injury and birth defects, FILSPARI is available only through a REMS approved by the FDA.

Per request from the FDA, the efficacy data contained in the FDA-approved label is a post-hoc sensitivity analysis that evaluates the first 281 randomized patients, a subset of the full trial population. The mean reduction in proteinuria from baseline in the post-hoc sensitivity analysis is 45% for FILSPARI versus 15% for the active control, irbesartan. Both the pre-specified and post-hoc sensitivity analyses have demonstrated that FILSPARI achieves a rapid and sustained reduction in proteinuria, with statistically significant and clinically meaningful improvement compared to the active comparator irbesartan. The PROTECT Study is fully enrolled and is scheduled to continue as planned on a blinded basis to assess the treatment effect on eGFR slope over 110 weeks in the confirmatory endpoint analysis.

Beginning in 2023, we plan to expand data generation through a sub study in the open-label extension of the ongoing PROTECT Study, as well as an open-label clinical study to investigate the safety and efficacy of sparsentan in combination with sodium glucose cotransporter-2 inhibitors (SGLT2i) for the treatment of IgAN.

In August 2022, we and Vifor (International) Ltd. ("CSL Vifor"), with whom we entered into a license and collaboration agreement ("License Agreement") in September 2021, announced that the European Medicines Agency ("EMA") had accepted for review the conditional marketing authorization application of sparsentan for the treatment of IgAN in Europe. A review decision is expected in the second half of 2023.

Clinical-Stage Programs:

Sparsentan for the treatment of FSGS

Sparsentan has been granted Orphan Drug Designation for the treatment of FSGS in the U.S. and Europe.

FSGS is a leading cause of end-stage kidney disease (ESKD) and nephrotic syndrome. There are currently no FDA-approved pharmacologic treatments for FSGS and there remains a high unmet need for patients living with FSGS as off-label treatments such as ACE/ARBs, steroids, and immunosuppressant agents are effective in only a subset of patients and use of some of these off-label treatments may be further inhibited by their safety profiles. Every year approximately 5,400 patients are diagnosed with FSGS and we estimate that there are more than 40,000 FSGS patients in the United States and a similar number in Europe with approximately half of them being candidates for sparsentan.

In 2016, we generated positive data from our Phase 2 DUET study in FSGS. In 2018, we announced the initiation of the Phase 3 clinical trial designed to serve as the basis for an NDA and MAA filing for sparsentan for the treatment of FSGS (the "DUPLEX Study"). 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 (UPCR) ≤1.5 g/g and a >40% reduction in UPCR 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 suggests that, as of the data cut-off, sparsentan has been generally well-tolerated and the overall safety results have been generally comparable between treatment groups. The confirmatory primary endpoint of the DUPLEX Study to support traditional 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 were continued in a blinded manner to assess the treatment effect on eGFR slope over 108 weeks in the confirmatory endpoint analysis.

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

In May 2023, we announced topline primary efficacy results from the pivotal Phase 3 DUPLEX Study of sparsentan in FSGS. At the end of the 108-week double-blind period, sparsentan was observed to have a 0.3 mL/min/1.73m^2 per year (95% CI: -1.74, 2.41) favorable difference on eGFR total slope and a 0.9 mL/min/1.73m^2 per year (95% CI: -1.27, 3.04) favorable difference on eGFR chronic slope compared to the active control irbesartan, which was not statistically significant. After 108 weeks of treatment, sparsentan achieved a mean reduction in proteinuria from baseline of 50%, compared to 32% for irbesartan. Although the DUPLEX Study did not achieve its two-year primary endpoint with statistical significance over the active control irbesartan, we are encouraged by topline results for the secondary endpoints on proteinuria and topline exploratory endpoints, including renal outcomes, which trended favorably for sparsentan. In addition, a preliminary review of the safety results through 108 weeks of treatment indicate sparsentan has been generally well-tolerated and the overall safety profile in the study to date has been generally consistent between treatment groups.

We are continuing to analyze the data to further evaluate the potential for sparsentan as a treatment for FSGS, and are planning to engage with regulators to explore a potential path forward for a supplemental New Drug Application (sNDA) in the U.S. Together with CSL Vifor, we also plan to engage with the EMA to determine the potential for a subsequent variation to the Conditional Marketing Authorization (CMA) of sparsentan for the treatment of FSGS, subject to a review decision on the pending application for CMA of sparsentan in IgA nephropathy. Given the high unmet need of FSGS patients, with no medicines currently approved for the condition, and the challenges associated with studying FSGS due to its heterogeneity and other attributes, we intend to pursue discussions with these regulators based on the totality of the data from the study and our clinical experience with sparsentan to date. While there is some regulatory precedent to evaluate drug candidates for potential approval despite the primary endpoint of a pivotal trial not being achieved, we are unable to predict if the regulatory agencies will be amenable to a submission based on the totality of data after not reaching statistical significance on the pre-specified primary endpoint.

If sparsentan receives marketing authorization in any of the licensed territories, CSL Vifor will be responsible for all commercialization activities in such licensed territories. We remain responsible for the clinical development of sparsentan and will retain all rights to sparsentan in the United States and rest of world outside of the licensed territories, provided that CSL Vifor has a right of negotiation to expand the licensed territories into Canada, China, Brazil and/or Mexico.

Pegtibatinase (TVT-058)

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

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

Following positive results from the first five cohorts of the ongoing Phase 1/2 COMPOSE Study, we are evaluating pegtibatinase in a final cohort in the COMPOSE Study to further inform our pivotal development program to ultimately support potential approval of pegtibatinase for the treatment of HCU. The additional cohort was initiated to inform and refine formulation work for future development and commercial purposes and to further evaluate the dose response curve for pegtibatinase. In the fourth quarter of 2022, enrollment completed in the sixth and final cohort of the ongoing Phase 1/2 COMPOSE Study. We anticipate reporting additional data from COMPOSE in mid-2023. In parallel with completing the final cohort in the COMPOSE Study, we are preparing for the potential initiation of a pivotal Phase 3 clinical trial of pegtibatinase in patients with HCU in the second half of 2023, subject to communications and feedback from the FDA and associated program assessments.

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

Chenodal

Chenodal (chenodeoxycholic acid or CDCA) is a naturally occurring bile acid that is approved for the treatment of people with radiolucent stones in the gallbladder. While indicated for radiolucent stones in the gallbladder, Chenodal has been recognized as the standard of care for cerebrotendinous xanthomatosis (CTX) for more than three decades, although it is not currently labeled for this indication. CTX is a rare, progressive and underdiagnosed bile acid synthesis disorder affecting many parts of the body. In September 2022, we were granted Fast Track designation by the FDA for the investigation of Chenodal for CTX. 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. During 2023, we expect to complete the ongoing Phase 3 RESTORE Study in CTX. Pending supportive data, we anticipate being in position to subsequently submit an NDA for a CTX indication.

Preclinical Programs:

We are a participant in a Cooperative Research and Development Agreement (CRADA), which forms a multi-stakeholder approach to pool resources with leading experts, and incorporate the patient perspective early in the therapeutic identification and development process. We are partnering with the National Institutes of Health's National Center for Advancing Translational Sciences (NCATS) and a leading patient advocacy organization, Alagille Syndrome Alliance, aimed at the identification of potential small molecule therapeutics for Alagille syndrome (ALGS). There are no treatment options currently approved for ALGS.

We are party to a collaboration agreement with PharmaKrysto Limited and their early-stage cystinuria discovery program, whereby we are responsible for funding all research and development expenses for the pre-clinical activities associated with the cystinuria program.

Other Commercial Products:

Thiola and Thiola EC (tiopronin)

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

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

In May 2021, a generic option for the 100 mg version of the original formulation of Thiola (tiopronin tablets) became available and in June 2022, a second option for the 100 mg version of the original formulation of Thiola (tiopronin tablets) was approved. These generic versions of the original formulation of Thiola have impacted sales, and these or additional generic versions of either formulation could have a material adverse impact on sales. In February 2023, a generic version of Thiola EC (100 mg and 300 mg) was approved by the FDA.

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 months ended March 31, 2023 compared to the three months ended March 31, 2022

Revenue

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

	Three Months Ended March 31,		
	2023	2022	Change
Bile acid products	$ 26,105	$ 25,075	$ 1,030
Tiopronin products	21,174	21,368	(194)
FILSPARI	3,004	—	3,004
Total net product revenues	50,283	46,443	3,840
License and collaboration revenue	6,710	2,044	4,666
Total revenue	$ 56,993	$ 48,487	$ 8,506

Net product sales

The increase in total net product revenues for the three months ended March 31, 2023 compared to the three months ended March 31, 2022 was primarily due the launch of FILSPARI in February 2023 and an increase in net sales of our bile acid products, offset slightly by a decrease in Thiola net sales. The increase in sales of our bile acid products was driven organically, whereas the decrease in Thiola net sales was a result of generic competition.

License and collaboration revenue

The increase in license and collaboration revenue for the three months ended March 31, 2023 compared to the three months ended March 31, 2022 was primarily due to a $3.3 million sale of active pharmaceutical ingredient to CSL Vifor in March 2023, at cost plus a margin, in preparation for a potential European approval and subsequent commercial launch.

Operating Expenses

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

	Three Months Ended March 31,		
	2023	2022	Change
Cost of goods sold - product sales	$ 2,088	$ 2,138	$ (50)
Cost of goods sold - license and collaboration	3,037	—	3,037
Total cost of goods sold	5,125	2,138	2,987
Research and development	59,913	56,611	3,302
Selling, general and administrative	72,245	46,788	25,457
Change in fair value of contingent consideration	6,756	9,080	(2,324)
	$ 144,039	$ 114,617	$ 29,422

Cost of goods sold

Cost of goods sold includes the cost of inventory sold, third party manufacturing and supply chain costs, product shipping and handling costs, and provisions for excess and obsolete inventory.

For the three months ended March 31, 2023 as compared to the three months ended March 31, 2022, our cost of goods sold increased by $3.0 million, due primarily to the sale of active pharmaceutical ingredient to CSL Vifor.

Sales of FILSPARI primarily consisted of zero-cost inventories, which favorably impacted gross margin for related sales. Prior to the February 2023 FDA accelerated approval of FILSPARI (sparsentan), we recognized approximately $7.5 million in research and development expenses related to the production of active pharmaceutical ingredient to support the commercial launch of FILSPARI. We expect to continue to benefit from the sale of previously expensed inventories through at least 2024.

Research and development expenses

Research and development costs include expenses related to sparsentan, pegtibatinase (TVT-058) and our other pipeline programs. We expense all research and development costs as they are incurred. Our research and development costs are comprised of salaries and bonuses, benefits, non-cash share-based compensation, license fees, milestones under license agreements, costs paid to third-party contractors to perform research, conduct clinical trials, and develop drug materials and delivery methods, manufacture drug product supplies to support clinical development, and associated overhead expenses and facilities costs. We charge direct internal and external program costs to the respective development programs. 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.

We currently have one Phase 1/2 clinical trial and three Phase 3 clinical trials in process that are in varying stages of activity, with ongoing non-clinical support studies. As such, clinical trial expenses will vary depending on the all the factors set forth above and may fluctuate significantly from quarter to quarter.

We routinely engage vendors and service providers for scientific research, clinical trial, regulatory compliance, manufacturing and other consulting services. We also make grants to research and non-profit organizations to conduct research which may lead to new intellectual properties that we may subsequently license under separately negotiated license agreements. Such grants may be funded in lump sums or installments.

The following table provides information regarding research and development expenses (*in thousands*):

	Three Months Ended March 31,		
	2023	2022	Change
External service provider costs:			
Sparsentan	$ 21,150	$ 19,372	$ 1,778
Pegtibatinase	10,411	9,079	1,332
General and other product candidates	5,289	8,936	(3,647)
Total external service provider costs	36,850	37,387	(537)
Internal personnel costs	23,063	19,224	3,839
Total research and development	$ 59,913	$ 56,611	$ 3,302

For the three months ended March 31, 2023 as compared to the three months ended March 31, 2022, our research and development expenses increased by $3.3 million. Internal personnel costs to support all programs increased by $3.8 million, reflecting increased headcount along with rising labor costs driven in part by inflation. External service provider costs decreased by $0.5 million, which included a $3.6 million decrease in general research and development costs, offset by increases of $1.8 million and $1.3 million for external service provider costs associated with the development of sparsentan and pegtibatinase, respectively.

Selling, general and administrative expenses

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

For the three months ended March 31, 2023 as compared to the three months ended March 31, 2022, our selling, general and administrative expenses increased by $25.5 million due to increased headcount as a result of operational growth, including rising labor costs driven in part by inflation, and commercial preparations and activity related to the U.S. launch of FILSPARI. Increases include combined employee compensation and stock compensation costs of $12.8 million, including a stock option modification that resulted in incremental compensation cost of $2.6 million, increases in commercial support expenses of $4.2 million and increases in various professional services expenses of $3.8 million.

Change in the valuation of contingent consideration

For the three months ended March 31, 2023 as compared to the three months ended March 31, 2022, the change in fair value of contingent consideration is due to the passage of time, updated revenue projections and changes in market driven discount rates, including fluctuations in treasury rates and credit spreads.

Other Income/Expenses

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

	Three Months Ended March 31,		
	2023	2022	Change
Interest income	$ 3,646	$ 278	$ 3,368
Interest expense	(2,940)	(2,515)	(425)
Other income, net	87	26	61
Loss on extinguishment of debt	—	(7,578)	7,578
	$ 793	$ (9,789)	$ 10,582

The $10.6 million change in our total other income (expense) for the three months ended March 31, 2023 as compared to the three months ended March 31, 2022, is primarily attributable to a $7.6 million loss on extinguishment of debt in connection with the partial repurchase in 2022 of the Convertible Senior Notes due 2025, along with a $3.4 million increase in interest income in 2023, which was driven by the impact of increases in short-term interest rates on our interest-bearing security investments.

Income Tax Benefit (Provision)

For the three months ended March 31, 2023, we recognized an income tax expense of $0.1 million as compared to an income tax expense of $0.1 million for the three months ended March 31, 2022.

At March 31, 2023, we had $11.5 million of unrecognized tax benefits. We did not recognize any interest or penalties related to unrecognized tax benefits during the three months ended March 31, 2023.

Liquidity and Capital Resources

We have financed our operations through a combination of borrowings, sales of our equity securities, and revenues generated from our commercialized products, including FILSPARI, Chenodal, Cholbam, Thiola and Thiola EC, along with proceeds from license and collaboration agreements. We have experienced significant growth in recent 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, including by adding additional members to our sales force in connection with the recent commercial launch of FILSPARI in the United States for IgAN. We anticipate that our expenses will continue to increase as we expand our sales and marketing infrastructure to commercialize FILSPARI and our other current approved products and any other new products for which we may obtain regulatory approval, advance the research and development of sparsentan for the treatment of IgAN and FSGS, along with the research and development for additional product candidates including pegtibatinase (TVT-058), and expand operational, financial, and management information systems and personnel, including personnel to support product development efforts and our obligations as a public company.

We believe that our available cash and short-term investments as of the date of this filing, together with anticipated cash generated from operations, will be sufficient to fund our anticipated level of operations beyond the next 12 months. We expect that our operating results will vary from quarter-to-quarter and year-to-year depending upon various factors including revenues, selling, general and administrative expenses, and research and development expenses, particularly with respect to our clinical and preclinical development activities. Our ability to fund our operations in subsequent years will depend upon certain factors which are beyond our control and may require us to obtain additional debt or equity capital or refinance all or a portion of our debt, including the 2025 Notes and 2029 Notes, on or before maturity. 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 completing development of products in our pipeline, obtaining regulatory approvals for these products and bringing these products to market, along with potential in-licensing of additional products approved by the FDA and selling and manufacturing these products.

We had the following balances at March 31, 2023 and December 31, 2022 (*in thousands*):

	March 31, 2023	December 31, 2022
Cash and Cash Equivalents	$ 161,376	$ 61,688
Marketable debt securities	400,137	388,557
Accumulated Deficit	(1,100,554)	(1,014,223)
Stockholders' Equity	189,140	42,851
Net Working Capital*	$ 474,259	$ 344,274
Net Working Capital Ratio**	4.63	3.42

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

As of March 31, 2023, we had cash and cash equivalents of $161.4 million and available-for-sale marketable debt securities of $400.1 million. Substantial sources of funds since the start of 2023, as summarized further below, include net proceeds of $215.8 million from an underwritten public offering of our common stock and pre-funded warrants to purchase our common stock.

Over the next 12 months, our expected financial obligations include, but are not limited to, funding our operations, operating lease payments, interest payments on our outstanding debt, royalties on sales of our existing commercialized products, research and development expenses pertaining to clinical and preclinical development activities across our pipeline, expenses associated with the launch of FILSPARI, including purchase commitments for manufactured product. Additional sources of cash over this period include net revenues from sales of our products, the sale or maturity of investments in our portfolio of marketable debt securities, and certain earned and potential milestone payments associated with sparsentan in connection with our license and collaboration arrangement with CSL Vifor.

Beyond the next 12 months and over the foreseeable future, our known commitments and potential financial obligations will likely include ongoing operations funding, operating lease payments, interest payments on our outstanding debt, royalties on sales of our existing commercialized products, research and development expenses pertaining to clinical and preclinical development activities across our pipeline, milestone and royalty payments associated with FILSPARI, pegtibatinase (TVT-058), and other developmental programs based upon the achievement of certain agreement-specific criteria, along with sales-based royalties and the repayment of principal on the outstanding 2025 Notes and 2029 Notes upon their respective maturities. Potential sources of cash over this time horizon may include net revenues from sales of our existing products and, if commercialized, our pipeline products, licensing revenue, the sale or maturity of marketable debt securities in our investment portfolio, the refinancing of all or a portion of our debt, including the 2025 Notes and 2029 Notes, on or before maturity, or the issuance of additional debt or equity. In addition, depending on prevailing market conditions, our liquidity requirements, contractual restrictions, and other factors, we may also from time to time seek to retire or purchase our outstanding debt, including the 2025 Notes or 2029 Notes, through cash purchases and/or exchanges for equity securities, in open market purchases, privately negotiated transactions or otherwise. In light of global and macroeconomic conditions, including rising interest rates, liquidity concerns at, and failures of, banks and other financial institutions, and volatility in the capital markets, we may not be able to successfully conduct financing or refinancing activity on favorable terms or at all.

Collaboration and License Proceeds

License and Collaboration Agreement with CSL Vifor

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

See Note 4 to our unaudited Consolidated Financial Statements for further discussion.

Equity Offerings

2023 Underwritten Public Offering of Common Stock

In February 2023, we sold an aggregate of approximately 9.7 million shares of our common stock and pre-funded warrants to purchase 1.25 million shares of our common stock in an underwritten public offering, at a price of $21.00 per share of common stock and $20.9999 per prefunded warrant. The pre-funded warrants are exercisable immediately, subject to certain beneficial ownership limitations which can be modified by the respective holders with at least 61 days' notice, and are exercisable for one share of our common stock. The exercise price of each pre-funded warrant is $0.0001 per share of common stock. The net proceeds to us from the offering, after deducting the underwriting discounts and offering expenses, were approximately $215.8 million.

At-the-Market Equity Offering

In February 2020, we entered into an Open Market Sale Agreement ("ATM Agreement") with Jefferies LLC, as agent (“Jefferies”), pursuant to which we may offer and sell, from time to time through Jefferies, shares of our common stock having an aggregate offering price of up to $100.0 million. Of the $100.0 million originally authorized for sale under the ATM Agreement, approximately $28.6 million were sold under our prior registration statement on Form S-3 (Registration No. 333-227182). An additional $51.9 million were sold under our effective registration statement on Form S-3 (Registration Statement No. 333-259311), which included $20.1 million in the year ended December 31, 2022. We did not sell any shares under the ATM Agreement during the three months ended March 31, 2023. As of March 31, 2023, an aggregate of $19.5 million remained eligible for sale under the ATM Agreement.

Operating Leases

Future Minimum Rental Commitments

We have future minimum rental commitments totaling $36.7 million arising from our operating leases. These commitments represent the aggregate base rent through August 2028.

See Note 7 to our unaudited Consolidated Financial Statements for further discussion.

Purchase Commitments

Manufactured Product

Certain of the Company's contractual arrangements with contract manufacturing organizations ("CMOs") require binding forecasts or commitments to purchase minimum amounts for the manufacture of drug product supply, which may be material to the Company's financial statements. As of March 31, 2023, we commitments to purchase $36.3 million in active pharmaceutical ingredient to be delivered in 2023.

Royalties and Contingent Cash Payments

Ligand License Agreement

In 2012, we entered into an agreement with Ligand Pharmaceuticals, Inc. ("Ligand") for a worldwide sublicense to develop, manufacture and commercialize sparsentan (the “Ligand License Agreement”). As consideration for the license, we are required to make substantial payments upon the achievement of certain milestones, totaling up to $114.1 million. Through March 31, 2023, we have paid $41.4 million for contractual milestone payments under the Ligand License Agreement, which includes a $23.0 million milestone payment to Ligand (and Bristol-Myers Squibb Company ("BMS")) in March 2023 that was triggered upon the accelerated approval of FILSPARI in February 2023. Upon commercialization of sparsentan or any products containing related compounds, we will be obligated to pay to Ligand an escalating annual royalty between 15% and 17% of net sales of all such products, with payments due quarterly. The Company began incurring costs associated with such royalties following the February 2023 approval of FILSPARI (sparsentan). For the three months ended March 31, 2023, the Company capitalized $0.5 million to intangible assets for royalties owed on net sales of FILSPARI.

The license agreement will continue until neither party has any further payment obligations under the agreement and is expected to continue for up to 20 years from the effective date. Ligand may terminate the license agreement due to (i) our insolvency, (ii) our material uncured breach of the agreement, (iii) our failure to use commercially reasonable efforts to develop and commercialize sparsentan as described above or (iv) certain other conditions. We may terminate the license agreement due to a material uncured breach of the agreement by Ligand.

See Note 9 to our unaudited Consolidated Financial Statements for further discussion.

Thiola License Agreement

In 2014, we entered into a license agreement with Mission Pharmacal ("Mission"), pursuant to which we obtained an exclusive, royalty-bearing license to market, sell and commercialize Thiola (tiopronin) in the United States and Canada, and a non-exclusive license to use know-how relating to Thiola to the extent necessary to market Thiola. Under the terms of the license agreement, as subsequently amended, which runs through May 2029, we are obligated to pay to Mission guaranteed minimum royalties equaling the greater of $2.1 million or 20% of our Thiola net sales generated outside of the United States during each calendar year.

Acquisition of Orphan Technologies Limited

In November 2020, we completed the acquisition of Orphan Technologies Limited (“Orphan”), including Orphan’s rare metabolic disorder drug pegtibatinase (TVT-058). We acquired Orphan by purchasing all of the outstanding shares. Under the Stock Purchase Agreement ("the Agreement"), we 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 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 product is granted.

Stock Purchase and Collaboration Agreement with PharmaKrysto

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

See Note 6 to our unaudited Consolidated Financial Statements for further discussion.

Borrowings

Convertible Senior Notes Due 2029

On March 11, 2022, we completed a registered underwritten public offering of $316.3 million aggregate principal amount of 2.25% Convertible Senior Notes due 2029 (“2029 Notes”). We issued the 2029 Notes under an indenture, dated as of September 10, 2018, as supplemented by the second supplemental indenture, dated as of March 11, 2022 (collectively, the “2029 Indenture”). The 2029 Notes will mature on March 1, 2029, unless earlier repurchased, redeemed, or converted. The 2029 Notes are senior unsecured obligations of ours and bear interest at an annual rate of 2.25%, payable semi-annually in arrears on March 1 and September 1 of each year, beginning on September 1, 2022. The 2029 Notes do not contain any financial or operating covenants or any restrictions on the payment of dividends, the issuance of other indebtedness or the issuance or repurchase of securities by us.

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 (collectively, the "2025 Indenture") with respect to the 2025 Notes. The 2025 Notes will mature on September 15, 2025, unless earlier repurchased, redeemed, or converted. The 2025 Notes are senior unsecured obligations of the Company and bear interest at an annual rate of 2.50%, payable semi-annually in arrears on March 15 and September 15 of each year, beginning on March 15, 2019. On March 11, 2022, coinciding with the issuance of the 2029 Notes, we completed our repurchase of $207.1 million of aggregate principal amount of 2025 Notes for cash. After giving effect to the repurchase, the total remaining principal amount outstanding under the 2025 Notes as of March 31, 2023 was $68.9 million. 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 us.

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. We expect to use cash flows 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, regulatory and other factors, many of which we cannot control. Factors that may affect financing requirements include, but are not limited to:

- the timing, progress, cost and results of our clinical trials, preclinical studies and other discovery and research and development activities;

- the timing and outcome of, and costs involved in, seeking and obtaining marketing approvals for our products, and in maintaining quality systems standards for our products;
- the timing of, and costs involved in, commercial activities, including product marketing, sales and distribution;
- our ability to successfully commercialize FILSPARI for IgAN, to obtain full regulatory approval for, and successfully commercialize, FILSPARI for the treatment of IgAN, and to obtain regulatory approval for, and successfully commercialize, sparsentan for FSGS and our other or future product candidates;
- increases or decreases in revenue from our marketed products, including decreases in revenue resulting from generic entrants or health epidemics or pandemics such as COVID-19;
- debt service obligations on the 2025 Notes and 2029 Notes;
- the number and development requirements of other product candidates that we pursue;
- 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;
- the emergence of competing technologies or other adverse market or technological developments; and
- the impacts of inflation and resulting cost increases.

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

Cash Flows

Cash Flows from Operating Activities

Cash used in operating activities for the three months ended March 31, 2023 was $81.1 million compared to cash used of $55.3 million for the three months ended March 31, 2022. The increase in cash used was primarily attributable to increased research and development and sales, general and administrative expenses.

Cash Flows from Investing Activities

Cash used in investing activities for the three months ended March 31, 2023 was $37.0 million compared to cash provided of $33.2 million for the three months ended March 31, 2022. The change was due to the increase in net purchases of marketable debt securities driven by reinvestment of proceeds from a more significant level of securities achieving maturity in the first quarter 2023 along with a $23.0 million milestone payment to Ligand (and BMS) in March 2023 that was triggered upon the accelerated approval of FILSPARI in February 2023.

Cash Flows from Financing Activities

Cash provided by financing activities for the three months ended March 31, 2023 was $217.0 million compared to cash provided of $113.9 million for the three months ended March 31, 2022. The increase in cash provided was due to the March 2023 issuance of common stock and pre-funded warrants through an underwritten public offering that provided $215.8 million, offset by net proceeds of $95.4 million from the March 2022 issuance of the 2029 Notes and repurchase of the 2025 Notes.

Other Matters

Adoption of New Accounting Standards

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

Critical Accounting Estimates

Our consolidated financial statements are prepared in accordance with accounting principles generally accepted in the United States of America. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and assumptions that affect the reported amount of assets, liabilities, revenue, costs and expenses, and related disclosures. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions and conditions. See our Annual Report on Form 10-K for the fiscal year ended December 31, 2022 for information about critical accounting estimates as well as a description of our other significant accounting policies.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Our primary exposure to market risk is related to changes in interest rates. As of March 31, 2023, we had cash equivalents and marketable debt securities of approximately $561.5 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 debt securities. Our marketable debt 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 $2.7 million impact on our investments.

The marketable debt securities held in our investment portfolio may subject us to credit risk, though our 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. Given these policy restrictions and our emphasis on preserving capital and liquidity while enhancing overall returns, we have not experienced material credit-related losses with our securities holdings.

We are also exposed to market risk related to changes in foreign currency exchange rates. From time to time, we enter into contracts with vendors that are located outside of the United States, which contracts are denominated in foreign currencies. We are subject to fluctuations in foreign currency rated in connection with these agreements. We do not currently hedge our foreign currency exchange rate risk.

Inflation generally affects us by increasing our salaries and fees paid to third-party contract service providers. Recent inflationary pressures have primarily impacted our operations through increased labor costs. While we continue to monitor the effects of macroeconomic factors, inflationary pressures have not affected our current outlook or business objectives.

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 not effective at the reasonable assurance level due to the material weakness in internal control over financial reporting described below.

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.

As disclosed in Part II, Item 9A of the Company's Annual Report on Form 10-K for the year ended December 31, 2022, our Chief Executive Officer and Chief Financial Officer concluded that our internal control over financial reporting was not effective as of December 31, 2022 due to the following material weakness: We did not design effective controls and procedures to evaluate the accounting for a certain pre-launch inventory contract affecting the timing of recognition of research and development expense. Such material weakness did not result in a restatement of previously issued annual consolidated financial statements or interim consolidated financial statements. The material weakness has not been fully remediated as of the date of this report.

Our evaluation did not identify any 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 March 31, 2023, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting. Our remediation plan is underway to address the material weakness mentioned above. During the quarter ended March 31, 2023, we designed and implemented controls for the timely accounting evaluation of research and development contracts that are intended to ensure appropriate expense recognition of certain pre-launch inventory. The material weakness will not be considered remediated until the applicable controls operate for a sufficient period of time and management has concluded, through testing, that these controls are operating effectively.

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 Consolidated Financial Statements--Note 13 Commitments and Contingencies: Legal Proceedings in Part I, Item 1, of this Quarterly Report on Form 10-Q.

Item 1A. Risk Factors

Our business, as well as an investment in our common stock, is highly speculative in nature and involves a high degree of risk. Our securities should be purchased only by persons who can afford to lose their entire investment. Carefully consider the risks and uncertainties described below together with all of the other information included herein, including the financial statements and related notes, before deciding to invest in our common stock. If any of the following risks actually occur, they could adversely affect our business, prospects, financial condition and results of operations. In such event(s), the market price of our common stock could decline and result in a loss of part or all of your investment. Accordingly, prospective investors should carefully consider, along with other matters referred to herein, the following risk factors in evaluating our business before purchasing any shares of our common stock. We have marked with an asterisk (*) those risk factors that were not included as separate risk factors in, or reflect changes to the similarly titled risk factors included in, Item 1A of our Annual Report on Form 10-K for the fiscal year ended December 31, 2022 as filed with the Securities and Exchange Commission (SEC) on February 23, 2023.

Risks Related to the Commercialization of Our Products

Our future prospects are highly dependent upon our ability to successfully develop and execute commercialization strategies for our products, including FILSPARI, and to attain market acceptance among physicians, patients and healthcare payers.*

Our ability to generate significant product revenues and to achieve commercial success in the near-term will depend almost entirely on our ability to successfully commercialize our products in the United States, including FILSPARI (sparsentan) to reduce proteinuria in adults with primary IgAN at risk of rapid disease progression, which was approved by the FDA in February 2023 under the FDA's accelerated approval regulations.

As a newly-approved product for a rare disease that had no previously-approved non-immunosuppressive treatment, the successful launch and commercialization of FILSPARI is subject to many risks. There are numerous examples of unsuccessful product launches and failures to meet high expectations of market potential, including by pharmaceutical companies with more experience and resources than we have. While we have established our commercial team and U.S. sales force, we will need to continue to train and further develop the team in order to successfully coordinate the ongoing launch and commercialization of FILSPARI in the United States. There are many factors that could cause the launch and commercialization of FILSPARI to be unsuccessful, including a number of factors that are outside our control. Because no non-immunosuppressive product has previously been approved by the FDA for the treatment of IgAN, it is difficult to estimate FILSPARI's market potential or the time it will take to increase patient and physician awareness of FILSPARI and change current treatment paradigms. The commercial success of FILSPARI depends on the extent to which patients and physicians accept and adopt FILSPARI for IgAN patients. For example, if the addressable patient population suffering from primary IgAN is smaller than we estimate, if it proves difficult to educate physicians as to the availability and potential benefits of FILSPARI, or if physicians are unwilling to prescribe or patients are unwilling to take FILSPARI, the commercial potential of FILSPARI will be limited. We also do not know how physicians, patients and payers will respond to the pricing of FILSPARI. Physicians may not prescribe FILSPARI and patients may be unwilling to use FILSPARI if coverage is not provided or reimbursement is inadequate to cover a significant portion of the cost. Thus, significant uncertainty remains regarding the commercial potential of FILSPARI. If the launch or commercialization of FILSPARI is unsuccessful or perceived as disappointing, the price of our common stock could decline significantly and long-term success of the product and our company could be harmed.

In order to operate our business and increase adoption and sales of our products, we need to continue to develop our commercial organization, including maintaining and growing a highly experienced and skilled workforce with qualified sales representatives.*

In order to successfully commercialize our products in the United States, we have built a specialized sales force. In order to successfully commercialize any approved products, we must continue to build our sales, marketing, distribution, managerial and other non-technical capabilities. 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 an effective sales force for our products, including the recently expanded sales force for FILSPARI or any other potential future approved products, we may not be able to generate sufficient product revenue in the United States. In addition, until the commencement of our commercial launch in February 2023, no one in our sales force had promoted FILSPARI or any other medicine for the treatment of IgAN patients. We are required to expend significant time and resources to train our sales force to be credible and persuasive in convincing physicians to prescribe and pharmacists to dispense our products. In addition, we must continually train our sales force to ensure that a consistent and appropriate message about our products is being delivered to our potential customers. We currently have limited resources compared to some of our competitors, and the continued development of our own commercial organization to market our products and any additional products we may develop or acquire will be expensive and time-consuming. We also cannot be certain that we will be able to continue to successfully develop this capability.

Similarly, if CSL Vifor does not effectively engage or maintain its sales force for sparsentan if approved in the Licensed Territories, our ability to recognize milestone payments and royalties from the Licensed Territories will be adversely affected.

We will need to continue to expend significant time and resources to train our sales forces to be credible and persuasive 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.

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

Pursuant to the terms of the License Agreement, we granted an exclusive license to CSL Vifor for the commercialization of sparsentan in the Licensed Territories, which consist of Europe, Australia and New Zealand. Consequently, the commercial success of sparsentan in the Licensed Territories will depend in significant part on the efforts of CSL Vifor, over which we will have limited control. In August 2022, Vifor Pharma Group was acquired by CSL Limited, parent company to CSL Behring and is now operating under the brand CSL Vifor. We do not currently know what effect, if any, this acquisition will ultimately have on our relationship with CSL Vifor. While our agreement with CSL Vifor remains in place following the acquisition, there is no guarantee that our collaboration with CSL Vifor will not be affected, adversely or otherwise, by the change in ownership. Moreover, in connection with the acquisition of CSL Vifor and related restructuring, substantially less resources could be devoted to the commercialization of sparsentan in the Licensed Territories, or such efforts could be discontinued entirely. If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell sparsentan in territories outside of the United States, if approved, our ability to generate product revenue outside of the United States and the Licensed Territories may be limited.

The commercial success of our products depends on them being considered to be effective drugs with advantages over other therapies.

The commercial success of our products FILSPARI, Thiola, Chenodal and Cholbam, and, if approved, sparsentan for the treatment of FSGS, 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 face substantial generic and other competition, and our operating results will suffer if we fail to compete effectively.*

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, Chenodal and Cholbam, 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 April 2021, a generic option for the 100 mg version of the original formulation of Thiola (tiopronin tablets) was approved by the FDA and an additional generic option of the original formulation of Thiola (tiopronin tablets) was approved in June 2022. Additional generic versions of Thiola may be approved in the future. During the year ended December 31, 2022, we experienced a decrease in total net product revenues compared to the year ended December 31, 2021, which was due in part to competition from generic tiopronin tablets (100 mg version of the original formulation). Our future net product revenues from Thiola may be materially impacted by competition from existing or additional generic versions of Thiola. In February 2023, a generic version of Thiola EC (100 mg and 300 mg) was approved by the FDA. Our future net product revenues from Thiola EC may also be materially impacted by competition from existing or additional generic versions of Thiola or Thiola EC.

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, any generic versions of Thiola EC, any generic versions of FILSPARI following the expiration of patent or regulatory exclusivity for the product, or generic versions of Cholbam or Chenodal or any of our other current or future products are approved, sales of that product likely would be negatively impacted, which could have a material adverse impact on our sales and profitability. 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.

The Drug Price Competition and Patent Term Restoration Act (commonly referred to as the "Hatch-Waxman Act") requires an ANDA applicant seeking FDA approval of its proposed generic product prior to the expiration of an Orange Book-listed patent (as defined below) to certify that the applicant believes that the patent is invalid, unenforceable and/or will not be infringed by the manufacture, use or sale of the drug for which the application has been submitted (a paragraph IV certification) and notify the NDA and patent holder of such certification (a paragraph IV notice). Upon receipt of a paragraph IV notice, the Hatch-Waxman Act allows the patent holder, with proper basis, to bring an action for patent infringement against the ANDA filer, asking that the proposed generic product not be approved until after the patent expires. For ANDAs that are filed ("received") after the listing of the patent in the Orange Book, if the patent holder commences a lawsuit within 45 days from receipt of the paragraph IV notice, the Hatch-Waxman Act provides a 30-month stay during which time the FDA cannot finally approve the generic's application. If the litigation is resolved in favor of the ANDA applicant during the 30-month stay period, the stay is lifted and the FDA may finally approve the ANDA if it is otherwise ready for approval. For ANDAs that are filed ("received") before the listing of the patent in the Orange Book, the 30-month stay provision of the Hatch-Waxman Act does not apply. It also may be possible, depending on the approved label, for an ANDA applicant to elect to submit a section viii statement certifying that its proposed ANDA label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent.

In October 2022, our licensor, Mission Pharmacal Company, was granted a patent covering the treatment of cystinuria by administering Thiola EC with food (US Patent No. 11,458,104, "the '104 patent") and has listed this patent in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). Following Mission's listing of the '104 patent in the Orange Book, and as of December 31, 2022, Mission has received three paragraph IV notice letters from three generic manufacturers notifying Mission that each has filed an ANDA seeking approval of a proposed generic version of Thiola EC (tiopronin) 100 mg and 300 mg oral tablets before expiration of the '104 patent and asserting that the '104 patent is not infringed and/or is invalid, with each such ANDA having been filed prior to the granting and listing of the '104 patent. The ANDA filed by Par Pharmaceutical Inc. (which had previously indicated to Mission that it may challenge the '104 patent through the Patent Trial and Appeal Board procedures at the United States Patent and Trademark Office), for a generic version of Thiola EC (100 mg and 300 mg) was approved by the FDA on February 24, 2023. Under our agreement with Mission, we have the right to enforce the '104 patent. We are evaluating these paragraph IV notices, and will evaluate any other paragraph IV notices received, on a case by case basis in order to determine whether to initiate patent litigation against any such generic manufacturer. There is no guarantee that the '104 patent will withstand any challenge at the Patent and Trademark Office or in litigation, if initiated. Patent litigation is expensive and time consuming, requires significant resources, may absorb significant time of our management and has an unpredictable outcome. If we determine not to pursue patent litigation or the patent is not upheld in litigation or administrative review or if a generic competitor is found not to infringe this patent, the resulting generic competition will likely negatively affect our business, financial condition and results of operations.

Healthcare reform initiatives, unfavorable pricing regulations, and changes in reimbursement practices of third-party payers or patients' access to insurance coverage could affect the pricing of and demand for our products.*

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 our current product candidates or any future 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 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 products.

We have no manufacturing capabilities and rely on third-party manufacturers who are sole source suppliers for manufacturing of FILSPARI, Thiola, Chenodal and Cholbam. 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. Because we are ultimately responsible for ensuring that our API and finished products are manufactured in accordance with cGMP regulations and similar regulatory requirements outside the United States, it is critical that we maintain effective management practices and oversight with respect to our third-party manufacturers, including routine auditing. 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 FILSPARI, Thiola, Chenodal or Cholbam and we are dependent on third-party distributors to distribute such products. 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 FILSPARI, Thiola, Chenodal and/or Cholbam could become disrupted, resulting in lost revenues, provider dissatisfaction, and/or patient dissatisfaction.

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

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

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

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs, providing eligibility for orphan drug exclusivity upon regulatory approval if certain jurisdictional-specific conditions are met. For example, FILSPARI has been granted orphan drug designation for the treatment of IgAN and has been awarded seven years of orphan drug exclusivity to reduce proteinuria in adults with primary IgAN at risk of rapid disease progression, generally a UPCR ≥1.5 gram/gram, and Cholbam was granted orphan drug designation in the United States and upon FDA approval of the marketing application in March 2015 was awarded seven years of orphan drug exclusivity, which expired in March 2022. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, that product is entitled to a period of marketing exclusivity, which precludes the applicable regulatory authority from approving another marketing application for the same drug for the same indication for that time period. The applicable period is seven years in the United States and ten years in Europe or, in the case of orphan drugs for which a pediatric investigation plan has been completed, 12 years. Even though we have been awarded orphan drug designation in the United States and Europe for sparsentan for the treatment of IgAN and FSGS and for pegtibatinase for the treatment of HCU, we may not be able to maintain it in Europe and the orphan drug designation may not result in orphan drug exclusivity in the United States for FSGS or Europe if approved. 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.

Risks Related to the Development of our Product Candidates

Our clinical trials are expensive and time-consuming and may fail to demonstrate the safety and efficacy of our product candidates.*

Before obtaining regulatory approval for the sale of any of our current or future product candidates, we must subject these product candidates to extensive nonclinical 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, or impact our willingness to pursue, 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 nonclinical testing or clinical trials or we may abandon projects that we expect to be clinically promising in light of cost or strategic considerations;
- 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 or the anticipated commercialization costs 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 more expensive than we originally anticipated, or we may not be able to reach agreements on acceptable terms with prospective suppliers or 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.

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.

Conducting clinical trials effectively in pursuit of regulatory approval requires significant resources, and the costs of conducting clinical trials varies depending on a number of factors, including the dosage of the study drug, trial size and duration. These costs may prove greater than we originally anticipated, which may result in us choosing to abandon or forgo clinical trials that we deem clinically promising as we actively strategize over time with respect to the allocation of our resources.

Our product development costs will also increase if we experience delays in testing or approvals. We do not know whether any nonclinical tests or clinical trials will be initiated as planned, will need to be restructured or will be completed on schedule, if at all. Significant nonclinical or clinical trial delays also could shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. In addition, 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.

For example, in our pivotal Phase 3 DUPLEX Study of sparsentan in FSGS, although we achieved the pre-specified interim FSGS partial remission of proteinuria endpoint after 36 weeks of treatment, the study did not achieve the primary efficacy eGFR slope endpoint over 108 weeks of treatment. While we intend to engage with the FDA to explore a potential path forward for a supplemental New Drug Application (sNDA) in the U.S. and work with our collaborator CSL Vifor to engage with the EMA to also explore a potential regulatory path forward in FSGS in Europe based on the DUPLEX data, there is no guarantee that we will be able to establish a pathway to a potential submission of sparsentan for FSGS based on the results from the DUPLEX Study, that the FDA and/or EMA will support an application for sparsentan in FSGS, or that sparsentan will be approved for FSGS. Moreover, while there is some regulatory precedent to evaluate drug candidates for potential approval despite the primary endpoint of a pivotal trial not being achieved, we are unable to predict if the regulatory agencies will be amenable to a submission based on the totality of data after not reaching statistical significance on the pre-specified primary endpoint.

In August 2021, we announced that our ongoing pivotal Phase 3 PROTECT Study of sparsentan in IgAN achieved its pre-specified primary efficacy endpoint after 36 weeks of treatment. Pursuant to the PROTECT Study protocol, patients are to continue in a blinded manner to assess the treatment effect on eGFR slope over two years in the confirmatory endpoint analyses of the 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 two years, it could jeopardize our ability to complete the study and submit for traditional regulatory approval for sparsentan in IgAN.

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

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

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

Success in nonclinical 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. Similarly, while we saw trends in favor of sparsentan in the two year confirmatory endpoint analysis in the DUPLEX Study in FSGS, the positive eGFR results from the open-label portion of the DUET study of sparsentan in FSGS were not replicated in the Phase 3 clinical trial with statistical significance. Similarly, the positive nonclinical data we have seen from pegtibatinase (TVT-058) being tested in a mouse model of homocystinuria and the positive topline results we reported in December 2021 from the ongoing Phase 1/2 clinical trial of pegtibatinase (TVT-058) may not be replicated in future studies. We cannot assure that any current or future clinical trials of sparsentan or pegtibatinase (TVT-058) will ultimately be successful.

Before obtaining regulatory approval to conduct clinical trials of our product candidates, we must conduct extensive nonclinical tests to demonstrate the safety of our product candidates in animals. Nonclinical 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 regulatory authorities related to our current or planned future clinical trials does not guarantee any particular outcome from or timeline for regulatory review, and expedited regulatory review pathways may not actually lead to faster development or approval.*

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

In 2018 we initiated the Phase 3 DUPLEX Study and the Phase 3 PROTECT Study. We initiated the DUPLEX Study and the PROTECT Study under the Subpart H pathway for potential accelerated approval in the United States, and potential conditional marketing authorization in Europe, in both jurisdictions based on change in proteinuria. Recognition of change in proteinuria as a surrogate endpoint in kidney disease is a relatively new regulatory development, and, as the field continues to evolve, new learnings may impact regulatory viewpoints.

In February 2023, the FDA granted accelerated approval to FILSPARI (sparsentan) to reduce proteinuria in adults with primary IgAN at risk of rapid disease progression, generally a UPCR ≥1.5 gram/gram. As a postmarketing requirement, we must complete the PROTECT Study and fulfill other post-marketing requirements. The EMA has accepted for review the conditional marketing authorization application of sparsentan for the treatment of IgAN in Europe, and a review decision is expected in the second half of 2023.

In May 2023, we announced that the DUPLEX Study did not achieve its two-year primary endpoint with statistical significance over the active control irbesartan. Although we are encouraged by the topline results for the secondary endpoints on proteinuria and topline exploratory endpoints, including renal outcomes, which trended favorably for sparsentan, and we are continuing to analyze the data to further evaluate the potential for sparsentan as a treatment for FSGS and plan to meet with the regulators to explore a potential path to a submission for sparsentan in FSGS, there is no guarantee that we or our collaborator CSL Vifor will be able to establish a pathway to a potential submission of sparsentan for FSGS based on the results from the DUPLEX Study, that the FDA and/or EMA will support an application for sparsentan in FSGS, or that sparsentan will be approved for FSGS. While there is some regulatory precedent to evaluate drug candidates for potential approval despite the primary endpoint of a pivotal trial not being achieved, we are unable to predict if the regulatory agencies will be amenable to a submission based on the totality of data after not reaching statistical significance on the pre-specified primary endpoint.

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

Although the EMA has accepted our conditional marketing authorization application for review, there can be no assurance that the study will proceed as planned and there can be no guarantee that the EMA will grant conditional marketing authorization in the EU for sparsentan for IgAN. Furthermore, even though sparsentan was granted accelerated approval for IgAN, there can be no assurance that the data from the ongoing PROTECT Study will support traditional approval of sparsentan as a treatment for IgAN.

Although the FDA has granted Fast Track and Breakthrough Therapy designations to pegtibatinase (TVT-058) for the treatment of HCU, there is no guarantee that we will be able to reach agreement with the FDA on the final study design for a proposed Phase 3 trial of pegtibatinase (TVT-058), that we will ultimately proceed with the proposed Phase 3 trial, or that pegtibatinase (TVT-058) will be approved for HCU in the future, on an expedited timeline or at all. We intend to use a surrogate endpoint, change in total homocysteine (tHcy) level, as a biomarker to demonstrate efficacy in the proposed Phase 3 pivotal trial and to support a future marketing application for TVT-058 for the treatment of HCU. While we have commenced discussions with the FDA regarding the use of this biomarker to support a future approval under the traditional or accelerated approval pathway, we will need to have further interactions with the FDA as part of the routine regulatory advancement of the program and will need to confirm with the FDA the use of total homocysteine as the pivotal endpoint for the study, align with the FDA on the details of the study, as well as on other elements of the program such as matters related to chemistry, manufacturing and controls. Prior to initiating the proposed Phase 3 trial, we will need to evaluate the clinical/regulatory pathway and the drug supply and product profile against the backdrop of the commercial landscape and opportunity to confirm strategic alignment within the program. Due to the inherent complexities of drug development, there is no guarantee that these factors will align in support of the proposed Phase 3 program.

Similarly, while we were granted Fast Track designation by the FDA for the investigation of Chenodal for CTX in September 2022, the Phase 3 RESTORE study may not ultimately support an NDA submission and the Fast Track designation may not ultimately lead to FDA approval of Chenodal for CTX on an expedited timeline or at all.

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

Interim, topline and preliminary data from our clinical trials that we announce or publish may change materially as more patient data become available and audit and verification procedures are complete.

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.

We and/or a collaborative partner are or will be subject to ongoing regulatory obligations and continued regulatory review for our approved products and any product candidates that receive regulatory approval.

The FDA's accelerated approval of FILSPARI is limited to adults with primary IgAN who are at risk of rapid disease progression, generally a UPCR ≥1.5 gram/gram, and is subject to our completion of the PROTECT Study. Any future regulatory approvals that sparsentan or any of our other product candidates receives may be subject to significant limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate.

In addition, our products, including FILSPARI, and any of our product candidates that are approved by the FDA or a comparable foreign regulatory authority, are or 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 FILSPARI and Cholbam. Further, we face risks relating to those post-marketing obligations, as well as the commercial acceptance of FILSPARI and Cholbam. If the regulatory approval for FILSPARI, Thiola, Chenodal and/or Cholbam are withdrawn for any reason, it would have a material adverse impact on our sales and profitability.

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

We depend on third-party clinical investigators and contract research organizations ("CROs") to conduct our clinical trials under agreements with us. The CROs play a significant role in the conduct of our clinical trials. Failure of the CROs to meet their obligations could adversely affect clinical development of our product candidates. The third-party clinical investigators are not our employees and we cannot control the timing or amount of resources they devote to our studies. If their performance is substandard, it could delay or prevent approval of our FDA applications. Moreover, these third-party investigators and CROs may also have relationships with other commercial entities, some of which may compete with us. If third-party investigators and CROs allocate their resources to assist our competitors at our expense, it could harm our competitive position. In response to COVID-19, we have engaged 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.

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, including FILSPARI, and any product candidates that receive marketing approval, that we or a collaboration partner bring to the market 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.

As part of the NDA review process for sparsentan for IgAN, the FDA required us to include a REMS and a boxed warning on the label regarding mandatory birth control for patients of child-bearing potential regarding risk of embryo-fetal toxicity, as has been required for other approved endothelin antagonists, and a REMS and boxed warning on the label for liver monitoring regarding potential risk of hepatotoxicity, as has been required for certain other approved endothelin antagonists. As part of the liver monitoring REMS, monthly monitoring of each patient is required for the first year the patient is on treatment, and quarterly thereafter. While we have taken efforts to streamline the REMS with the cadence of typical patient monitoring and have implemented convenience-focused features within the REMS program, the existence of monthly liver monitoring has the potential to be viewed as an impediment to prescribing FILSPARI. Also, while we intend to utilize our continued clinical trial experience with FILSPARI and post-marketing data gathering commitment to potentially support lifting of the liver monitoring REMS in the future following sufficient experience with FILSPARI and if supported by the data, there is no guarantee that the data will support this endeavor, or even if we believe it does, that the FDA will agree with it.

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

The market opportunities for our products and product candidates may be smaller than we believe they are.

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

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

The FDA-approved label of FILSPARI is currently limited to adult patients with IgAN at risk of rapid disease progression, generally a UPCR ≥1.5 gram/gram. Based on our interactions with the FDA, we believe that the FDA has imposed the rapid disease progression limitation on the FILSPARI label because of the accelerated approval pathway under which the product has been approved, and that there should be an opportunity to further expand the label to cover a broader population of IgAN patients following the conclusion of the confirmatory portion of the PROTECT Study, pending supportive data. However, there can be no guarantee that this will be the case.

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

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

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

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 certain of our commercial products. If we are unable to obtain and maintain protection for the intellectual property relating to our technology and products, their value 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 license to a granted U.S. patent covering the treatment of cystinuria by administering Thiola EC with food (U.S. Patent No. 11,458,104, "the '104 patent"), as well as a pending U.S. patent application directed to Thiola EC, we do not know whether the pending U.S. patent application or any future patent application will result in a granted patent covering Thiola EC. More generally, we may not be able to obtain additional issued patents relating to our technology or products. Even if issued, patents issued to us or our licensors, including for example the 104 patent, 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.

Patent laws vary by country. Some countries have compulsory licensing laws under which a patent owner may be required to grant licenses to third parties. Some countries do not grant or enforce patents related to medical treatments, or limit enforceability in the case of a public emergency. In addition, many countries limit the enforceability of patents against government agencies or government contractors. If we are unable to obtain or enforce patents related to medical treatments in certain countries, or we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our business may be adversely affected.

The intellectual property systems in other countries can be destabilized as a result of political events, during which the ability to obtain, maintain and enforce intellectual property protection in the affected country may be uncertain and evolving. For example, as a result of the ongoing war between Ukraine and Russia, Russian officials have suggested that they may treat patents or patent applications owned by parties from certain countries, including the United States, as unenforceable and/or provide for zero compensation compulsory licenses to such patents or patent applications. Recent court decisions in Russia have raised questions about the strength of trademark protections in Russia. The U.S. government’s response to political events may also negatively affect our ability to obtain, maintain and enforce intellectual property protection in the affected country. For example, the U.S. government has issued sanctions against Russia related to the ongoing war in Ukraine, and as a result of these sanctions, it may not be possible to pay fees necessary for prosecution and maintenance of Russian patent applications and patents in the absence of licenses or exclusions set forth by the U.S. government authorizing transactions in connection with intellectual property. Payments for trademark protection may be similarly impacted. The U.S. Department of the Treasury has issued General License No. 31, authorizing such transactions to allow filing, prosecution and maintenance of Russian patents and trademarks. Uncertainties regarding political events, including the ongoing war between Ukraine and Russia, as well as any resulting losses of intellectual property protection, could harm our business.

Our product FILSPARI 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. 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 IgAN as well as glomerulosclerosis, including FSGS.

For products we develop based on a new chemical entity not previously approved by the FDA, we expect that in addition to the protection afforded by our patent filings that we will be able to obtain five years regulatory exclusivity via the provisions of the Food, Drug, and Cosmetic ("FDC") Act and possibly seven years regulatory exclusivity via the orphan drug provisions of the FDC Act. In the case of sparsentan, the periods of regulatory exclusivity may, if certain conditions are satisfied, be extended by six months on the basis of pediatric exclusivity, thereby resulting in exclusivity periods of 5.5 years and 7.5 years, respectively. In addition, we may be able to obtain up to five years patent term extension (to compensate for regulatory approval delay) for one patent covering such a product for its FDA-approved use. Such a patent, like the periods of regulatory exclusivity, also may be extended by a further six months on the basis of pediatric exclusivity if certain conditions are satisfied.

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

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

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

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

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

We expect to rely on orphan drug status to develop and commercialize certain of our products and 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, IgAN and FSGS and homocystinuria, respectively. While we have been granted these orphan designations, we will not be able to rely on these designations to exclude other companies from manufacturing or selling these molecules for the same indication beyond these time frames. Furthermore, in Europe, orphan drug status is re-evaluated in connection with the marketing authorization review process and a product candidate must re-qualify as of such time in order to maintain orphan drug status. In addition, any marketing exclusivity in Europe can be reduced from ten years to six years if the initial designation criteria have significantly changed since the market authorization of the orphan product.

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

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

In March 2010, President Obama signed the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the "PPACA"), a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The PPACA revised the definition of "average manufacturer price" for reporting purposes, which could increase the amount of Medicaid drug rebates to states. The PPACA also increased the mandated Medicaid rebate from 15.1% to 23.1% of the average manufacturer price, expanded the rebate to Medicaid managed care utilization and increased the types of entities eligible for the federal 340B drug discount program. Further, the law imposed a significant annual fee on companies that manufacture or import certain branded prescription drug products. There have been executive, judicial, Congressional, and political challenges to certain aspects of the PPACA. For example, 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. Further, on August 16, 2022, President Biden signed the Inflation Reduction Act of 2022 ("IRA") into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in PPACA marketplaces through plan year 2025. The IRA also eliminates the "donut hole" under the Medicare Part D program beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost and through a newly established manufacturer discount program. It is possible that the PPACA will be subject to judicial or Congressional challenges in the future. It is unclear how any additional 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, President Obama signed into law the Budget Control Act of 2011, which, among other things, 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, will stay in effect until 2032 unless additional Congressional action is taken. Additionally, in January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers, including hospitals and imaging centers.

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

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

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

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

A primary trend in the United States healthcare industry and elsewhere is toward cost containment. We expect the changes made by PPACA, other legislation impacting the Medicare program and the 340B program, and the increasing emphasis on managed care to continue to put pressure on pharmaceutical product pricing. As these concerns continue to grow over the need for tighter oversight, there remains the possibility that the Heath Resources and Services Administration or another agency under the U.S. Department of Health and Human Services ("HHS") will propose regulations or that Congress will explore changes to the 340B program through legislation. There have also been a number of initiatives pending at the state and federal level that could negatively impact the reimbursement for products approved under the accelerated approval pathway in the United States by restricting patient access or establishing differential payment models. Certain states are also in the process of establishing Patient Drug Affordability Boards with the authority in some cases to set upper payment limits.

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, in July 2021, the Biden administration released an executive order that included multiple provisions aimed at prescription drugs. In response to Biden's executive order, on September 9, 2021, HHS released a Comprehensive Plan for Addressing High Drug Prices that outlines principles for drug pricing reform. The plan sets out a variety of potential legislative policies that Congress could pursue as well as potential administrative actions HHS can take to advance these principles. In addition, the IRA, among other things (i) directs HHS to negotiate the price of certain high-expenditure, single-source drugs and biologics covered under Medicare and (ii) imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation. These provisions will take effect progressively starting in fiscal year 2023, although they may be subject to legal challenges. HHS has and will continue to issue and update guidance as these programs are implemented. It is currently unclear how the IRA will be implemented but it is likely to have a significant impact on the pharmaceutical industry. In addition, in response to the Biden administration's October 2022 executive order, on February 14, 2023, HHS released a report outlining three new models for testing by the Center for Medicare and Medicaid Innovation which will be evaluated on their ability to lower the cost of drugs, promote accessibility, and improve quality of care. It is unclear whether the models will be utilized in any health reform measures in the future. At the state level, 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.

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.

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 $30 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 as we obtain marketing approval for additional product candidates in development, but we may be unable to obtain commercially reasonable product liability insurance. On occasion, juries have awarded large judgments in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or series of claims brought against us would decrease our cash reserves and could cause our stock price to fall.

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

Several of our competitors have substantially greater financial, research and development, distribution, manufacturing and marketing experience and resources than we do and represent substantial long-term competition for us. Other companies may succeed in developing and marketing products that are more effective and/or less costly than any products that may be developed and marketed by us, or that are commercially accepted before any of our products. Factors affecting competition in the pharmaceutical and drug industries vary, depending on the extent to which a competitor is able to achieve a competitive advantage based on its proprietary technology and ability to market and sell drugs. The industry in which we compete is characterized by extensive research and development efforts and rapid technological progress. Although we believe that our orphan drug status and proprietary position with respect to sparsentan may give us a competitive advantage, new developments are expected to continue and there can be no assurance that discoveries by others will not render such potential products noncompetitive. Furthermore, competitors could enter the market with generic versions of our products. For example, a generic option for the 100 mg version of the original formulation of Thiola (tiopronin tablets) was approved by the FDA in May 2021 and a second 100 mg version of the original formulation of Thiola (tiopronin tablets) was approved by the FDA in June 2022. Also, a generic option for the 100 mg and 300 mg versions of Thiola EC was approved by the FDA in late February 2023.

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 nonclinical, 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 FILSPARI and for our development stage product candidates. We expect the manufacturers of each product or 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;
- less control over cost increases resulting from inflationary pressures affecting raw materials and other supply chain components;
- 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 are 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. We are ultimately responsible for ensuring that our API and finished products are manufactured in accordance with cGMP regulations and similar regulatory requirements outside the United States, and it is therefore critical that we maintain effective management practices and oversight with respect to our third-party manufacturers, including routine auditing. 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. A health epidemic or pandemic and associated vaccine or treatment development and manufacturing efforts may increase demand for the services supplied by many third-party manufacturers, including some of those that we utilize for our products and product candidates, which may result in 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 products and 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 and negatively affect our results of operations.

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

Risks Related to Our Business

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

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

We have experienced significant growth over the past five years in the number of our employees and the scope of our operations. We have expanded our sales and marketing, compliance and legal functions in addition to expansion of all functions to support a commercial organization. We have also expanded our operations in connection with the commercial launch of FILSPARI in the United States, including by adding additional members to our sales force, and expect to continue to hire additional staff in the coming months. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities, continue to recruit and train additional qualified personnel, and successfully integrate such expanded operations into our existing business. 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, including in connection with the ongoing commercial launch of FILSPARI in the United States. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability on the part of our management to manage growth could delay the execution of our business plans or disrupt our operations.

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

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

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

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

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

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

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

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

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

Health epidemics or pandemics could materially adversely affect our business, results of operations and financial condition.

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

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

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

Moreover, COVID-19 continues to evolve, and the extent to which COVID-19 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 emergence, infectiousness and severity of new variants, travel restrictions and social distancing in the United States and other countries, business closures or business disruptions, global supply challenges, and the effectiveness of actions taken in the United States and other countries to contain and treat the disease. New health epidemics or pandemics may emerge that result in similar or more severe disruptions to our business.

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 nonclinical development activities. We anticipate that our expenses will continue to increase as we:

- continue the ongoing portion of the Phase 3 trial of FILSPARI for the treatment of IgAN to the confirmatory endpoint and through the open-label extension period;
- continue the open label portions of DUET and DUPLEX;
- continue the research and development of additional product candidates, including pegtibatinase (TVT-058);
- expand our sales and marketing infrastructure to commercialize our current approved products, and any other 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.

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 nonclinical 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 may not be successful enough in these activities to generate revenues that are substantial enough to recoup the expenses we have expended in conducting these activities to achieve profitability. Pursuant to the Ligand License Agreement, we are obligated to pay to Ligand an escalating annual royalty between 15% and 17% of net sales of FILSPARI and any other products containing sparsentan or related compounds, which will impact our potential future profit from the commercialization of FILSPARI in the United States and sparsentan for the treatment of IgAN in Europe, if approved, as well as sparsentan for the treatment of FSGS, if approved. 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 to 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.*

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

Management believes our ability to continue our operations depends on our ability to sustain and grow revenue, results of operations and our ability to access capital markets when necessary to accomplish our strategic objectives. Management believes that we may incur losses in the immediate future. We expect that our operating results will vary significantly from quarter-to-quarter and year-to-year as a result of investments in research and development, specifically our clinical and nonclinical 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 timing, progress, cost and results of our clinical trials, preclinical studies and other discovery and research and development activities;
- the timing of, and costs involved in, seeking and obtaining marketing approvals for our products, and in maintaining quality systems standards for our products;
- the timing of, and costs involved in, commercial activities, including product marketing, sales and distribution;
- our ability to successfully commercialize FILSPARI in adult patients with IgAN, and to obtain regulatory approval for and successfully commercialize our other or future product candidates;
- increases or decreases in revenue from our marketed products, including decreases resulting from generic entrants or health epidemics or pandemics such as COVID-19;
- debt service obligations on the 2025 Notes and 2029 Notes;
- the number and development requirements of other product candidates that we pursue;
- our ability to manufacture sufficient quantities of our products to meet expected demand;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property related claims;
- 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;
- the emergence of competing products and technologies and other adverse market developments;
- the extent to which we acquire or invest in businesses, products and technologies; and
- the potential impacts of inflation and resulting cost increases.

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

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

- results of clinical trials of our product candidates or those of our competitors;
- our entry into or the loss of a significant collaboration;
- regulatory or legal developments in the United States and other countries, including changes in the health care payment systems;
- our ability to obtain and maintain marketing approvals from the FDA or similar regulatory authorities outside the United States;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors and issuance of new or changed securities analysts' reports or recommendations;

- general economic, industry and market conditions, including the impacts thereon of inflation and rising interest rates, actual or anticipated bank failures, COVID-19, Russia's invasion of Ukraine and global geopolitical tensions;
- results of clinical trials conducted by others on drugs that would compete with our product candidates;
- developments or disputes concerning patents or other proprietary rights;
- public concern over our product candidates or any products approved in the future;
- litigation;
- communications from government officials regarding health care costs or pharmaceutical pricing;
- future sales or anticipated sales of our common stock by us or our stockholders; and
- the other factors described in this “Risk Factors” section.

In addition, the stock markets, and in particular, the Nasdaq Stock 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 acquired company or product into our own operations.

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 litigation matters, which could result in substantial costs, divert management's attention and otherwise have a material adverse effect on our business, operating results or financial condition.*

From time to time we may become involved in certain litigation matters, including those described in Note 13 of the 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, the Centers for Medicare & Medicaid Services ("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 nonclinical 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 Act cases against pharmaceutical manufacturers and other healthcare entities, including substantially increased funding for healthcare fraud enforcement activities, enhanced investigative powers, amendments to the federal False Claims Act that make it easier for the government and whistleblowers to pursue cases for alleged kickback and false claim violations and public reporting of certain payments and transfers of value by certain pharmaceutical manufacturers to physicians and teaching hospitals nationwide. We anticipate that government scrutiny of pharmaceutical sales and marketing practices will continue for the foreseeable future and subject us to the risk of further government investigations and enforcement actions. Responding to a government investigation or enforcement action would be expensive and time-consuming, and could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

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

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

Additionally, the federal Physician Payments Sunshine Act within the PPACA, and its implementing regulations, require that certain manufacturers of drugs, devices, biologicals and medical supplies to report annually information related to certain payments or other transfers of value made or distributed to physicians (defined to include doctors, dentists, optometrists, podiatrists, and chiropractors), other healthcare professionals (such as physician assistants and nurse practitioners), and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, physicians and teaching hospitals and to report annually certain ownership and investment interests held by physicians and their immediate family members.

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

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

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

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 nonclinical 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 nonclinical 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 nonclinical, clinical or other studies. In addition, varying interpretations of the data obtained from nonclinical and clinical testing could delay, limit or prevent regulatory approval of a product candidate. Any regulatory approval we ultimately obtain may be limited or subject to restrictions or post approval commitments that render the approved product not commercially viable. Any FDA or other regulatory approval of our product candidates, once obtained, may be withdrawn, including for failure to comply with regulatory requirements or if clinical or manufacturing problems follow initial marketing.

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

In the ordinary course of business, we collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, process) personal data and other sensitive information, including proprietary and confidential business data, trade secrets, intellectual property, data we collect about trial participants in connection with clinical trials, and sensitive third-party data. Our data processing activities may subject us to numerous data privacy and security obligations, such as various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contracts, and other obligations that govern the processing of personal data by us and on our behalf.

In the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal data privacy laws, consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act) and other similar laws (e.g., wiretapping laws). For example, HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), and their respective implementing regulations, imposes specific requirements relating to the privacy, security and transmission of individually identifiable health information. Among other things, HITECH, through its implementing regulations, makes certain of HIPAA's privacy and security standards directly applicable to business associates, defined as a person or organization, other than a member of a covered entity's workforce, that creates, receives, maintains or transmits protected health information for or on behalf of a covered entity for a function or activity regulated by HIPAA as well as their covered subcontractors. In addition, the California Consumer Privacy Act of 2018 ("CCPA") imposes obligations on businesses to which it applies. These obligations include, without limitation, providing specific disclosures in privacy notices, affording California residents certain rights related to their personal data, and requiring businesses subject to the CCPA to implement certain measures to effectuate California residents' personal data rights. The CCPA allows for statutory fines for noncompliance (up to $7,500 per violation). Although the CCPA exempts some data processed in the context of clinical trials, the CCPA increases compliance costs and potential liability with respect to other personal data we maintain about California residents. In addition, the California Privacy Rights Act of 2020 ("CPRA"), operative on January 1, 2023, expands the CCPA, by applying it to personal information of business representatives and employees, establishing a new California Privacy Protection Agency to implement and enforce the CCPA (as amended) and adding a new right for individuals to correct their personal information. Other states have enacted data privacy laws, including Virginia and Colorado, both of which differ from the CPRA and become effective in 2023. Additional data privacy and security legislation has been proposed at the federal, state, and local levels in recent years. While these states, like the CCPA, also exempt some data processed in the context of clinical trials, such laws could increase our potential liability, increase compliance costs, or adversely affect our business.

Outside the United States, an increasing number of laws, regulations, and industry standards apply to data privacy and security. For example, the European Union's General Data Protection Regulation ("EU GDPR"), the United Kingdom's GDPR ("UK GDPR"), Brazil's General Data Protection Law (Lei Geral de Proteção de Dados Pessoais, or "LGPD") (Law No. 13,709/2018), and China's Personal Information Protection Law ("PIPL") impose strict requirements for processing personal data. For example, the EU GDPR imposes significant and complex burdens on processing personal data, particularly for processing "special category personal data" (such as personal data related to health and genetic information), which could be relevant to our operations in the context of our conduct of clinical trials and is of interest to relevant regulators. Under the EU GDPR, government regulators may impose temporary or definitive bans on data processing, as well as fines of up to 20 million euros or 4% of annual global revenue, whichever is greater. Further, under the EU GDPR, individuals may initiate litigation related to processing of their personal data, as well as consumer protection organizations authorized at law to represent data subjects' interests.

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

In the ordinary course of business, we may transfer personal data from Europe and other jurisdictions to the United States or other countries. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal data to other countries. In particular, the European Economic Area (EEA) and the United Kingdom (UK) have significantly restricted the transfer of personal data to the United States and other countries whose privacy laws it believes are inadequate. Other jurisdictions may adopt similarly stringent interpretations of their data localization and cross-border transfer laws, which could make it more difficult to transfer information across jurisdictions or prevent us from conducting business in certain countries. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and UK to the United States in compliance with law, such as the EEA and UK's standard contractual clauses, these mechanisms are subject to legal challenges, and there is no assurance that we can satisfy or rely on these measures to lawfully transfer personal data to the United States.

If we are unable to implement a valid compliance mechanism for cross-border personal data transfers, or if the requirements for a legally-compliant transfer are too onerous, we may face significant adverse consequences, including increased exposure to regulatory actions, substantial fines and injunctions against processing or transferring personal data from Europe. Inability to import personal data from Europe to the United States may significantly and negatively impact our business operations, including by limiting our ability to conduct clinical trial activities in Europe and elsewhere; limiting our ability to collaborate with CROs, service providers, contractors and other companies that are subject to such cross-border data transfer or localization laws; the need to relocate part of or all of our business or data processing activities to other jurisdictions at significant expense; or requiring us to increase our personal data processing capabilities and infrastructure in foreign jurisdictions at significant expense. Additionally, companies that transfer personal data out of the EEA and UK to other jurisdictions, particularly to the United States, are subject to increased scrutiny from regulators, individual litigants, and activities groups. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers out of Europe for allegedly violating the GDPR's cross-border data transfer limitations.

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

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

In the ordinary course of our business, we and our third-party service providers may process proprietary, confidential, and sensitive data, including personal data (such as health-related data and data related to our clinical trials), intellectual property, and trade secrets (collectively, sensitive information). We may rely upon third-party service providers and technologies to operate critical business systems to process sensitive information in a variety of contexts, including, without limitation, third-party providers of cloud-based infrastructure, encryption and authentication technology, employee email, and other functions. We also rely on third-party service providers to provide certain products, including active pharmaceutical ingredients, to operate our business, including in China. Our ability to monitor these third parties' information security practices is limited, and these third parties may not have adequate information security measures in place. While we may be entitled to damages if our third-party service providers fail to satisfy their privacy or security-related obligations to us, any award may be insufficient to cover our damages, or we may be unable to recover such award. In addition, supply-chain attacks have increased in frequency and severity, and we cannot guarantee that third parties' infrastructure in our supply chain or our third-party partners' supply chains have not been compromised. We may share or receive sensitive information with or from third parties.

Cyberattacks, malicious internet-based activity, and online and offline fraud are prevalent and continue to increase. These threats are becoming increasingly difficult to detect. These threats come from a variety of sources, including traditional computer “hackers,” threat actors, personnel (such as through theft or misuse), "hacktivists", organized criminal threat actors, sophisticated nation-states, and nation-state-supported actors. Some actors now engage and are expected to continue to engage in cyberattacks, including without limitation nation-state actors for geopolitical reasons and in conjunction with military conflicts and defense activities. During times of war and other major conflicts, including the war in Ukraine, we and the third parties upon which we rely may be vulnerable to a heightened risk of these attacks, including retaliatory cyberattacks that could materially disrupt our systems and operations, supply chain, and ability to produce, sell and distribute our goods and services. We and the third parties upon which we rely may be subject to a variety of other evolving threats, including, but not limited to, social-engineering attacks (including through phishing attacks), malicious code (such as viruses and worms), malware (including as a result of advanced persistent threat intrusions), denial-of-service attacks (such as credential stuffing), credential harvesting, personnel misconduct or error, ransomware attacks, supply-chain attacks, software bugs, server malfunctions, software or hardware failures, loss of data or other information technology assets, adware, telecommunications failures, and other similar threats. In particular, ransomware attacks, including those from organized criminal threat actors, nation-states and nation-state supported actors, are becoming increasingly prevalent and severe and can lead to significant interruptions, delays, or outages in our operations, disruption of clinical trials, loss of data (including data related to clinical trials), loss of income, significant extra expenses to restore data or systems, reputational loss and the diversion of funds. To alleviate the financial, operational and reputational impact of a ransomware attack, it may be preferable to make extortion payments, but we may be unwilling or unable to do so (including, for example, if applicable laws prohibit such payments). Additionally, remote work has become more common and has increased risks to our information technology systems and data, as more of our employees utilize network connections, computers, and devices outside our premises or network, including working at home, while in transit, and in public locations. Future or past business transactions (such as acquisitions or integrations) could also expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. Furthermore, we may discover security issues that were not found during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

Any of the previously identified or similar threats could cause a security incident or other interruption. A security incident or other interruption could result in unauthorized, unlawful, or accidental acquisition, modification, destruction, loss, alteration, encryption, disclosure of, or access to our sensitive information. A security incident or other interruption could disrupt our ability (and that of third parties upon whom we rely) to provide our products and services. We may expend significant resources or modify our business activities (including our clinical trial activities) to try to protect against security incidents. Certain data privacy and security obligations require us to implement and maintain specific security measures, industry-standard or reasonable security measures to protect our information technology systems and sensitive information.

While we have implemented security measures designed to protect against security incidents, there can be no assurance that these measures will be effective. We take steps to detect and remediate vulnerabilities, but we may not be able to detect and remediate all vulnerabilities because the threats and techniques used to exploit the vulnerability change frequently and are often sophisticated in nature. Therefore, such vulnerabilities could be exploited but may not be detected until after a security incident has occurred. These vulnerabilities pose material risks to our business. Despite our efforts to identify and remediate vulnerabilities, if any, in our information technology systems, our efforts may not be successful and could result in a material disruption of our programs and operations. For example, the loss of clinical trial data from completed or ongoing clinical trials for any of our product candidates could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Further, we may experience delays in developing and deploying remedial measures designed to address any such identified vulnerabilities.

Applicable data security obligations may require us to notify relevant stakeholders of any security incidents, including affected individuals, customers, and regulators. Such disclosures are costly, and the disclosures or the failure to comply with such requirements, could lead to adverse consequences. If we (or a third party upon whom we rely) experience a security incident or are perceived to have experienced a security incident, we may experience adverse consequences. These consequences may include: government enforcement actions (for example, investigations, fines, penalties, audits, and inspections); additional reporting requirements and/or oversight; restrictions on processing sensitive information (including personal data); litigation (including class claims); indemnification obligations; negative publicity; reputational harm; monetary fund diversions; interruptions in our operations (including availability of data); financial loss; and other similar harms. Security incidents and attendant consequences may cause customers to stop using our products or services, deter new customers from using our products or services, and negatively impact our ability to grow and operate our business. In addition to experiencing a security incident, third parties may gather, collect, or infer sensitive information about us from public sources, data brokers, or other means that reveals competitively sensitive details about our organization and could be used to undermine our competitive advantage or market position.

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

Uncertainties in the interpretation and application of existing, new and proposed tax laws and regulations could materially affect our tax obligations and effective tax rate.

The tax regimes to which we are subject or under which we operate are unsettled and may be subject to significant change. The issuance of additional guidance related to existing or future tax laws, or changes to tax laws or regulations proposed or implemented by the current or a future U.S. presidential administration, Congress, or taxing authorities in other jurisdictions, including jurisdictions outside of the United States, could materially affect our tax obligations and effective tax rate. To the extent that such changes have a negative impact on us, including as a result of related uncertainty, these changes may adversely impact our business, financial condition, results of operations, and cash flows.

The amount of taxes we pay in different jurisdictions depends on the application of the tax laws of various jurisdictions, including the United States, to our international business activities, tax rates, new or revised tax laws, or interpretations of tax laws and policies, and our ability to operate our business in a manner consistent with our corporate structure and intercompany arrangements. The taxing authorities of the jurisdictions in which we operate may challenge our methodologies for pricing intercompany transactions pursuant to our intercompany arrangements or disagree with our determinations as to the income and expenses attributable to specific jurisdictions. If such a challenge or disagreement were to occur, and our position was not sustained, we could be required to pay additional taxes, interest, and penalties, which could result in one-time tax charges, higher effective tax rates, reduced cash flows, and lower overall profitability of our operations. Our financial statements could fail to reflect adequate reserves to cover such a contingency. Similarly, a taxing authority could assert that we are subject to tax in a jurisdiction where we believe we have not established a taxable connection, often referred to as a "permanent establishment" under international tax treaties, and such an assertion, if successful, could increase our expected tax liability in one or more jurisdictions.

Effective January 1, 2022, legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act eliminated the option to deduct research and development expenses for tax purposes in the year incurred and requires taxpayers to capitalize and subsequently amortize such expenses over five years for research activities conducted in the United States and over 15 years of research activities conducted outside the United States. Unless the United States Department of the Treasury issues regulations that narrow the application of this provision to a smaller subset of our research and development expenses or the provision is deferred, modified, or repealed by Congress, in future years we may experience a material decrease in our cash flows from operations and an offsetting similarly sized increase in our net deferred tax assets over these amortization periods. The actual impact of this provision will depend on multiple factors, including the amount of research and development expenses we will incur and whether we conduct our research and development activities inside or outside the United States and our overall net operating loss position.

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

Under current law, our federal net operating losses ("NOLs") generated in tax years beginning after December 31, 2017, may be carried forward indefinitely, but the deductibility of such federal NOLs is limited to 80% of taxable income. As of December 31, 2022, we had federal NOLs of $90.2 million. It is uncertain if and to what extent various states will conform to federal tax laws. In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation's ability to use its pre-change NOL carryforwards and other pre-change U.S. tax attributes (such as research tax credits) to offset its post-change income or taxes may be limited. It is possible that we have experienced an ownership change in the past. In addition, we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control.

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

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

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

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical FDA, SEC and other government employees and stop critical activities. If a prolonged government shutdown occurs, or if the FDA or EDA experience resource constraints, it could significantly impact the ability of the applicable regulatory agency 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.

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

There is an increasing focus from certain investors, employees, partners, and other stakeholders concerning ESG matters. While we have internal efforts directed at ESG matters and preparations for increased future disclosures, we may be perceived by certain stakeholders as not acting responsibly in connection with these matters, which could negatively impact us. Moreover, the SEC has recently proposed, and may continue to propose, certain mandated ESG reporting requirements, such as the SEC's proposed rules designed to enhance and standardize climate-related disclosures, which, if finally approved, would significantly increase our compliance and reporting costs and may also result in disclosures that certain investors or other stakeholders deem to negatively impact our reputation and/or that harm our stock price. In addition, given our business model, we currently do not report our environmental emissions and absent a legal requirement to do so we currently do not plan to report our environmental emissions, and lack of reporting could result in certain investors declining to invest in our common stock.

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

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

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

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

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

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

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

In addition, we rely on third-party manufacturers, some of whom are located in China, to manufacture API for FILSPARI and certain of our product candidates. 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 staffing shortages, COVID-19 or other health epidemic or pandemic), could impair our ability to meet commercial demand for FILSPARI, 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.

We have identified a material weakness in our internal control over financial reporting, and our financial controls and procedures may not in the future be sufficient to ensure timely and reliable reporting of financial information, which could, if not remediated, result in a material misstatement in our financial statements and could adversely affect our future results of operations and our stock price.*

A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting such that there is a reasonable possibility that a material misstatement of our annual or interim consolidated financial statements will not be prevented or detected on a timely basis.

As disclosed under Item 9A of our Annual Report on Form 10-K for the fiscal year ended December 31, 2022, there was a material weakness in our internal control over financial reporting as of December 31, 2022 because we did not design effective controls and procedures to evaluate the accounting for a certain pre-launch inventory contract affecting the timing of recognition of research and development expense.

As a result of the material weakness, we added controls for the timely accounting evaluation of research and development contracts that are intended to ensure appropriate expense recognition of certain pre-launch inventory. We do not believe that any of our remedial controls have been fully implemented or operated for a sufficient period of time or number of occurrences to allow for sufficient testing to determine the controls' operating effectiveness. If we are unable to remediate this material weakness, or are otherwise unable to maintain effective internal control over financial reporting or disclosure controls and procedures, our ability to record, process and report financial information accurately, and to prepare financial statements within required time periods, could be adversely affected, which could subject us to litigation or investigations requiring management resources and payment of legal and other expenses and negatively impact the price of our common stock. In addition, we could be subject to sanctions or investigations by the SEC or other regulatory authorities, which would require additional financial and management resources.

Furthermore, investor perceptions of our company may suffer as a result of the current material weakness or any future material weakness in our internal controls, and this could cause a decline in the market price of our stock. Any failure of our internal control over financial reporting could have a material adverse effect on our stated operating results, result in an adverse opinion on our internal control over financial reporting from our independent registered public accounting firm, and harm our reputation.

Adverse developments affecting the financial services industry could adversely affect our current and projected business operations and our financial condition and results of operations.*

Adverse developments that affect financial institutions, such as events involving liquidity that are rumored or actual, have in the past and may in the future lead to bank failures and market-wide liquidity problems. For example, on March 10, 2023, Silicon Valley Bank (SVB) was closed by the California Department of Financial Protection and Innovation, which appointed the Federal Deposit Insurance Corporation (FDIC) as receiver. Similarly, on March 12, 2023, Signature Bank and Silvergate Capital Corp. were each swept into receivership. In addition, on May 1, 2023, the FDIC seized First Republic Bank and sold its assets to JPMorgan Chase & Co. While the U.S. Department of Treasury, FDIC and Federal Reserve Board have implemented a program to provide up to $25 billion of loans to financial institutions secured by certain of such government securities held by financial institutions to mitigate the risk of potential losses on the sale of such instruments, widespread demands for customer withdrawals or other needs of financial institutions for immediate liquidity may exceed the capacity of such program, there is no guarantee that such programs will be sufficient. Additionally, it is uncertain whether the U.S. Department of Treasury, FDIC and Federal Reserve Board will provide access to uninsured funds in the future in the event of the closure of other banks or financial institutions, or that they would do so in a timely fashion.

While we have not experienced any adverse impact to our liquidity or to our current and projected business operations, financial condition or results of operations as a result of the matters relating to SVB, Signature Bank, Silvergate Capital Corp and First Republic Bank, uncertainty remains over liquidity concerns in the broader financial services industry, and our business, our business partners or industry as a whole may be adversely impacted in ways that we cannot predict at this time.

Although we assess our banking relationships as we believe necessary or appropriate, our access to cash in amounts adequate to finance or capitalize our current and projected future business operations could be significantly impaired by factors that affect the financial institutions with which we have banking relationships. These factors could include, among others, events such as liquidity constraints or failures, the ability to perform obligations under various types of financial, credit or liquidity agreements or arrangements, disruptions or instability in the financial services industry or financial markets, or concerns or negative expectations about the prospects for companies in the financial services industry. These factors could also include factors involving financial markets or the financial services industry generally. The results of events or concerns that involve one or more of these factors could include a variety of material and adverse impacts on our current and projected business operations and our financial condition and results of operations. These could include, but may not be limited to, delayed access to deposits or other financial assets or the uninsured loss of deposits or other financial assets; or termination of cash management arrangements and/or delays in accessing or actual loss of funds subject to cash management arrangements.

In addition, widespread investor concerns regarding the U.S. or international financial systems could result in less favorable commercial financing terms, including higher interest rates or costs and tighter financial and operating covenants, or systemic limitations on access to credit and liquidity sources, thereby making it more difficult for us to acquire financing on acceptable terms or at all. Any decline in available funding or access to our cash and liquidity resources could, among other risks, adversely impact our ability to meet our operating expenses, financial obligations or fulfill our other obligations, result in breaches of our financial and/or contractual obligations or result in violations of federal or state wage and hour laws. Any of these impacts, or any other impacts resulting from the factors described above or other related or similar factors not described above, could have material adverse impacts on our liquidity and our current and/or projected business operations and financial condition and results of operations.

We maintain our cash at financial institutions, often in balances that exceed federally insured limits.*

We maintain the majority of our cash and cash equivalents in accounts at banking institutions in the United States that we believe are of high quality. Cash held in these accounts often exceed the FDIC insurance limits. If such banking institutions were to fail, we could lose all or a portion of amounts held in excess of such insurance limitations. As noted above, the FDIC recently took control of SVB, Signature Bank, Silvergate Capital Corp and First Republic Bank. In the event of failure of any of the financial institutions where we maintain our cash and cash equivalents, there can be no assurance that we would be able to access uninsured funds in a timely manner or at all. Any inability to access or delay in accessing these funds could adversely affect our business and financial position.

Risks Related to our Indebtedness and Investments

Our indebtedness could adversely affect our financial condition.*

As of March 31, 2023, we had approximately $385 million of total debt outstanding, classified as long term. As a result of our indebtedness, a portion of our cash flow will be required to pay interest and principal on the 2025 Notes and 2029 Notes if the notes are not converted to shares of common stock prior to maturity. We may not generate sufficient cash flow from operations or have future borrowings available to enable us to repay our indebtedness or to fund other liquidity needs.

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

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

We expect to use cash flow from operations and outside financings to meet our current and future financial obligations, including funding our operations, debt service and capital expenditures. Our ability to make these payments depends on our future performance, which will be affected by financial, business, economic and other factors, many of which we cannot control. Our business may not generate sufficient cash flow from operations in the future, which could result in our being unable to repay indebtedness, or to fund other liquidity needs. If we do not generate sufficient cash from operations, we may be forced to reduce or delay our business activities and capital expenditures, sell assets, obtain additional debt or equity capital or restructure or refinance all or a portion of our debt, including the 2025 Notes and 2029 Notes, on or before maturity. We cannot make any assurances that we will be able to accomplish any of these alternatives on terms acceptable to us, or at all. In addition, the terms of existing or future indebtedness may limit our ability to pursue any of these alternatives. In addition, we may from time to time seek to retire or purchase our outstanding debt, including the 2025 Notes or 2029 Notes, through cash purchases and/or exchanges for equity securities, in open market purchases, privately negotiated transactions or otherwise. Such repurchases or exchanges, if any, will depend on prevailing market conditions, our liquidity requirements, contractual restrictions, and other factors. The amounts involved in any such transactions, individually or in the aggregate, may be material. Further, any such purchases or exchanges may result in us acquiring and retiring a substantial amount of such indebtedness, which could impact the trading liquidity of such indebtedness.

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

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

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

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

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

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

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

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

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

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

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

General Risk Factors

Unstable market, economic and geopolitical conditions may have serious adverse consequences on our business, financial condition and stock price.

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

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

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 Pre-Funded Warrant (incorporated by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K, filed with the SEC on March 1, 2023).
10.1†	The Company's 2023 Executive Officer Annual Bonus Plan (incorporated by reference to Exhibit 10.12 to the Company's Annual Report on Form 10-K, filed with the SEC on February 23, 2023).
10.2†	Employment Agreement, effective January 1, 2022, between the Company and Jula Inrig, M.D.
31.1	Chief Executive Officer's Certification pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Chief Financial Officer's Certification pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Chief Executive Officer's Certification pursuant to Section 906 of the Sarbanes Oxley Act of 2002
32.2	Chief Financial Officer's Certification pursuant to Section 906 of the Sarbanes Oxley Act of 2002
101.INS	Inline XBRL Instance Document
101.SCH	Inline XBRL Taxonomy Extension Schema Document
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Taxonomy Extension Presentation Linkbase Document
104	The cover page to this Quarterly Report on Form 10-Q has been formatted in Inline XBRL

† Indicates management contract or compensatory plan.

SIGNATURES

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

Date: May 4, 2023	**TRAVERE THERAPEUTICS, INC.**		
	By:	/s/ Eric M. Dube	
		Name:	Eric M. Dube
		Title:	Chief Executive Officer
	By:	/s/ Christopher Cline	
		Name:	Christopher Cline
		Title:	Chief Financial Officer

EXHIBIT 10.2

EMPLOYMENT AGREEMENT

THIS EMPLOYMENT AGREEMENT (this "***Agreement***") is effective as January 1, 2022 (the "***Effective Date***") and is entered into by and between **Travere Therapeutics, Inc**., a Delaware corporation (hereinafter the "***Company***"), and **Jula Inrig, M.D.** (hereinafter "***Executive***").

R E C I T A L S

WHEREAS, the Company and Executive wish to set forth in this Agreement the terms and conditions under which Executive will be employed by the Company on and after the Effective Date hereof;

NOW, THEREFORE, the Company and Executive, in consideration of the mutual promises set forth herein, agree as follows:

NATURE OF EMPLOYMENT

1.1 Effect of Agreement. This Agreement shall govern the terms of Executive's employment with the Company on and after the Effective Date until it is terminated by either the Company or Executive pursuant to the terms set forth in Section 6.

1.2 At-Will Employment. Executive shall continue to be employed on an at-will basis by the Company and therefore either Executive or the Company may terminate the employment relationship and this Agreement at any time, with or without Cause (as defined herein) and with or without advance notice, subject to the provisions of Section 6.

EMPLOYMENT DUTIES

2.1 Position; Responsibilities. Executive agrees to serve the Company in the position of Chief Medical Officer. Executive shall report directly to, and perform such duties as assigned by, the President and Chief Executive Officer of the Company (the "***CEO***").

2.2 Full Time Attention. During employment with the Company, Executive shall devote Executive's best efforts and full business time and attention to the business of the Company and the performance of the services customarily incident to such position and to such other services as assigned from time to time by the CEO and/or the Board of Directors of the Company. This is an exempt position, and Executive shall not be entitled to any overtime compensation.

2.3 Other Activities. Except upon the prior written consent of the CEO, Executive shall not during the period of employment engage, directly or indirectly, in any other business activity (whether or not pursued for pecuniary advantage) that is or may be competitive with, or that might place Executive in a competing position to that of the Company or any other corporation or entity that directly or indirectly controls, is controlled by, or is under common control with the Company, provided that Executive may own less than 2% of the outstanding securities of any such publicly traded competing corporation.

COMPENSATION

3.1 Base Salary. For services to be rendered hereunder, Executive shall receive a Base Salary at an annual rate of $445,000, payable semi-monthly in equal installments in accordance with the Company's normal payroll practices. In connection with the Company's annual performance review process, Executive's Base Salary may be reviewed and adjusted by the Compensation Committee of the Board of Directors (the "***Compensation Committee***") from time to time in its sole discretion.

3.2 Annual Incentive Bonus. In addition to any other bonus Executive may be awarded by the Compensation Committee, Executive shall be eligible to receive an annual incentive bonus as determined by the Compensation Committee based upon the achievement by the Company of annual corporate goals established by the Board of Directors or the Compensation Committee and Executive's individual performance during the applicable year, subject to the terms and conditions of the Executive Officer Annual Bonus Plan. Executive's annual incentive bonus at target will be 50% of Executive's Base Salary (the "***Target Annual Bonus***"). No annual incentive bonus is guaranteed, and the Compensation Committee in consultation with the independent members of the Board of Directors shall, in its sole discretion, determine whether the annual corporate goals have been attained and whether annual incentive bonus shall be paid. Except as provided in Section 6 herein, Executive must be actively employed by the Company and must not have provided any notice of resignation on the date the annual incentive bonus is paid in order to earn and receive such bonus. The annual incentive bonus, if any, shall be paid in a lump sum no later than March 15th of the fiscal year that follows the performance year, subject to applicable payroll deductions and withholdings.

3.3 Equity.

(a) Subject to approval by the Board of Directors or Compensation Committee, the Company will grant Executive the following equity awards (collectively, the "***Initial Equity Awards***"): (i) a stock option to purchase 80,000 shares of Company common stock (the "***Option***"), and (ii) a time-based restricted stock unit award covering 20,000 shares of Company common stock (the "***Time-Based RSU***"). The Option is a non-qualified stock option, has a ten (10)-year term and will vest over four (4) years, with one-fourth (1/4th) vesting on the one (1) year anniversary of the grant date and the remaining three-fourths (3/4ths) vesting over the following three (3) years in thirty-six (36) equal monthly installments. The Time-Based RSU will vest over four (4) years, with one-fourth (1/4th) vesting on each anniversary of the grant date. Except as expressly provided pursuant to the accelerated vesting provisions in Section 6 below, the vesting of each Initial Equity Award is subject to Executive's continuous service through each applicable vesting date. Executive acknowledges and agrees that each of the Initial Equity Awards is intended to be a material inducement to Executive's acceptance of the Company's offer of employment with the Company, and will be granted outside the Company's 2018 Equity Incentive Plan, as amended (the "***2018 Plan***"), but pursuant to the terms of the 2018 Plan as if such awards were granted under the 2018 Plan. The Initial Equity Awards shall also be governed by the terms and conditions of the respective applicable equity award agreements Executive will be required to execute as a condition to receiving such awards.

(b) Subject to approval by the Compensation Committee, in consultation with the independent members of the Board of Directors, Executive will be eligible to receive additional Stock Awards on terms to be determined by the Compensation Committee at the time of any such grant. The determination whether to grant any additional Stock Award to Executive is in the sole discretion of the Compensation Committee, in consultation with the independent members of the Board of Directors. For all purposes of this Agreement, "***Stock Awards***" shall mean any rights granted by the Company to Executive with respect to the common stock of the Company, including, without limitation, the Initial Equity Awards and other stock options, stock appreciation rights, restricted stock, stock bonuses and restricted stock units.

3.4. Inducement Advance. Executive shall receive a one-time cash inducement advance (the "***Inducement Advance***") in the total amount of $125,000, subject to applicable withholding, which shall be deemed earned when Executive successfully completes two (2) years of continuous employment from the Effective Date. The Inducement Advance will be paid, in advance of it being earned, within thirty (30) days following the Effective Date. Should Executive's employment terminate within twelve (12) months after the Effective Date pursuant to a Voluntary Resignation (as set forth in Section 6.7 herein) or a Termination by the Company for Cause (as set forth in Section 6.4 herein), Executive shall be required to repay to the Company 100% of the Inducement Advance. Should Executive's employment terminate after twelve (12) months but within twenty-four (24) months after the Effective Date pursuant to a Voluntary Resignation (as set forth in Section 6.7 herein) or a Termination by the Company for Cause (as set forth in Section 6.4 herein), Executive shall be required to repay to the Company 50% of the Inducement Advance. Such repayment shall be made in cash, by wire or by certified check within thirty (30) days following Executive's employment separation date.

3.5 Withholdings. All compensation and benefits payable to Executive under this Agreement or otherwise in connection with Executive's employment with the Company shall be subject to all federal, state, local taxes and other withholdings and similar taxes and payments required by applicable law.

BENEFITS

4.1 Vacation; Holidays; Time Off. Executive shall be eligible to accrue twenty (20) days of paid vacation per annum, in accordance with Company policy as in effect from time to time. Vacation accrual shall be subject to an accrual cap and shall be prorated based on the number of days worked during any calendar year. Unused accrued vacation shall, subject to the accrual cap, carry over to the following year. Executive shall also be eligible for holidays, personal days, and paid sick leave in accordance with the Company's applicable policies as in effect and as amended from time to time.

4.2 Benefits. During Executive's employment hereunder, the Company shall also provide Executive with the health insurance benefits it generally provides to its other senior management employees. As Executive becomes eligible in accordance with applicable criteria adopted by the Company, the Company shall provide Executive with the right to participate in and to receive benefits from life, accident, disability, medical, and savings plans and similar benefits made available generally to employees of the Company as such plans and benefits may be adopted by the Company. With respect to long-term disability insurance coverage, Executive will pay all premiums for such coverage with after-tax dollars, and the Company will reimburse Executive for the premium costs so paid by Executive, which reimbursement benefit shall be taxable income, subject to withholding. The amount and extent of benefits to which Executive is entitled shall be governed by the specific benefit plan as it may be amended from time to time.

4.3 Business Expense Reimbursement. During the term of this Agreement, Executive shall be entitled to receive proper reimbursement for all reasonable out-of-pocket expenses incurred by Executive (in accordance with the policies and procedures established by the Company for its senior executive officers) in performing services hereunder. Executive agrees to furnish to the Company adequate records and other documentary evidence of such expense for which Executive seeks reimbursement. Such expenses shall be reimbursed and accounted for under the policies and procedures established by the Company, and such reimbursement shall be made promptly, but in no event later than December 31 of the calendar year following the year in which such expenses were incurred by Executive.

CONFIDENTIALITY

5.1 Confidential Information. As a condition of employment, Executive represents and warrants that Executive has executed and shall abide by, the Company's standard Confidentiality Agreement, a copy of which is attached as **Exhibit A** and incorporated herein by reference.

5.2 Return of Property. All documents, records, apparatus, equipment and other physical property which is furnished to or obtained by Executive in the course of Executive's employment with the Company shall be and remain the sole property of the Company. Executive agrees that, upon the termination of employment with the Company for any reason, Executive shall promptly return all such property (whether or not it pertains to Confidential Information as defined in the Confidentiality Agreement), and shall not to make or retain copies, reproductions or summaries of any such property.

5.3 No Use of Prior Confidential Information. Executive will not intentionally disclose to the Company or use on its behalf any confidential information belonging to any of Executive's former employers or any other third party. Executive further agrees that Executive will not bring onto Company premises any unpublished documents or property belonging to any former employer or other person to whom Executive has an obligation of confidentiality. Executive hereby represents that Executive has disclosed to the Company any contract Executive has signed that may restrict Executive's activities on behalf of the Company, and that Executive is not subject to or a party to any employment agreement, non-competition covenant, or other agreement that would be breached by, or prohibit Executive from, executing this Agreement and performing fully Executive's duties and responsibilities hereunder.

TERMINATION

6.1 General. As set forth in Section 1.2 herein, Executive shall be employed on an at-will basis by the Company. Notwithstanding the foregoing, Executive's employment and this Agreement may be terminated in such manner as set forth in this Section 6: (a) Executive's Death (Section 6.2); (b) Executive's Disability (Section 6.3); (c) Termination by the Company for Cause (Section 6.4); (d) Termination by the Company without Cause (Section 6.5); (e) Termination by Executive due to a Constructive Termination (Section 6.6); or (f) Voluntary Resignation (Section 6.7).

6.2 By Death. Executive's employment and this Agreement shall terminate automatically upon the death of Executive. In such event:

(a) Bonus. The Company shall pay to Executive's beneficiaries or Executive's estate, as the case may be, a lump sum amount equal to Executive's Target Annual Bonus (as defined in Section 3.2) for the Company's fiscal year in which

Executive's death occurs multiplied by a fraction, the numerator of which is the number of full months of employment completed by Executive in such fiscal year and the denominator of which is 12. Such amount shall be paid as soon as administratively practicable, but in no event later than March 15 following the year in which Executive's death occurred.

(b) **Accrued Compensation.** The Company shall pay to Executive's beneficiaries or Executive's estate, as the case may be, any accrued Base Salary, any vested deferred compensation (other than pension plan or profit-sharing plan benefits that will be paid in accordance with the applicable plan), any benefits under any plans of the Company (other than pension and profit-sharing plans) in which Executive is a participant to the full extent of Executive's rights under such plans, any accrued vacation pay and any appropriate business expenses incurred by Executive in connection with Executive's duties hereunder, all to the date of termination (collectively, the "***Accrued Compensation***").

(c) **No Severance Compensation.** The compensation and benefits set forth in Sections 6.2(a) and (b) herein shall be the only compensation and benefits provided by the Company in the event of Executive's death and no other severance compensation or benefits shall be provided.

6.3 **By Disability.** If Executive is prevented from performing Executive's duties hereunder by reason of any physical or mental incapacity that results in Executive's satisfaction of all requirements necessary to receive benefits under the Company's long-term disability plan due to a total disability, then, to the extent permitted by law, the Company may terminate the employment of Executive and this Agreement at or after such time. In such event, and if Executive signs the General Release set forth as **Exhibit B** or such other form of release as the Company may require (the "***Release***") on or within the time period set forth therein, but in no event later than forty-five (45) days after the termination date and allows such Release to become effective (the "***Release Effective Date***"), then:

(a) **Accrued Compensation**. The Company shall pay to Executive all Accrued Compensation (as defined in Section 6.2(b) herein).

(b) **Base Salary Continuation**. The Company shall continue to pay Executive's Base Salary, less required withholdings, for a period of twelve (12) months (the "***Disability Base Salary Payments***") following Executive's separation from service; provided that the Disability Base Salary Payments shall be reduced by any insurance or other payments to Executive under policies and plans sponsored by the Company, even if premiums are paid by Executive. Subject to the provisions of Section 6.11, the Disability Base Salary Payments shall be paid in accordance with the Company's standard payroll practices; provided, however, that any amounts that would otherwise be scheduled to be paid prior to the Release Effective Date shall instead accrue and be paid during the first payroll period following the Release Effective Date, and all other payments shall be made as originally scheduled.

(c) **Bonus**. The Company shall pay to Executive a lump sum amount equal to Executive's Target Annual Bonus (as defined in Section 3.2) for the Company's then-current fiscal year multiplied by a fraction, the numerator of which is the number of full months of employment completed by Executive in the current fiscal year and the denominator of which is 12. Such payment shall be made within ten (10) days following the Release Effective Date.

(d) **Stock Awards**. The vesting of all outstanding Stock Awards held by Executive shall be accelerated such that the amount of shares vested under such Stock Awards shall equal that number of shares that would have been vested if Executive had continued to render services to the Company for 12 continuous months after the date of Executive's termination of employment.

(e) **Health Insurance Benefits**. To the extent provided by the federal COBRA law or, if applicable, state insurance laws, and by the Company's current group health insurance policies, Executive will be eligible to continue Executive's group health insurance benefits at Executive's own expense. If Executive timely elects continued coverage under COBRA, the Company shall pay Executive's COBRA premiums, and any applicable Company COBRA premiums, necessary to continue Executive's then-current coverage for a period of twelve (12) months after the date of Executive's termination of employment; *provided, however,* that any such payments will cease if Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such premiums. Executive agrees to immediately notify the Company in writing of any such enrollment. Notwithstanding the foregoing, if the Company determines, in its sole discretion, that it cannot provide the foregoing benefit without potentially incurring financial costs or penalties under applicable law (including, without limitation, Section 2716 of the Public Health Service Act), the Company shall in lieu thereof provide to Executive a taxable monthly amount to continue Executive's group health insurance coverage in effect on the date of separation from service (which amount shall be based on the premium for the first month of COBRA coverage), which payments shall be made regardless of whether Executive elects COBRA continuation coverage and shall commence in the month following the month in which Executive incurs a separation from service and shall end on the earlier of (x) the date on which Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such amounts and (y) twelve (12) months after the date of Executive's separation from service.

(f) **Disability Plans.** Nothing in this Section 6.3 shall affect Executive's rights under any disability plan in which Executive is a participant.

6.4 Termination by the Company for Cause.

(a) **No Liability.** The Company may terminate Executive's employment and this Agreement for Cause (as defined below) without liability at any time. In such event, the Company shall pay Executive all Accrued Compensation (as defined in Section 6.2(b) herein), but no other compensation or reimbursement of any kind, including without limitation, any severance compensation or benefits, shall be paid or provided, and thereafter the Company's obligations hereunder shall terminate. For clarity, the foregoing sentence shall not have any impact on any awards or other benefits that are vested as of the date of such termination.

(b) **Definition of "Cause."** For purposes of this Agreement, "***Cause***" shall mean one or more of the following:

(i) Executive's intentional commission of an act, or intentional failure to act, that materially injures the business of the Company; *provided, however,* that in no event shall any business judgment made in good faith by Executive and within Executive's defined scope of authority constitute a basis for termination for Cause under this Agreement;

(ii) Executive's intentional refusal or intentional failure to act in accordance with any lawful and proper direction or order of the Board of Directors or the CEO;

(iii) Executive's material breach of Executive's fiduciary, statutory, contractual, or common law duties to the Company (including any material breach of this Agreement, the Confidentiality Agreement, or the Company's written policies);

(iv) Executive's indictment for or conviction of, or plea of guilty or nolo contendere to, any felony or any crime involving dishonesty; or

(v) Executive's participation in any fraud or other act of willful misconduct against the Company;

provided, *however*, that in the event that any of the foregoing events is reasonably capable of being cured (as determined in the sole and absolute discretion of the Board of Directors of the Company), the Company shall provide written notice to Executive describing the nature of such event and Executive shall thereafter have ten (10) business days to cure such event.

6.5 Termination by the Company without Cause.

(a) The Company's Right. The Company may terminate Executive's employment and this Agreement without Cause (as defined in Section 6.4(b) herein) at any time by giving thirty (30) days advance written notice to Executive.

(b) Severance Benefits. If the Company terminates Executive's employment without Cause, and if Executive signs the Release on or within the time period set forth therein (but in no event later than forty-five (45) days after the termination date) and allows such Release to become effective, then:

(i) **Accrued Compensation**. The Company shall pay to Executive all Accrued Compensation (as defined in Section 6.2(b) herein).

(ii) **Cash Compensation Amount Payments**. The Company shall pay Executive an amount equal to (A) Executive's annual Base Salary plus Executive's Target Annual Bonus (as defined in Section 3.2 herein) multiplied by (B) 1.0 (the "***Cash Compensation Amount***"). Subject to the provisions of Section 6.11, the Cash Compensation Amount will be paid in equal installments on the Company's standard payroll dates over a period of twelve (12) months following Executive's separation from service; provided, however, that any amounts that would otherwise be scheduled to be paid prior to the Release Effective Date shall instead accrue and be paid during the first payroll period following the Release Effective Date, and all other payments shall be made as originally scheduled.

(iii) **Stock Awards.** The vesting of all outstanding Stock Awards held by Executive shall be accelerated such that the amount of shares vested under such Stock Awards shall equal that number of shares that would have been vested if Executive had continued to render services to the Company for twelve (12) continuous months after the date of Executive's termination of employment.

(iv) **Health Insurance Benefits**. To the extent provided by the federal COBRA law or, if applicable, state insurance laws, and by the Company's current group health insurance policies, Executive will be eligible to continue Executive's group health insurance benefits at Executive's own expense. If Executive timely elects continued coverage under COBRA, the Company shall pay Executive's COBRA premiums, and any applicable Company COBRA premiums, necessary to continue Executive's then-current coverage for a period of twelve (12) months after the date of Executive's termination of employment; *provided, however,* that any such payments will cease if Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such premiums. Executive agrees to immediately notify the Company in writing of any such enrollment.

Notwithstanding the foregoing, if the Company determines, in its sole discretion, that it cannot provide the foregoing benefit without potentially incurring financial costs or penalties under applicable law (including, without limitation, Section 2716 of the Public Health Service Act), the Company shall in lieu thereof provide to Executive a taxable monthly amount to continue Executive's group health insurance coverage in effect on the date of separation from service (which amount shall be based on the premium for the first month of COBRA coverage), which payments shall be made regardless of whether Executive elects COBRA continuation coverage and shall commence in the month following the month in which Executive incurs a separation from service and shall end on the earlier of (x) the date on which Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such amounts and (y) twelve (12) months after the date of Executive's separation from service.

6.6 Termination by Executive due to a Constructive Termination.

(a) Executive's Right. Executive may resign Executive's employment and terminate this Agreement at any time as a result of a Constructive Termination (as defined in Section 6.6(c) herein).

(b) Severance Benefits. If Executive resigns Executive's employment and terminates this Agreement as a result of a Constructive Termination, and if Executive signs the Release on or within the time period set forth therein (but in no event later than forty-five (45) days after the termination date) and allows such Release to become effective, then Executive shall receive all of the severance benefits set forth in Section 6.5(b) herein.

(c) Definition of "Constructive Termination." For purposes of this Agreement, "***Constructive Termination***" shall mean a resignation of employment and termination of this Agreement by Executive for one or more of the following actions that are taken by the Company without Executive's prior written consent:

(i) Assignment to, or withdrawal from, Executive of any duties or responsibilities that results in a material diminution in such Executive's authority, duties or responsibilities as in effect immediately prior to such change;

(ii) A material diminution in the authority, duties or responsibilities of the supervisor to whom Executive is required to report;

(iii) A material reduction by the Company of Executive's annual Base Salary;

(iv) A relocation of Executive's primary work location to a location that is more than forty (40) miles from Executive's then-current primary work location immediately prior to such relocation, provided that a relocation of Executive's primary work location to the Company's office in San Diego, California from remote work or an opportunity to relocate which is accepted by Executive in writing, in either case, will not be considered a relocation of Executive's primary work location for purposes of this definition; or

(v) A material breach by the Company of any provision of this Agreement or any other enforceable written agreement between Executive and the Company;

provided however, that Executive must first provide the Company's CEO and Board of Directors with written notice specifying the condition giving rise to a Constructive Termination within ninety (90) days following the initial existence of such condition; and Executive's notice must specify that Executive intends to terminate Executive's employment no earlier than thirty (30) days after providing such notice, the Company must be given an opportunity to cure such condition within thirty (30) days following its receipt of such notice and avoid paying benefits, and if such condition is not reasonably cured within such period, Executive must resign from all positions Executive then holds with the Company not later than thirty (30) days after the expiration of the cure period.

6.7 Voluntary Resignation. Executive may resign Executive's employment and terminate this Agreement at any time for any reason other than due to a Constructive Termination (as defined in Section 6.6(c) herein). In such event, the Company shall pay Executive all Accrued Compensation (as defined in Section 6.2(b) herein), but no other compensation or reimbursement of any kind, including without limitation, any severance compensation or benefits shall be paid, and thereafter the Company's obligations hereunder shall terminate.

6.8 Change in Control.

(a) Severance Benefits. If (i) within three (3) months prior to, or on or within twelve (12) months after, the consummation of a Change in Control (as defined in Section 6.8(b) herein), (1) the Company terminates Executive's employment and this Agreement without Cause pursuant to Section 6.5 herein or (2) Executive resigns from employment and terminates this Agreement as a result of a Constructive Termination pursuant to Section 6.6 herein, and (ii) in either event (1) or (2), Executive signs the Release on or within the time period set forth therein, but in no event later than forty-five (45) days after the termination date and allows such Release to become effective, then Executive shall receive the following severance benefits in lieu of any severance benefits set forth in Section 6.5(b) or Section 6.6(b) herein:

(i) **Accrued Compensation**. The Company shall pay to Executive all Accrued Compensation (as defined in Section 6.2(b) herein).

(ii) **CIC Cash Compensation Amount Payment**. The Company shall pay Executive an amount equal to (A) Executive's annual Base Salary plus Executive's Target Annual Bonus (as defined in Section 3.2 herein) multiplied by (B) 1.5 (collectively, the "***CIC Cash Compensation Amount***"). The CIC Cash Compensation Amount will be paid in one lump sum within ten (10) days following the Release Effective Date.

(iii) **Stock Awards**. The vesting of all outstanding Stock Awards held by Executive shall be accelerated in full, effective as of the Release Effective Date.

(iv) **Health Insurance Benefits**. To the extent provided by the federal COBRA law or, if applicable, state insurance laws, and by the Company's current group health insurance policies, Executive will be eligible to continue Executive's group health insurance benefits at Executive's own expense. If Executive timely elects continued coverage under COBRA, the Company shall pay Executive's COBRA premiums, and any applicable Company COBRA premiums, necessary to continue Executive's then-current coverage for a period of eighteen (18) months after the date of Executive's termination of employment; *provided, however,* that any such payments will cease if Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such premiums. Executive agrees to immediately notify the Company in writing of any such enrollment.

Notwithstanding the foregoing, if the Company determines, in its sole discretion, that it cannot provide the foregoing benefit without potentially incurring financial costs or penalties under applicable law (including, without limitation, Section 2716 of the Public Health Service Act), the Company shall in lieu thereof provide to Executive a taxable monthly amount to continue Executive's group health insurance coverage in effect on the date of separation from service (which amount shall be based on the premium for the first month of COBRA coverage), which payments shall be made regardless of whether Executive elects COBRA continuation coverage and shall commence in the month following the month in which Executive incurs a separation from service and shall end on the earlier of (x) the date on which Executive voluntarily enrolls in a health insurance plan offered by another employer or entity during the period in which the Company is paying such amounts and (y) eighteen (18) months after the date of Executive's separation from service.

(b) For purposes of this Agreement, a "***Change in Control***" shall have occurred if at any time following the Effective Date, any of the following events shall occur:

(i) The Company is merged, or consolidated, or reorganized into or with another corporation or other legal person, and as a result of such merger, consolidation or reorganization less than 50% of the combined voting power of the then-outstanding securities of such corporation or person immediately after such transaction are held in the aggregate by the holders of voting securities of the Company immediately prior to such transaction;

(ii) The Company sells all or substantially all of its assets or any other corporation or other legal person and thereafter, less than 50% of the combined voting power of the then-outstanding voting securities of the acquiring or consolidated entity are held in the aggregate by the holders of voting securities of the Company immediately prior to such sale;

(iii) There is a report filed after the date of this Agreement on Schedule 13D or Schedule 14D-1 (or any successor schedule, form or report), each as promulgated pursuant to the Securities Exchange Act of 1934 (the "***Exchange Act***") disclosing that any person (as the term "person" is used in Section 13(d)(3) or Section 14(d)(2) of the Exchange Act) has become the beneficial owner (as the term beneficial owner is defined under Rule 13d-3 or any successor rule or regulation promulgated under the Exchange Act) representing 50% or more of the combined voting power of the then-outstanding voting securities of the Company; or

(iv) During any period of two (2) consecutive years following the Effective Date, individuals who at the beginning of any such period constitute the directors of the Company cease for any reason to constitute at least a majority thereof unless the election to the nomination for election by the Company's shareholders of each director of the Company first elected during such period was approved by a vote of at least two-thirds of the directors of the Company then still in office who were directors of the Company at the beginning of such period.

6.9 Mitigation. Except as otherwise specifically provided herein, Executive shall not be required to mitigate the amount of any payment provided under this Agreement by seeking other employment or self-employment, nor shall the amount of any payment provided for under this Agreement be reduced by any compensation earned by Executive as a result of employment by another employer or through self-employment or by retirement benefits after the date of Executive's termination of employment from the Company, except as provided herein.

6.10 Coordination. If upon termination of employment, Executive becomes entitled to rights under other plans, contracts or arrangements entered into by the Company, this Agreement shall be coordinated with such other arrangements so that Executive's rights under this Agreement are not reduced, and that any payments under this Agreement offset the same types of payments otherwise provided under such other arrangements, but do not otherwise reduce any payments or benefits under such other arrangements to which Executive becomes entitled.

6.11 Application of Section 409A. Notwithstanding anything to the contrary herein, the following provisions apply to the extent severance benefits provided herein are subject to Section 409A of the Code and the regulations and other guidance thereunder and any state law of similar effect (collectively "***Section 409A***"). Severance benefits shall not commence until Executive has a "separation from service" for purposes of Section 409A. If Executive is a "specified employee" within the meaning of 409A(a)(2)(B)(i) of the Code, any installment payments of Disability Base Salary Payments pursuant to Section 6.3(b) or Cash Compensation Amounts pursuant to Sections 6.5(b) or 6.6(b) that are triggered by a separation from service shall be accelerated to the minimum extent necessary so that (a) the lesser of (y) the total cash severance payment amount, or (z) six (6) months of such installment payments are paid no later than March 15 of the calendar year following such termination, and (b) all amounts paid pursuant to the foregoing clause (a) will constitute separate payments for purposes of Section 1.409A-2(b)(2) of the Treasury Regulations and thus will be payable pursuant to the "short-term deferral" rule set forth in Section 1.409A-1(b)(4) of the Treasury Regulations. It is intended that if Executive is a "specified employee" within the meaning of Section 409A(a)(2)(B)(i) of the Code at the time of such separation from service the foregoing provision shall result in compliance with the requirements of Section 409A(a)(2)(B)(i) of the Code because payments to Executive will either be payable pursuant to the "short-term deferral" rule set forth in Section 1.409A-1(b)(4) of the Treasury Regulations or will not be paid until at least six (6) months after separation from service. The severance benefits are intended to qualify for an exemption from application of Section 409A or comply with its requirements to the extent necessary to avoid adverse personal tax consequences under Section 409A, and any ambiguities herein shall be interpreted accordingly.

6.12 Parachute Payments.

(a) If any payment or benefit (including payments or benefits pursuant to this Agreement) that Executive would receive in connection with a Change in Control or otherwise (“***Payment***”) would (1) constitute a “parachute payment” within the meaning of Section 280G of the Code, and (2) but for this sentence, be subject to the excise tax imposed by Section 4999 of the Code (the “***Excise Tax***”), then such Payment shall be equal to the Reduced Amount. The "Reduced Amount" shall be either (x) the largest portion of the Payment that would result in no portion of the Payment being subject to the Excise Tax or (y) the largest portion, up to and including the total, of the Payment, whichever amount, after taking into account all applicable federal, state and local employment taxes, income taxes, and the Excise Tax (all computed at the highest applicable marginal rate), results in Executive's receipt, on an after-tax basis, of the greater economic benefit notwithstanding that all or some portion of the Payment may be subject to the Excise Tax. If a reduction in payments or benefits constituting "parachute payments" is necessary so that the Payment equals the Reduced Amount, Executive shall have no rights to any additional payments and/or benefits, and reduction shall occur in the manner that results in the greatest economic benefit for Executive. If more than one method of reduction will result in the same economic benefit, the items so reduced will be reduced pro rata.

(b) In the event it is subsequently determined by the Internal Revenue Service that some portion of the Reduced Amount as determined pursuant to clause (x) in the preceding paragraph is subject to the Excise Tax, Executive agrees to promptly return to the Company a sufficient amount of the Payment so that no portion of the Reduced Amount is subject to the Excise Tax. For the avoidance of doubt, if the Reduced Amount is determined pursuant to clause (y) in the preceding paragraph, Executive will have no obligation to return any portion of the Payment pursuant to the preceding sentence.

(c) The independent registered public accounting firm engaged by the Company for general audit purposes as of the day prior to the effective date of the event described in Section 280G(b)(2)(A)(i) of the Code will perform the foregoing calculations. If the independent registered public accounting firm so engaged by the Company is serving as accountant or auditor for the individual, entity or group effecting such Change in Control or similar transaction, the Company will appoint a nationally recognized independent registered public accounting firm to make the determinations required hereunder. The Company will bear all expenses with respect to the determinations by such independent registered public accounting firm required to be made hereunder. Any good faith determinations of the independent registered public accounting firm made hereunder will be final, binding and conclusive upon the Company and Executive.

GENERAL PROVISIONS

7.1 Governing Law. The validity, interpretation, construction and performance of this Agreement and the rights of the parties thereunder shall be interpreted and enforced under California law without reference to principles of conflicts of laws.

7.2 Assignment; Successors; Binding Agreement.

(a) No Assignment. Executive may not assign, pledge or encumber Executive’s interest in this Agreement or any part thereof.

(b) **Assumption by Successor**. The Company will require any successor (whether direct or indirect, by purchase, merger, consolidation or otherwise) to all or substantially all of the business and/or assets of the Company, by operation of law or by agreement in form and substance reasonably satisfactory to Executive, to assume and agree to perform this Agreement in the same manner and to the same extent that the Company would be required to perform it if no such succession had taken place.

(c) **Binding Agreement**. This Agreement shall inure to the benefit of and be enforceable by Executive's personal or legal representatives, executors, administrators, successors, heirs, distributee, devisees and legatees. If Executive should die while any amount is at such time payable to Executive hereunder, all such amounts, unless otherwise provided herein, shall be paid in accordance with the terms of this Agreement to Executive's devisee, legates or other designee or, if there be no such designee, to Executive's estate.

7.3 **Notice.** For the purposes of this Agreement, notices and all other communications provided for in this Agreement shall be in writing and shall be deemed to have been duly given when delivered or mailed by certified or registered mail, return receipt requested, postage prepaid, addressed to the respective addresses set forth below or to such other address as either party may have furnished to the other in writing in accordance herewith, except that notice of change of address shall be effective only upon receipt.

To the Company: Travere Therapeutics, Inc.
3611 Valley Centre Drive, Suite 300
San Diego, CA 92130

To Executive: Jula Inrig, M.D.

7.4 **Modification; Waiver; Entire Agreement.** This Agreement constitutes the complete, final and exclusive embodiment of the entire agreement between Executive and the Company with regard to this subject matter. It is entered into without reliance on any promise or representation, written or oral, other than those expressly contained herein, and it supersedes any other such promises, warranties or representations. No provisions of this Agreement may be modified, waived or discharged unless such waiver, modification or discharge is agreed to in writing signed by Executive and such officer as may be specifically designated by the Board of Directors of the Company. No waiver by either party hereto at any time of any breach by the other party of, or compliance with, any condition or provision of this Agreement to be performed by such other party shall be deemed a waiver of similar or dissimilar provisions or conditions at the same or any prior or subsequent time.

7.5 **Validity.** The invalidity or unenforceability of any provision of this Agreement shall not affect the validity or enforceability of any other provision of this Agreement, which shall remain in full force and effect.

7.6 **Controlling Document.** Except to the extent described in Section 6.10, in case of conflict between any of the terms and conditions of this Agreement and any document herein referred to, the terms and conditions of this Agreement shall control.

7.7 Executive Acknowledgment. Executive acknowledges (a) that Executive has consulted with or has had the opportunity to consult with independent counsel of Executive's own choice concerning this Agreement, and has been advised to do so by the Company, and (b) that Executive has read and understands the Agreement, is fully aware of its legal effect, and has entered into it freely based on Executive's own judgment.

7.8 Dispute Resolution. To ensure the timely and economical resolution of disputes that may arise between Executive and the Company, both Executive and the Company mutually agree that pursuant to the Federal Arbitration Act, 9 U.S.C. §1-16, and to the fullest extent permitted by applicable law, Executive and the Company will submit solely to final, binding and confidential arbitration any and all disputes, claims, or causes of action arising from or relating to: (i) the negotiation, execution, interpretation, performance, breach or enforcement of this Agreement; or (ii) Executive's employment or service relationship with the Company (including but not limited to all statutory claims); or (iii) the termination of Executive's employment or service relationship with the Company (including but not limited to all statutory claims). **BY AGREEING TO THIS ARBITRATION PROCEDURE, BOTH EXECUTIVE AND THE COMPANY WAIVE THE RIGHT TO RESOLVE ANY SUCH DISPUTES THROUGH A TRIAL BY JURY OR JUDGE OR THROUGH AN ADMINISTRATIVE PROCEEDING.** The arbitrator shall have the sole and exclusive authority to determine whether a dispute, claim or cause of action is subject to arbitration under this Section and to determine any procedural questions which grow out of such disputes, claims or causes of action and bear on their final disposition. All claims, disputes, or causes of action under this Section, whether by Executive or the Company, must be brought solely in an individual capacity, and shall not be brought as a plaintiff (or claimant) or class member in any purported class or representative proceeding, nor joined or consolidated with the claims of any other person or entity. The arbitrator may not consolidate the claims of more than one person or entity, and may not preside over any form of representative or class proceeding. To the extent that the preceding sentences in this Section are found to violate applicable law or are otherwise found unenforceable, any claim(s) alleged or brought on behalf of a class shall proceed in a court of law rather than by arbitration. Any arbitration proceeding under this Section shall be presided over by a single arbitrator and conducted by JAMS, Inc. ("***JAMS***") in San Diego, California, or as otherwise agreed to by Executive and the Company, under the then applicable JAMS rules for the resolution of employment disputes (available upon request and also currently available at http://www.jamsadr.com/rules-employment-arbitration/). Executive and the Company both have the right to be represented by legal counsel at any arbitration proceeding, at each party's own expense. The arbitrator shall: (i) have the authority to compel adequate discovery for the resolution of the dispute; (ii) issue a written arbitration decision, to include the arbitrator's essential findings and conclusions and a statement of the award; and (iii) be authorized to award any or all remedies that Executive or the Company would be entitled to seek in a court of law. The Company shall pay all JAMS arbitration fees in excess of the amount of court fees that would be required of Executive if the dispute were decided in a court of law. This Section shall not apply to any action or claim that cannot be subject to mandatory arbitration as a matter of law, including, without limitation, claims brought pursuant to the California Private Attorneys General Act of 2004, as amended, the California Fair Employment and Housing Act, as amended, and the California Labor Code, as amended, to the extent such claims are not permitted by applicable law to be submitted to mandatory arbitration and such applicable law is not preempted by the Federal Arbitration Act or otherwise invalid (collectively, the "***Excluded Claims***"). In the event Executive intends to bring multiple claims, including one of the Excluded Claims listed above, the Excluded Claims may be filed with a court, while any other claims will remain subject to mandatory arbitration. Nothing in this Section is intended to prevent either

Executive or the Company from obtaining injunctive relief in court to prevent irreparable harm pending the conclusion of any such arbitration. Any final award in any arbitration proceeding hereunder may be entered as a judgment in the federal and state courts of any competent jurisdiction and enforced accordingly.

7.9 Remedies.

(a) Injunctive Relief. The parties agree that the services to be rendered by Executive hereunder are of a unique nature and that in the event of any breach or threatened breach of any of the covenants contained herein, the damage or imminent damage to the value and the goodwill of the Company's business will be irreparable and extremely difficult to estimate, making any remedy at law or in damages inadequate. Accordingly, the parties agree that the Company shall be entitled to injunctive relief against Executive in the event of any breach or threatened breach of any such provisions by Executive, in addition to any other relief (including damage) available to the Company under this Agreement or under law.

(b) Exclusive. Both parties agree that the remedy specified in Section 7.9(a) above is not exclusive of any other remedy for the breach by Executive of the terms hereof.

7.10 Counterparts. This Agreement may be executed in one or more counterparts, all of which taken together shall constitute one and the same Agreement. Counterparts may be delivered via facsimile, electronic mail (including pdf or any electronic signature process complying with the U.S. federal ESIGN Act of 2000), or other transmission method, and any counterpart so delivered shall be deemed to have been duly and validly delivered and be valid and effective for all purposes. Electronic signatures shall be deemed original signatures for purposes of this Agreement and all matters related thereto, with such electronic signatures having the same legal effect as original signatures. The parties agree that this Agreement may be electronically signed.

[Signature page to follow]

Executed by the parties as follows:

Executive **Travere Therapeutics, Inc.**

By: /s/ Jula Inrig By: /s/ Eric Dube
Jula Inrig, M.D. Eric Dube, Ph.D.
President and Chief Executive Officer

Date: January 1, 2022 Date: January 1, 2022

EXHIBIT A

Confidentiality Agreement

EXHIBIT B

General Release
[To be signed <u>on or after</u> employment termination date]

Pursuant to the terms of the Employment Agreement between Travere Therapeutics, Inc. (the “***Company***”) and Jula Inrig, M.D. (“***Executive***”) dated January 1, 2022, (the “***Agreement***”), the parties hereby enter into the following General Release (the “***Release***”):

1. Accrued Salary and Vacation. Within the timeframes proscribed by applicable law, the Company will pay Executive any accrued salary and accrued and unused vacation to which Executive is entitled by law, regardless of whether Executive signs this Release.

2. General Release. Executive hereby generally and completely releases the Company and its directors, officers, employees, shareholders, partners, agents, attorneys, predecessors, successors, parent and subsidiary entities, insurers, affiliates, and assigns (collectively the “***Released Parties***”) of and from any and all claims, liabilities and obligations, both known and unknown, arising out of or in any way related to events, acts, conduct, or omissions occurring at any time prior to or at the time that Executive signs this Release.

3. Scope of Release. This general release includes, but is not limited to: (1) all claims arising out of or in any way related to Executive’s employment with the Company or the termination of that employment; (2) all claims related to Executive’s compensation or benefits from the Company, including salary, bonuses, commissions, vacation pay, expense reimbursements, severance pay, fringe benefits, stock, stock options, or any other ownership or equity interests in the Company; (3) all claims for breach of contract, wrongful termination, and breach of the implied covenant of good faith and fair dealing (including claims based on or arising under the Agreement); (4) all tort claims, including claims for fraud, defamation, emotional distress, and discharge in violation of public policy; and (5) all federal, state, and local statutory claims, including but not limited to claims for discrimination, harassment, retaliation, attorneys’ fees, or other claims arising under the federal Civil Rights Act of 1964 (as amended), the federal Americans with Disabilities Act of 1990, the federal Age Discrimination in Employment Act (as amended) (“***ADEA***”), the federal Family and Medical Leave Act, the California Labor Code (as amended), the California Family Rights Act, and the California Fair Employment and Housing Act (as amended).

4. ADEA Waiver. Executive acknowledges that Executive is knowingly and voluntarily waiving and releasing any rights Executive may have under the ADEA, and that the consideration given for the waiver and release in the preceding paragraph is in addition to anything of value to which Executive is already entitled. If Executive is age forty (40) or older upon execution of this Release, Executive further acknowledges that Executive has been advised by this writing that, (1) Executive’s waiver and release do not apply to any rights or claims that may arise after the date Executive signs this Release; (2) Executive should consult with an attorney prior to signing this Release (although Executive may choose voluntarily not to do so); (3) Executive has twenty-one (21) days to consider this Release (although Executive may choose voluntarily to sign it earlier); (4) Executive has seven (7) days following the date Executive signs this Release to revoke it by providing written notice of revocation to the Company’s General Counsel; and (5) this Release will not be effective until the date upon which the revocation period has expired, which will be the eighth (8^{th}) calendar day after the date Executive signs it provided that Executive does not revoke it. If Executive is under forty (40) years of age upon execution of this Release, the Release will be effective upon signing and not revocable.

5. Waiver of Unknown Claims. EXECUTIVE UNDERSTANDS THAT THIS RELEASE INCLUDES A RELEASE OF ALL KNOWN AND UNKNOWN CLAIMS. Executive acknowledges that Executive has read and understands Section 1542 of the California Civil Code which reads as follows: **"A general release does not extend to claims that the creditor or releasing party does not know or suspect to exist in his or her favor at the time of executing the release and that, if known by him or her, would have materially affected his or her settlement with the debtor or released party."** Executive hereby expressly waives and relinquishes all rights and benefits under that section and any law or legal principle of similar effect in any jurisdiction with respect to Executive's respective release of claims herein, including but not limited to Executive's release of unknown and unsuspected claims.

6. Excluded Claims. Executive understands that notwithstanding the foregoing, the following are not included in the Released Claims (the "***Excluded Claims***"): (i) any rights or claims for indemnification Executive may have pursuant to any written indemnification agreement to which Executive is a party, the charter, bylaws, or operating agreements of any of the Released Parties, or under applicable law; or (ii) any rights which are not waivable as a matter of law. In addition, Executive understands that nothing in this release prevents Executive from filing, cooperating with, or participating in any proceeding before the Equal Employment Opportunity Commission, the Department of Labor, or any similar government agency, except that Executive acknowledges and agrees that Executive shall not recover any monetary benefits in connection with any such claim, charge or proceeding with regard to any claim released herein. Executive hereby represents and warrants that, other than the Excluded Claims, Executive is not aware of any claims Executive has or might have against any of the Released Parties that are not included in the Released Claims.

7. Executive Representations. Executive hereby represents that Executive has been paid all compensation owed and for all hours worked; Executive has received all the leave and leave benefits and protections for which Executive is eligible, pursuant to the Family and Medical Leave Act, the California Family Rights Act, or otherwise; and Executive has not suffered any on-the-job injury for which Executive has not already filed a workers' compensation claim.

8. Continuing Obligations. Executive acknowledges and reaffirms Executive's continuing obligations under Executive's previously signed Confidentiality Agreement and agree to abide by those continuing obligations.

9. Return of Company Property. Executive represents and warrants that Executive has returned to the Company all Company documents (and all copies thereof) and other Company property in Executive's possession or control, including, but not limited to, Company files, notes, drawings, records, business plans and forecasts, contact information, financial information, specifications, training materials, computer-recorded information, tangible property including, but not limited to, computers, credit cards, entry cards, identification badges and keys; and any materials of any kind that contain or embody any proprietary or confidential information of the Company (and all reproductions thereof). Executive represents that Executive has made a diligent search to locate any such documents, property and information within the required timeframe. In addition, if Executive has used any personally owned computer, server, e-mail system, mobile phone, portable electronic device (e.g., smartphone, iPad or the like), (collectively, "***Personal Systems***") to receive, store, prepare or transmit any Company confidential or proprietary data, materials or information, then Executive has permanently deleted and expunged all such Company confidential or proprietary information from such Personal Systems without retaining any copy or reproduction in any form (in whole or in part). Executive agrees that Executive will neither use nor possess Company property following the employment termination date. **Executive's timely compliance with this paragraph is a condition precedent to Executive's receipt of any severance benefits from the Company.**

10. Nondisparagement. Executive agrees not to disparage the Company, its parent, or its or their officers, directors, employees, shareholders, affiliates and agents, in any manner likely to be harmful to its or their business, business reputation, or personal reputation (although Executive may respond accurately and fully to any question, inquiry or request for information as required by legal process).

11. Cooperation. Executive agrees not to voluntarily (except in response to legal compulsion) assist any third party in bringing or pursuing any proposed or pending litigation, arbitration, administrative claim or other formal proceeding against the other party, or against the Company's parent or subsidiary entities, affiliates, officers, directors, employees or agents. Executive further agrees to reasonably cooperate with the other party, by voluntarily (without legal compulsion) providing accurate and complete information, in connection with such other party's actual or contemplated defense, prosecution, or investigation of any claims or demands by or against third parties, or other matters, arising from events, acts, or failures to act that occurred during the period of Executive's employment by the Company.

12. No Admission of Liability. The parties agree that this Release, and performance of the acts required by it, does not constitute an admission of liability, culpability, negligence or wrongdoing on the part of anyone, and will not be construed for any purpose as an admission of liability, culpability, negligence or wrongdoing by any party and/or by any party's current, former or future parents, subsidiaries, related entities, predecessors, successors, officers, directors, shareholders, agents, employees and assigns. The parties specifically acknowledge and agree that this Release is a compromise of disputed claims and that the Company denies any liability for any matter released herein.

Executive **Travere Therapeutics, Inc.**

By:__ By:__

Date: __ Date: __

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: May 4, 2023

/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, Christopher Cline, 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: May 4, 2023

/s/ Christopher Cline
Christopher Cline
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 March 31, 2023 (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: May 4, 2023

/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 March 31, 2023 (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: May 4, 2023

/s/ Christopher Cline
Christopher Cline
Chief Financial Officer
(Principal Financial Officer)