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 September 30, 2025

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 October 27, 2025 was 89,472,327.

TRAVERE THERAPEUTICS, INC.

Form 10-Q
For the Fiscal Quarter Ended September 30, 2025

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FORWARD-LOOKING STATEMENTS

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

Although forward-looking statements in this report reflect the good faith judgment of our management, such statements can only be based on facts and factors currently known by us. Consequently, forward-looking statements are inherently subject to risks and uncertainties and actual results and outcomes may differ materially from the results and outcomes discussed in or anticipated by the forward-looking statements. Factors that could cause or contribute to such differences in results and outcomes include, without limitation, those specifically addressed under the headings “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in our Annual Report on Form 10-K for the fiscal year ended December 31, 2024, 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, and to attain market acceptance among physicians, patients and healthcare payers.
- International trade policies, including tariffs, sanctions and trade barriers may adversely affect our business, financial condition, results of operations and prospects.
- Our clinical trials are expensive and time-consuming and may fail to demonstrate the safety and efficacy of our product candidates.
- Success in nonclinical 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 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.
- 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 a highly experienced and skilled workforce with qualified sales representatives.
- 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.
- 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 protection for the intellectual property relating to our technology and products, their value will be adversely affected.
- 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.

- 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.
- We will likely experience fluctuations in operating results and could incur substantial losses.
- Negative publicity regarding any of our products could impair our ability to market any such product and may require us to spend time and money to address these issues.
- We may need substantial funding and may be unable to raise capital when needed.
- We may not receive some or all of the potential milestone and/or royalty payments from our corporate and licensing transactions.
- 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)

	September 30, 2025	December 31, 2024
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 110,930	$ 58,535
Marketable debt securities, at fair value	143,600	312,166
Accounts receivable, net	82,984	27,116
Inventory	5,548	6,200
Prepaid expenses and other current assets	28,048	12,685
Total current assets	371,110	416,702
Long-term inventory	31,999	35,656
Property and equipment, net	4,320	5,336
Operating lease right of use assets	11,464	14,295
Intangible assets, net	109,435	103,974
Other assets	10,253	18,162
Total assets	$ 538,581	$ 594,125
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 18,225	$ 23,534
Accrued expenses	105,762	86,028
Convertible debt, current portion	—	68,678
Operating lease liabilities, current portion	5,734	5,405
Other current liabilities	5,353	17,106
Total current liabilities	135,074	200,751
Convertible debt, less current portion	311,370	310,310
Operating lease liabilities, less current portion	12,645	17,191
Other non-current liabilities	5,928	6,796
Total liabilities	465,017	535,048
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 September 30, 2025 and December 31, 2024	—	—
Common stock $0.0001 par value; 200,000,000 shares authorized; 89,456,626, and 87,452,835 issued and outstanding as of September 30, 2025 and December 31, 2024, respectively	9	9
Additional paid-in capital	1,550,051	1,506,315
Accumulated deficit	(1,475,442)	(1,447,167)
Accumulated other comprehensive loss	(1,054)	(80)
Total stockholders' equity	73,564	59,077
Total liabilities and stockholders' equity	$ 538,581	$ 594,125

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Net product sales	$ 113,150	$ 61,001	$ 283,852	$ 153,161
License and collaboration revenue	51,709	1,897	77,187	5,227
Total revenue	164,859	62,898	361,039	158,388
Operating expenses:				
Cost of goods sold	1,585	1,626	7,786	5,191
Research and development	51,890	51,679	148,141	155,429
Selling, general and administrative	86,453	65,619	235,508	194,618
In-process research and development	—	—	—	65,205
Restructuring	—	123	—	1,035
Total operating expenses	139,928	119,047	391,435	421,478
Operating income (loss)	24,931	(56,149)	(30,396)	(263,090)
Other income, net:				
Interest income	3,047	3,570	10,129	14,022
Interest expense	(2,751)	(2,777)	(8,452)	(8,365)
Other income (expense), net	487	520	511	(2,737)
Total other income, net	783	1,313	2,188	2,920
Income (loss) from continuing operations before income tax	25,714	(54,836)	(28,208)	(260,170)
Income tax (provision) benefit on continuing operations	(8)	84	(67)	(192)
Income (loss) from continuing operations, net of tax	25,706	(54,752)	(28,275)	(260,362)
Loss from discontinued operations, net of tax	—	(59)	—	(919)
Net income (loss)	$ 25,706	$ (54,811)	$ (28,275)	$ (261,281)
Per share data				
Net income (loss) per common share, basic	$ 0.29	$ (0.70)	$ (0.32)	$ (3.37)
Net income (loss) per common share, diluted	$ 0.28	$ (0.70)	$ (0.32)	$ (3.37)
Weighted average common shares outstanding, basic	89,230,420	77,779,379	88,847,209	77,473,161
Weighted average common shares outstanding, diluted	102,618,560	77,779,379	88,847,209	77,473,161
Comprehensive income (loss):				
Net income (loss)	$ 25,706	$ (54,811)	$ (28,275)	$ (261,281)
Foreign currency translation (loss) gain	(284)	(803)	(702)	230
Unrealized gain (loss) on marketable debt securities	42	1,421	(272)	210
Comprehensive income (loss)	$ 25,464	$ (54,193)	$ (29,249)	$ (260,841)

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 September 30, 2025						Three Months Ended September 30, 2024					
	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' Deficit
	Shares	Amount					Shares	Amount				
Balance - June 30	**89,102,347**	**$ 9**	**$1,534,698**	**$ (812)**	**$ (1,501,148)**	**$ 32,747**	**76,456,562**	**$ 8**	**$1,348,865**	**$ (1,634)**	**$ (1,332,092)**	**$ 15,147**
Share based compensation	—	—	10,901	—	—	10,901	—	—	7,729	—	—	7,729
Issuance of common stock under the equity incentive plan and proceeds from exercise	354,279	—	4,090	—	—	4,090	202,480	—	571	—	—	571
Employee stock purchase program purchase and expense	—	—	362	—	—	362	—	—	292	—	—	292
Foreign currency translation adjustments	—	—	—	(284)	—	(284)	—	—	—	(803)	—	(803)
Unrealized gain on marketable debt securities	—	—	—	42	—	42	—	—	—	1,421	—	1,421
Net income (loss)	—	—	—	—	25,706	25,706	—	—	—	—	(54,811)	(54,811)
Balance - September 30	**89,456,626**	**$ 9**	**$1,550,051**	**$ (1,054)**	**$ (1,475,442)**	**$ 73,564**	**77,909,042**	**$ 8**	**$1,357,457**	**$ (1,016)**	**$ (1,386,903)**	**$ (30,454)**

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)

	Nine Months Ended September 30, 2025						Nine Months Ended September 30, 2024					
	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' Deficit
	Shares	Amount					Shares	Amount				
Balance - December 31	**87,452,835**	**$ 9**	**$1,506,315**	**$ (80)**	**$ (1,447,167)**	**$ 59,077**	**75,367,117**	**$ 7**	**$1,327,881**	**$ (1,456)**	**$ (1,125,622)**	**$ 200,810**
Share based compensation	—	—	32,410	—	—	32,410	—	—	26,763	—	—	26,763
Issuance of common stock under the equity incentive plan and proceeds from exercise	1,883,440	—	8,796	—	—	8,796	1,052,425	1	571	—	—	572
Employee stock purchase program purchase and expense	120,351	—	2,530	—	—	2,530	239,500	—	2,242	—	—	2,242
Foreign currency translation adjustments	—	—	—	(702)	—	(702)	—	—	—	230	—	230
Unrealized (loss) gain on marketable debt securities	—	—	—	(272)	—	(272)	—	—	—	210	—	210
Net loss	—	—	—	—	(28,275)	(28,275)	—	—	—	—	(261,281)	(261,281)
Balance - September 30	**89,456,626**	**$ 9**	**$1,550,051**	**$ (1,054)**	**$ (1,475,442)**	**$ 73,564**	**77,909,042**	**$ 8**	**$1,357,457**	**$ (1,016)**	**$ (1,386,903)**	**$ (30,454)**

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

	For the Nine Months Ended September 30,	
	2025	**2024**
Cash Flows From Operating Activities:		
Net loss	$ (28,275)	$ (261,281)
Net loss from discontinued operations	—	(919)
Net loss from continuing operations	(28,275)	(260,362)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	42,606	31,464
Share based compensation	33,404	27,699
Foreign currency remeasurement impact	(2,466)	—
In-process research and development	—	65,205
Other	(1,500)	5,022
Changes in operating assets and liabilities:		
Accounts receivable	(55,641)	(4,017)
Inventory	3,839	(3,486)
Prepaid expenses and other current and non-current assets	(8,104)	(4,903)
Accounts payable	(6,332)	(18,573)
Accrued expenses	11,501	(38,794)
Deferred revenue	(2,815)	(5,557)
Other current and non-current liabilities	(9,111)	4,909
Net cash used in operating activities - continuing operations	(22,894)	(201,393)
Net cash used in operating activities - discontinued operations	—	(362)
Net cash used in operating activities	(22,894)	(201,755)
Cash Flows From Investing Activities:		
Proceeds from the sale and maturity of marketable debt securities	241,175	271,285
Purchase of marketable debt securities	(69,217)	—
Purchase of fixed assets	(375)	(82)
Purchase of intangible assets	(38,918)	(26,569)
Other	66	—
Payment of milestone	—	(65,000)
Net cash provided by investing activities	132,731	179,634
Cash Flows From Financing Activities:		
Payment of guaranteed minimum royalty	(1,575)	(1,575)
Repayment of 2025 convertible senior notes	(68,904)	—
Proceeds from exercise of stock options	8,796	571
Proceeds from issuances under the employee stock purchase plan	1,537	1,307
Net cash (used in) provided by financing activities	(60,146)	303
Effect of exchange rate changes on cash	2,704	51
Net increase (decrease) in cash and cash equivalents	52,395	(21,767)
Cash and cash equivalents, beginning of year	58,535	58,176
Cash and cash equivalents, end of period	**$ 110,930**	**$ 36,409**

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

TRAVERE THERAPEUTICS, INC. AND SUBSIDIARIES
NOTES TO THE UNAUDITED 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 and metabolic diseases. The Company regularly evaluates and, where appropriate, acts on opportunities to expand its product pipeline and approved products through licenses and acquisitions of products in areas that will serve rare disease patients with serious unmet medical need and that the Company believes offer attractive growth characteristics.

Approved Products:

FILSPARI® (sparsentan)

On September 5, 2024, the FDA granted full approval of FILSPARI® (sparsentan) to slow kidney function decline in adults with primary Immunoglobulin A nephropathy (IgAN) who are at risk of disease progression. FILSPARI is the only oral, once-daily, non-immunosuppressive medication that directly targets glomerular injury in the kidney by blocking two critical pathways of IgAN disease progression (endothelin-1 and angiotensin II).

FILSPARI had previously been granted accelerated approval in February 2023 based on the surrogate marker of proteinuria. Full approval was based on positive long-term confirmatory results from the PROTECT Study demonstrating that FILSPARI significantly slowed kidney function decline over two years compared to irbesartan.

In September 2021, the Company entered into a license and collaboration agreement (the "License Agreement") with Vifor (International) Ltd. ("CSL Vifor"). In April 2024, the Company and CSL Vifor announced that the European Commission had granted conditional marketing authorization (“CMA”) for FILSPARI (sparsentan) for the treatment of adults with primary IgAN with a urine protein excretion ≥1.0 g/day (or urine protein-to-creatinine ratio ≥0.75 g/g), and in April 2025, the Company and CSL Vifor announced that the European Commission has converted the CMA into a standard marketing authorization (“MA”) for FILSPARI for the treatment of adults with primary IgAN with a urine protein excretion ≥1.0 g/day (or urine protein-to-creatinine ratio ≥0.75 g/g). The MA is granted for all member states of the European Union, as well as in Iceland, Liechtenstein and Norway. As a result of the standard MA approval, the Company received a regulatory milestone payment of $17.5 million in May 2025 under the terms of the License Agreement. Additionally, in September 2025, the Company recognized a $40.0 million milestone for market access initiatives in certain countries; payment of the milestone was received in the fourth quarter of 2025. FILSPARI became commercially available in Europe under the CMA in August 2024, with an initial launch in Germany and Austria. In October 2024, the Company and CSL Vifor announced that Swissmedic has granted temporary marketing authorization for FILSPARI for the treatment of adults with primary IgAN with a urine protein excretion ≥1.0 g/day (or urine protein-to-creatinine ratio ≥0.75 g/g). In April 2025, the Medicines and Healthcare products Regulatory Agency (MHRA) in the UK converted its conditional approval of FILSPARI in IgAN to standard approval.

In January 2024, the Company entered into an exclusive licensing agreement with Renalys Pharma, Inc. ("Renalys"), to bring sparsentan for the treatment of IgAN to patients in Japan and other countries in Asia. Renalys holds regional rights to sparsentan for Japan, South Korea, Taiwan, Brunei, Cambodia, Indonesia, Laos, Malaysia, Myanmar, the Philippines, Singapore, Thailand, and Vietnam. Following successful meetings with the Pharmaceuticals and Medical Devices Agency (PMDA) in 2023, in the second quarter of 2024 Renalys initiated an open label registration study of sparsentan in Japan to support potential approval of sparsentan in Japan. In July 2024, Renalys announced that the first patient was dosed in the study, and in January 2025, Renalys announced achievement of full enrollment in the study. In December 2024, Renalys announced that sparsentan received Orphan Drug Designation from the Japanese Ministry of Health, Labour and Welfare for the indication of primary IgA nephropathy as of November 27, 2024. In October 2025, Renalys announced that it had completed data collection for the primary endpoint in the Phase 3 clinical trial of sparsentan for IgAN and that it had reached an agreement with the PMDA regarding development plans for two new Phase 3 clinical trials of sparsentan, one investigating the use of sparsentan in focal segmental glomerulosclerosis (FSGS) and the other in Alport syndrome, in Japan. Under the terms of the licensing agreement, Renalys is responsible for development, regulatory matters, and commercialization in the licensed territories.

Thiola® and Thiola EC® (tiopronin tablets)

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.

Clinical-Stage Programs:

Sparsentan for the treatment of FSGS

Sparsentan remains a novel investigational product candidate which has been granted Orphan Drug Designation for the treatment of FSGS in the U.S. and the European Economic Area countries (the “EEA”). In December 2023, the Company announced that it had completed a planned Type C meeting with the FDA to discuss previously reported results from the Phase 3 DUPLEX Study of sparsentan in FSGS. The FDA acknowledged the high unmet need for approved therapies as well as the challenges in studying FSGS but indicated that the two-year results from the Phase 3 DUPLEX Study alone were not sufficient to support an sNDA submission. In February 2025, the Company announced that it had completed a Type C meeting with the FDA and in March 2025, the Company announced that it had submitted an sNDA to the FDA seeking traditional approval of FILSPARI for the treatment of FSGS. In May 2025, the Company announced that the FDA accepted the sNDA, assigned a Prescription Drug User Fee Act ("PDUFA") target action date of January 13, 2026, and initially indicated that it planned to hold an advisory committee meeting to discuss the application. In September 2025, following further review of the sNDA, the FDA informed the Company that an advisory committee meeting is no longer needed. The sNDA remains under review by the FDA with a PDUFA

target action date of January 13, 2026. The sNDA submission is supported by results from the Phase 3 DUPLEX Study and the Phase 2 DUET Study. If approved, FILSPARI could become the first and only FDA-approved medicine indicated for FSGS.

Pegtibatinase

Pegtibatinase 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. In May 2023, the Company announced positive topline results from cohort 6 in the Phase 1/2 COMPOSE Study. In December 2023, the Company initiated the pivotal Phase 3 HARMONY Study to support the potential approval of pegtibatinase for the treatment of classical HCU. The HARMONY Study is a global, randomized, multi-center, double-blind, placebo-controlled Phase 3 clinical trial designed to evaluate the efficacy and safety of pegtibatinase as a novel treatment to reduce total homocysteine ("tHcy") levels. In the beginning of 2024, the first patients were dosed in the HARMONY Study.

In September 2024, the Company announced a voluntary pause of enrollment in the Phase 3 HARMONY Study. The voluntary enrollment pause enables the Company to work to address necessary process improvements in manufacturing scale-up to support commercial scale manufacturing as well as full enrollment in the HARMONY Study. Patients currently enrolled in pegtibatinase studies continue to receive study medication from small scale batches which are unaffected by the scale-up process. Currently enrolled patients will be able to continue on study medication as scheduled for the duration of the trials they are participating in. The voluntary enrollment pause was enacted following the Company's determination that the desired drug substance profile was not achieved in the initial scale-up process. The Company has manufactured the first commercial-scale batches designed to enable the continuation of the Phase 3 program.

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

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, 2024, filed with the SEC on February 21, 2025. 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, 2024 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. These reclassifications did not have an impact on total assets or total liabilities in the Consolidated Balance Sheets or net loss in the Consolidated Statements of Operations and Comprehensive Loss.

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 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 judgment 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 has been satisfied. Revenue from collaboration and licensing agreements may also include sales of inventory, at cost plus a margin, which is recorded in license and collaboration revenue.

The Company utilizes significant judgment 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 judgment 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 ingredients to the Company's collaboration partners, at cost or at cost plus a margin.

The following table summarizes cost of goods sold for the three and nine months ended September 30, 2025 and 2024 (*in thousands*):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Cost of goods sold - product sales	$ 1,308	$ 1,626	$ 4,083	$ 5,059
Cost of goods sold - license and collaboration	277	—	3,703	132
Total cost of goods sold	$ 1,585	$ 1,626	$ 7,786	$ 5,191

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.

Prior to the February 2023 FDA accelerated approval of FILSPARI (sparsentan), the Company expensed the production of active pharmaceutical ingredients purchased to support the commercial launch of FILSPARI, in research and development expenses. For the three and nine months ended September 30, 2025 and 2024, sales of FILSPARI primarily consisted of zero-cost inventories. As of September 30, 2025, the Company had approximately $0.9 million of zero-cost inventory remaining, the majority of which the Company expects will be consumed in 2025. The Company began capitalizing inventory costs associated with FILSPARI following the February 2023 accelerated approval.

Research and Development Expenses

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

Earnings (Loss) Per Share

The Company calculates basic earnings per share by dividing net income/(loss) by the weighted average number of shares outstanding during the period. Pre-funded warrants issued and sold by the Company to purchase shares of its common stock are included in the calculation of basic net loss per common share if the exercise price of the pre-funded warrant represents little consideration and is non-substantive in relation to the price paid for the warrant, and if the warrants are immediately exercisable with no further vesting conditions or contingencies associated with them.

The Company's diluted earnings/(loss) per share computation includes the effect, if any, of shares that would be issuable upon the exercise of outstanding stock options, convertible debt and RSUs, reduced by the number of shares which are assumed to be purchased by the Company from the resulting proceeds at the average market price during the year, when such amounts are dilutive to the earnings per share calculation. The potential dilutive effect of stock options and RSUs during the period are calculated in accordance with the treasury stock method, but are excluded if the effect is anti-dilutive. The potential dilutive effect of convertible debt outstanding during the period is calculated using the if-converted method assuming the conversion of convertible debt as of the earliest period reported or at the date of issuance, if later, but is excluded if the effect is anti-dilutive. In accordance with ASC 260, Earnings per Share, if a company had a discontinued operation, the company uses income from continuing operations, adjusted for preferred dividend and similar adjustments, as its control number to determine whether potential common shares are dilutive.

Intangible Assets with Cost Accumulation Model

In 2014, the Company entered into a license agreement with Mission Pharmacal ("Mission") in which the Company obtained the exclusive right to license the trademark of Thiola ("Mission License Agreement"). 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 intangible asset under the cost accumulation model ("Thiola Intangible"). The additional cost basis is subsequently amortized over the remaining estimated useful 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 September 30, 2025 as such royalties are not yet probable and estimable.

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”). The acquisition of the Ligand License Agreement qualified as an asset acquisition under the principles of ASC 805 in effect at the time of acquisition. The license agreement requires the Company to make royalty payments based on net sales of FILSPARI (sparsentan) and milestone payments. The liabilities for royalties and milestone payments are recognized in the period in which they become probable and estimable, which is typically in the period corresponding with the respective sales or achievement of the milestone. The Company records an offsetting increase to the cost basis of the intangible asset under the cost accumulation model following the approval of FILSPARI. The additional cost basis is subsequently amortized over the remaining estimated useful life.

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 judgments 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.

Equity Securities

The Company applies the equity method of accounting for investments when it has significant influence, but no controlling interest in the investee. Judgment regarding the level of influence over each equity method investment includes key factors such as ownership interest, representation on the board of directors, participation in joint steering committees and material intercompany transactions. Upon investment, the Company evaluates any basis difference between the

carrying value and fair value of the Company's proportionate share of the investee's net assets. Basis differences relating to in-process research and development ("IPR&D") are expensed when the investee is not considered a business as defined in ASC 805, *Business Combinations,* due to substantially all of the estimated fair value of the gross assets being concentrated in a group of similar IPR&D assets with no alternative future use. For the three and nine months ended September 30, 2025, the Company did not have any basis adjustments. For the three and nine months ended September 30, 2024, the Company recognized zero and $3.4 million, respectively, in other (expense) income, net in the Company's Consolidated Statements of Operations and Comprehensive Loss for basis adjustments and reduced the equity method investment's carrying value to zero, as the Company's proportionate share of the basis difference exceeded the carrying value. See Note 6 for further discussion. Investments accounted for using the equity method are reported on a lag of up to three months if the financial statements of the investee are not available in sufficient time for the Company to apply the equity method as of the current reporting date.

Discontinued Operations

Discontinued operations is presented when there is a disposal of a component or a group of components that in the Company's judgment represents a strategic shift that will have a major effect on the Company's operations and financial results. Results of operations directly related to discontinued operations are aggregated into a single line item in the Consolidated Statements of Operations and Comprehensive Loss for all periods presented. See Note 18 for further discussion. Unless otherwise noted, amounts and disclosures throughout the Notes to the unaudited consolidated financial statements relate to the Company's continuing operations.

Restructuring

Restructuring charges consist primarily of employee severance, one-time termination benefits related to the reduction of its workforce, and other costs. Liabilities for costs associated with a restructuring activity are recognized when the liability is incurred and are measured at fair value. One-time termination benefits are expensed at the date the entity notifies the employee, unless the employee must provide future service, in which case the benefits are expensed ratably over the service period. Termination benefits are calculated based on regional benefit practices and local statutory requirements.

In December 2023, the Company initiated a restructuring plan that resulted in a reduction of its workforce, primarily impacting non-field-based employees. Restructuring costs were primarily comprised of one-time termination benefits including severance, continuation of health insurance coverage, and other benefits for a specified period of time. As of December 31, 2024, the Company had recognized a total of $13.8 million in connection with the restructuring and it is no longer incurring restructuring expenses.

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.

In September 2025, the FASB issued Accounting Standards Update ("ASU") No. 2025-06—Intangibles—Goodwill and Other—Internal-Use Software (Subtopic 350-40): Targeted Improvements to the Accounting for Internal-Use Software. This ASU removed the language around project stages that was used to assess when costs could be capitalized for an internal-use software. The update also requires internal-use software to be disclosed under the ASC 360 Property, Plant, and Equipment guidance. The guidance is effective for annual periods beginning after December 15, 2027. The Company is currently assessing the impact of this ASU on the Company's accounting policies and the financial statements.

In July 2025, the FASB issued ASU No. 2025-05—Financial Instruments—Credit Losses (Topic 326): Measurement of Credit Losses for Accounts Receivable and Contract Assets. This ASU added a practical expedient that assumes that current conditions as of the balance sheet date do not change for the remaining life of the asset when estimating expected credit losses for current accounts receivable and current contract assets. The guidance is effective for annual periods beginning after December 15, 2025. The Company is currently evaluating the impact of the adoption of this standard on the accounting for credit losses.

In July 2025, the One Big Beautiful Bill Act ("OBBBA") was signed into law, which enacts significant changes to U.S. tax and related laws. Some of the provisions of the new tax law affecting corporations include but are not limited to expensing of domestic research expenses, calculating the limit on the interest expense based on 30 percent of EBITDA (rather than EBIT), and 100 percent bonus depreciation on eligible property acquired after January 19, 2025. There is no material change to the Company's effective income tax rate and its net deferred federal income tax assets from the new tax law as the Company maintains a full valuation allowance.

In November 2024, the FASB issued ASU No. 2024-04, Debt with Conversion and Other Options (Subtopic 470-20): Induced Conversions of Convertible Debts Instruments. This ASU clarifies the requirements for determining whether to account for certain early settlements of convertible debt instruments as induced conversions or extinguishments. The guidance is effective for fiscal years beginning after December 15, 2025, with early adoption permitted for entities that have adopted ASU 2020-06. The Company is currently evaluating the impact of the adoption of this standard on the accounting for the Company's convertible notes.

In November 2024, the FASB issued ASU No. 2024-03, Income Statement - Reporting Comprehensive Income - Expense Recognition Disclosures. This ASU will require entities to provide enhanced disclosures related to certain expense categories included in income statement captions. The ASU aims to increase transparency and provide investors with more detailed information about the nature of expenses reported on the face of the income statement. The new standard does not change the requirements for the presentation of expenses on the face of the income statement. Under this ASU, entities are required to disaggregate, in a tabular format, expense captions presented on the face of the income statement — excluding earnings or losses from equity method investments — if they include any of the following expense categories: purchases of inventory, employee compensation, depreciation, intangible asset amortization, and depreciation or depletion. For any remaining items within each relevant expense caption, entities must provide a qualitative description of the nature of those expenses. The new ASU is effective, as clarified by ASU No. 2025-01, for annual reporting periods beginning after December 15, 2026 and interim reporting periods within annual reporting periods beginning after December 15, 2027. Early adoption is permitted. The Company is currently evaluating the impact of the adoption of this standard on the related disclosures.

In December 2023, the FASB issued ASU No. 2023-09, Improvements to Income Tax Disclosures. This ASU does not change accounting for income taxes but requires new disclosures focusing on two areas, the effective rate reconciliation and taxes paid. This new standard is effective for public business entities for annual periods beginning after December 15, 2024. The Company plans to adopt the standard as required for the December 31, 2025 annual financial statements and interim periods thereafter. The Company does not expect the adoption of ASU 2023-09 to have a material impact on the consolidated financial statements.

NOTE 3. REVENUE RECOGNITION

Product Sales, Net

Product sales consist of FILSPARI 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 representing over 98% of the Company's net product sales.

The Company sells FILSPARI to two direct-to-patient specialty pharmacies in the United States. The Company sells its tiopronin 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 tiopronin products, product sales are recognized upon delivery to the patient. The Company receives payments from sales of its tiopronin 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 nine months ended September 30, 2025 and 2024, adjustments to net product revenue related to performance obligations satisfied in previous periods were $1.0 million and $0.5 million, respectively.

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 accrued expenses 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 accrued expenses 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.

Other Fees: The Company pays service fees to certain customers based on a contractually fixed percentage of the wholesale acquisition cost ("WAC") and fees for data. Other fees are recorded as an offset to revenue based on contractual terms at the time revenue from the sale is recognized.

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 and nine months ended September 30, 2025 and 2024 (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
FILSPARI	$ 90,900	$ 35,619	$ 218,668	$ 82,578
Tiopronin products	22,250	25,382	65,184	70,583
Total net product sales	$ 113,150	$ 61,001	$ 283,852	$ 153,161

NOTE 4. COLLABORATION AND LICENSE AGREEMENTS

License Agreement with CSL Vifor

In September 2021, the Company entered into a license and collaboration agreement (“CSL Vifor License Agreement”) with Vifor (International) Ltd. (“CSL Vifor”), pursuant to which the Company granted an exclusive license to CSL Vifor for the commercialization of FILSPARI in Europe, Australia and New Zealand. In June 2025, the CSL Vifor License Agreement was amended in order to, among other things, expand the license to cover the following additional countries: Bahrain, Brazil, Chile, Israel, Kuwait, Oman, Qatar, Saudi Arabia and the United Arab Emirates (together with Europe, Australia and New Zealand, the "CSL Vifor Licensed Territories") and to provide that the license rights to each additional country will revert to the Company if CSL Vifor does not take certain specified actions within specified timelines with respect to such country. CSL Vifor also has first right of negotiation to expand the licensed territories into Canada and/or Mexico. Under the terms of the CSL Vifor 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 FILSPARI in the CSL Vifor Licensed Territories. For the three and nine months ended September 30, 2025, the Company recognized $40.0 million for a market access milestone as the amount was probable of being achieved at September 30, 2025, and was subsequently received in the fourth quarter of 2025. For the nine months ended September 30, 2025, the Company recognized a regulatory milestone of $17.5 million as a result of the European Commission granting standard MA approval.

Licensing Agreement with Renalys

In January 2024, the license agreement (“Renalys License Agreement”) between the Company and Renalys came into effect. Pursuant to the terms of the Renalys License Agreement, the Company granted an exclusive license to Renalys for the development and commercialization of sparsentan in Japan, South Korea, Taiwan and other specified Asian countries ("Renalys Licensed Territories"). Under the terms of the Renalys License Agreement, the Company received a non-refundable upfront payment and will be eligible to receive up to $120.0 million in aggregate regulatory, development and sales-based milestones. The Company is also entitled to receive tiered double-digit to mid-20 percent royalties of annual net sales of sparsentan in the Renalys Licensed Territories. In addition, the Company received an option to purchase shares of common stock of Renalys (“Option Agreement”), which it exercised in January 2024. The Company also had the option to purchase all equity securities of Renalys at any time prior to the top-line results of the Phase 3 trial in Japan (“Buyout Right”).

Under the Renalys License Agreement, Renalys will be responsible for all development and commercialization activities in the Renalys Licensed Territories. The Renalys License Agreement will remain in effect, unless terminated earlier, until the expiration of all royalty terms for sparsentan in the Renalys Licensed Territories. Each party has the right to terminate the Renalys License Agreement for the other party’s uncured material breach or insolvency, or if the time required for performance under the Renalys License Agreement by the other party is extended due to a force majeure event that continues for nine months or more. Renalys may terminate the Renalys License Agreement for any reason upon prior written notice to the Company. The Company may terminate the Renalys License Agreement if Renalys abandons development in Japan or South Korea prior to first commercial sales of sparsentan in either Japan or South Korea.

The Company assessed the Renalys 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 a joint steering committee overseeing the development and commercial 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 Renalys License Agreement totaled $8.3 million, consisting of the fixed non-refundable upfront payment, milestone payment and estimated fair value of the Option Agreement. The variable development-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 initial 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 has been satisfied.

The Company concluded that Renalys represents a customer and applied relevant guidance from ASC 606 to evaluate the accounting under the Renalys License Agreement. In accordance with this guidance, the Company concluded that the promise to grant the license is distinct, resulting in one performance obligation as the license has stand-alone functionally at contract inception. The Buyout Right precludes transferring control of the license to Renalys under ASC 606 and the Company’s option to repurchase the common stock at a price greater than the original license premium results in accounting for the Renalys License Agreement as a financing arrangement. The transaction price was originally recorded in other current liabilities as a result of the Buyout Right. During the three months ended September 30, 2025, the Buyout Right was relinquished and the Company recognized the entire Renalys deferred revenue balance of $9.3 million in license and collaboration revenue on the Consolidated Statements of Operations and Comprehensive Loss.

See Note 6 for further discussion of VIEs.

For the three months ended September 30, 2025, the Company recognized $51.7 million in license and collaboration revenue, which included a market access milestone of $40.0 million associated with the CSL Vifor License Agreement, $9.3 million in license revenue associated with the Renalys License Agreement and $2.4 million for royalties earned on net sales of FILSPARI in the CSL Vifor Licensed Territories. For the nine months ended September 30, 2025 the Company recognized $77.2 million in license and collaboration revenue, which included regulatory and market access milestones totaling $57.5 million associated with the CSL Vifor License Agreement, $9.3 million associated with the Renalys License Agreement, the sale of $3.8 million of active pharmaceutical ingredients, $2.9 million for clinical development activities, based upon the ratio of costs incurred to total estimated costs and $3.7 million for royalties earned on net sales of FILSPARI in the CSL Vifor Licensed Territories.

For the three and nine months ended September 30, 2024, the Company recognized $1.8 million and $5.1 million, respectively, 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 September 30, 2025 and December 31, 2024 was zero and $2.8 million, respectively.

NOTE 5. MARKETABLE DEBT SECURITIES

The Company's marketable debt securities as of September 30, 2025 and December 31, 2024 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*):

	September 30, 2025	December 31, 2024
Marketable debt securities:		
Commercial paper	$ 45,465	$ 73,325
Corporate debt securities	93,134	203,816
Securities of government sponsored entities	5,001	35,025
Total available-for-sale marketable debt securities	$ 143,600	$ 312,166

In addition to funding operations, the decrease in the marketable debt securities balance as of September 30, 2025 is due to the repayment of the 2025 Notes in September 2025. See Note 10 for further discussion of Convertible Notes Payable.

The following is a summary of short-term marketable debt securities classified as available-for-sale as of September 30, 2025 (*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	$ 45,450	$ 15	$ —	$ 45,465
Corporate debt securities	Less than 1	88,156	40	(7)	88,189
Securities of government-sponsored entities	Less than 1	5,000	1	—	5,001
Total maturity less than 1 year		138,606	56	(7)	138,655
Corporate debt securities	1 to 2	4,899	46	—	4,945
Total maturity 1 to 2 years		4,899	46	—	4,945
Total available-for-sale marketable debt securities		$ 143,505	$ 102	$ (7)	$ 143,600

The following is a summary of short-term marketable debt securities classified as available-for-sale as of December 31, 2024 (*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	$ 73,410	$ 1	$ (86)	$ 73,325
Corporate debt securities	Less than 1	203,395	483	(62)	203,816
Securities of government-sponsored entities	Less than 1	34,993	33	(1)	35,025
Total available-for-sale marketable debt securities		$ 311,798	$ 517	$ (149)	$ 312,166

For the three and nine months ended September 30, 2025 and 2024, realized gains and losses on marketable debt securities were immaterial. As of September 30, 2025 and December 31, 2024, the accrued interest receivable related to the Company's marketable debt securities was $1.5 million and $2.3 million, respectively, 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 September 30, 2025 (*in thousands*):

	Less Than 12 Months		12 Months or Greater		Total	
Description of Securities	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
Corporate debt securities	$ 35,411	$ 7	$ —	$ —	$ 35,411	$ 7
Total	$ 35,411	$ 7	$ —	$ —	$ 35,411	$ 7

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, 2024 (*in thousands*):

	Less Than 12 Months		12 Months or Greater		Total	
Description of Securities	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
Commercial paper	$ 68,446	$ 86	$ —	$ —	$ 68,446	$ 86
Corporate debt securities	40,112	56	9,969	6	50,081	62
Securities of government-sponsored entities	—	—	4,975	1	4,975	1
Total	$ 108,558	$ 142	$ 14,944	$ 7	$ 123,502	$ 149

As of September 30, 2025 and December 31, 2024, the amortized cost of the available-for-sale marketable debt securities in an unrealized loss position was $35.4 million and $123.7 million, respectively.

As of September 30, 2025 and December 31, 2024, 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 nine months ended September 30, 2025 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

Stock Purchase and Collaboration Agreement with PharmaKrysto

In March 2022, the Company entered into a Collaboration Agreement with PharmaKrysto Limited ("PharmaKrysto"), a privately held pre-clinical stage company and concurrently entered into a Stock Purchase Agreement (together, the "Agreements") whereby the Company acquired 5% of the outstanding common shares of PharmaKrysto. The Agreements granted the Company an option to purchase the remaining outstanding shares of PharmaKrysto for $5.0 million upon the occurrence of a subsequent pre-clinical milestone, which expired on March 8, 2025. As the option was not exercised by the Company prior to expiration, the rights granted to the Company under the Agreements ceased, and the previously purchased common shares were transferred back to PharmaKrysto for immaterial consideration. The Company deconsolidated PharmaKrysto as of March 8, 2025, resulting in an immaterial amount recognized in the Consolidated Statements of Operations and Comprehensive Loss for the nine months ended September 30, 2025.

Licensing Agreement with Renalys

In January 2024, the Renalys License Agreement between the Company and Renalys came into effect and the Company exercised its option to purchase shares of common stock of Renalys. The Company determined that Renalys is a VIE as they could require additional funding to support development and commercial activities. The Company has variable interests in Renalys, including an equity interest, Buyout Right and performance-related payments under the Renalys License Agreement that absorb variability from the performance of Renalys.

In order to determine the primary beneficiary of Renalys, the Company evaluated its variable interest to identify if the Company had the power to direct the activities that most significantly impact the economic performance. Based upon the capital structure, governing documents and overall business operations, the Company determined that it is not the primary beneficiary as it does not have the power to direct the activities that most significantly impact the economic performance of Renalys and does not have an obligation to absorb losses.

The carrying amount of the liabilities related to the Company's variable interests was zero and $8.9 million as of September 30, 2025 and December 31, 2024, respectively, included in other current liabilities in the Company's Consolidated Balance Sheets. During the three and nine months ended September 30, 2025, the Buyout Right was relinquished and the Company recognized the entire Renalys deferred revenue balance of $9.3 million in license and collaboration revenue on the Consolidated Statements of Operations and Comprehensive Loss. The Company is not required to provide additional funding. The creditors have no recourse to the general credit or assets of the Company.

NOTE 7. LEASES

As of September 30, 2025, the Company had an 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 right-of-use assets totaling $34.6 million and lease liabilities totaling $34.5 million. The total right-of-use 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 five 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 right-of-use asset and lease liability measurements. The measurement of the lease term occurs from the February 2021 occupancy date of the office space.

In November 2024, the Company entered into a sublease for a portion of the premises. The term of the sublease runs from January 2025 through August 2028. The Company's sublease arrangement has been classified as an operating lease with sublease income recognized on a straight-line basis over the term of the sublease arrangement. To measure the Company's periodic sublease income, the Company elected to use a practical expedient under ASC 842 to aggregate non-lease components with the related lease components because (i) the timing and pattern of transfer for the non-lease components and the related lease components are the same and (ii) the lease components, if accounted for separately, would be classified as an operating lease.

The Company also had an operating lease with Esprit Investments Limited for office space located in Dublin, Ireland, which was entered into in October 2022 for a term of five years. In January 2025, the Company entered into a lease assignment for the Dublin office space, which released the Company of its obligations under the original lease. As a result, the Company derecognized the operating lease right-of-use asset and lease liability in the Consolidated Balance Sheets.

The following is a schedule of the future minimum rental commitments for the Company's operating lease reconciled to the lease liability and right-of-use asset as of September 30, 2025 (*in thousands*):

	September 30, 2025
2025 (remaining)	$ 1,654
2026	6,775
2027	6,978
2028	4,781
2029 and thereafter	—
Total undiscounted future minimum payments	20,188
Present value discount	(1,809)
Total lease liability	18,379
Unamortized lease incentives	(2,777)
Cash payments in excess of straight-line lease expense	(4,138)
Total right-of-use asset	$ 11,464

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

	September 30, 2025	December 31, 2024
Weighted-average remaining lease term in years	2.9	3.7
Weighted-average discount rate	6.47 %	6.48 %

For the three and nine months ended September 30, 2025, the Company recorded $1.1 million and $3.3 million, respectively, in expense related to operating leases, including amortized tenant improvement allowances. For the three and nine months ended September 30, 2024, the Company recorded $1.3 million and $3.7 million, respectively, 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 September 30, 2025, the fair value of the Company's 2.25% Convertible Senior Notes due 2029 was $360.5 million. As of December 31, 2024, the fair value of the Company's 2.5% Convertible Senior Notes due 2025, now repaid, was $68.2 million and the fair value of the Company's 2.25% Convertible Senior Notes due 2029 was $302.1 million. The fair values were estimated utilizing market quotations and are considered Level 2.

The following table presents the Company's assets, measured and recognized at fair value on a recurring basis, classified under the appropriate level of the fair value hierarchy as of September 30, 2025 (*in thousands*):

	As of September 30, 2025			
	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	$ 110,930	$ 110,930	$ —	$ —
Marketable debt securities, available-for-sale	143,600	—	143,600	—
Total	$ 254,530	$ 110,930	$ 143,600	$ —

The following table presents the Company's assets, measured and recognized at fair value on a recurring basis, classified under the appropriate level of the fair value hierarchy as of December 31, 2024 (*in thousands*):

	As of December 31, 2024			
	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	$ 58,535	$ 51,060	$ 7,475	$ —
Marketable debt securities, available-for-sale	312,166	—	312,166	—
Total	$ 370,701	$ 51,060	$ 319,641	$ —

NOTE 9. INTANGIBLE ASSETS

Ligand License Agreement

In 2012, the Company entered into the Ligand License Agreement for a worldwide sublicense to develop, manufacture and commercialize FILSPARI. As consideration for the license, the Company is required to make substantial payments upon the achievement of certain milestones, totaling up to $114.1 million. Through September 30, 2025 the Company has capitalized $47.2 million for contractual milestones achieved under the Ligand License Agreement. Pursuant to the Ligand License Agreement, the Company is obligated to pay to Ligand (and Bristol-Myers Squibb Company ("BMS")) an escalating royalty between 15% and 17% of net sales of FILSPARI and any other products containing FILSPARI or related compounds, with payments due quarterly. The Company began incurring costs associated with such royalties following the February 2023 approval of FILSPARI. For the three and nine months ended September 30, 2025, the Company capitalized royalties owed on net sales of FILSPARI to intangible assets of $15.6 million and $35.9 million, respectively, and $5.4 million and $12.4 million, for the three and nine months ended September 30, 2024, respectively. The cost of historical milestones paid and royalty payments are being amortized to selling, general and administration on a straight-line basis through April 30, 2033.

Mission License Agreement

In 2014, the Company entered into the Mission License Agreement, pursuant to which it 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 Mission License Agreement, as subsequently amended, which runs through May 2029, the Company is obligated to pay Mission the greater of $2.1 million, representing the guaranteed minimum royalty, or 20% of its Thiola net sales generated globally during each calendar year. The Company has capitalized $181.5 million related to the Thiola intangible asset which consists of an up-front license fee, professional fees, present value of the guaranteed minimum royalties and any additional payment obligations through September 30, 2025 in excess of minimum royalties. The Company has not capitalized any royalties in excess of the annual contractual minimum as of September 30, 2025 as royalties are capitalized in the period in which they become probable and estimable, which is typically in the period corresponding with the respective sales.

The following table sets forth amortizable intangible assets as of September 30, 2025 and December 31, 2024 (*in thousands*):

	September 30, 2025	December 31, 2024
Finite-lived intangible assets	$ 271,288	$ 224,293
Less: accumulated amortization	(162,606)	(121,072)
Net carrying value	$ 108,682	$ 103,221

Amortization expense for the three and nine months ended September 30, 2025 was $15.4 million and $41.5 million, respectively, and $10.8 million and $30.1 million, for the three and nine months ended September 30, 2024, respectively, recorded in selling, general and administrative expenses.

As of September 30, 2025 and December 31, 2024, the Company had goodwill of $0.8 million.

NOTE 10. CONVERTIBLE NOTES PAYABLE

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

	September 30, 2025	December 31, 2024
2.25% convertible senior notes due 2029	$ 316,250	$ 316,250
2.50% convertible senior notes due 2025	—	68,904
Unamortized debt issuance costs - 2.25% convertible senior notes due 2029	(4,880)	(5,940)
Unamortized debt issuance costs - 2.50% convertible senior notes due 2025	—	(226)
Total convertible senior notes, net of unamortized debt discount and debt issuance costs	$ 311,370	$ 378,988

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 September 30, 2025, 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 Indenture, 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. The Company 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%.

The 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 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 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 2029 Notes at September 30, 2025.

The 2029 Notes are classified on the Company's Consolidated Balance Sheets as long-term convertible debt at September 30, 2025 and December 31, 2024.

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 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. 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. After giving effect to the repurchase, the total remaining principal amount outstanding under the 2025 Notes was $68.9 million. On September 15, 2025, the 2025 Notes matured and the Company repaid the remaining principal amount outstanding of $68.9 million plus accrued interest.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Contractual interest expense	$ 2,138	$ 2,210	$ 6,557	$ 6,629
Amortization of debt issuance costs	420	432	1,286	1,293
Total interest expense for the 2025 and 2029 Notes	$ 2,558	$ 2,642	$ 7,843	$ 7,922

Total interest expense recognized for the three and nine months ended September 30, 2025 was $2.8 million and $8.5 million, respectively, and $2.8 million and $8.4 million, for the three and nine months ended September 30, 2024, respectively

NOTE 11. ACCRUED EXPENSES

Accrued expenses at September 30, 2025 and December 31, 2024 consisted of the following (*in thousands*):

	September 30, 2025	December 31, 2024
Sales discounts, rebates, and allowances	$ 29,503	$ 10,585
Compensation related costs	27,587	35,166
Accrued royalties	19,764	12,309
Research and development	16,863	16,090
Selling, general and administrative	8,622	6,154
Miscellaneous accrued expenses	3,423	5,724
Total accrued expenses	$ 105,762	$ 86,028

NOTE 12. NET INCOME (LOSS) PER COMMON SHARE

Basic and diluted net income (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. In accordance with *ASC 260, Earnings per Share*, if a company had a discontinued operation, the company uses income from continuing operations, adjusted for preferred dividend and similar adjustments, as its control number to determine whether potential common shares are dilutive.

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 were immediately exercisable upon issuance, and were exercised in the third quarter of 2024, resulting in the issuance of 1.25 million shares of the Company's common stock. Due to the nominal exercise price of the pre-funded warrants and the lack of any contingencies to exercise, the shares underlying the pre-funded warrants have been included in the calculation of basic net loss per common share since the date the warrants were issued.

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. For the periods that the Company has reported net losses, all potentially dilutive securities are anti-dilutive and, accordingly, basic net loss per share equals diluted net loss per share.

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

	Three Months Ended			Nine Months Ended		
	September 30, 2025					
	Shares	Net Income	EPS	Shares	Net Loss	EPS
Basic income (loss) per share	89,230,420	$ 25,706	$ 0.29	88,847,209	$ (28,275)	$ (0.32)
Interest expense associated with convertible debt, net of tax		2,558				
Dilutive income (loss) per share	102,618,560	$ 28,264	$ 0.28	88,847,209	$ (28,275)	$ (0.32)

	Three Months Ended			Nine Months Ended		
	September 30, 2024					
	Shares	Net Loss	EPS	Shares	Net Loss	EPS
Continuing operations	77,779,379	$ (54,752)	$ (0.70)	77,473,161	$ (260,362)	$ (3.36)
Discontinued operations	77,779,379	(59)	—	77,473,161	(919)	(0.01)
Basic and diluted loss per share	77,779,379	$ (54,811)	$ (0.70)	77,473,161	$ (261,281)	$ (3.37)

The calculation of diluted weighted average common shares outstanding for the three months ended September 30, 2025 was as follows:

	Three Months Ended September 30, 2025
Basic weighted average common shares outstanding	89,230,420
Dilutive common share equivalents - restricted stock units	1,120,629
Dilutive common share equivalents - convertible debt	11,389,096
Dilutive common share equivalents - options	878,415
Diluted weighted average common shares outstanding	102,618,560

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Convertible debt	—	11,697,953	11,593,869	11,697,953
Options	5,948,756	10,235,428	9,947,928	10,759,644
Restricted stock units	55,566	3,809,142	4,368,415	3,828,261
Total anti-dilutive shares	6,004,322	25,742,523	25,910,212	26,285,858

NOTE 13. COMMITMENTS AND CONTINGENCIES

Commitments

Certain of the Company's contractual arrangements with 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.

Contingencies

In November 2020, the Company completed the acquisition of Orphan Technologies Limited (“Orphan”), including Orphan’s rare metabolic disorder drug pegtibatinase. The Company acquired Orphan by purchasing all of the outstanding shares. Under the stock purchase agreement, the Company has also agreed to make contingent cash payments up to an aggregate of $427.0 million based on the achievement of certain development, regulatory and commercialization events as set forth in the Agreement, as well as additional tiered mid-single digit royalty payments based upon future net sales of any pegtibatinase products in the U.S. 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.

In accordance with ASC 450, *Contingencies*, contingent cash payments will be accrued for when it is probable that a liability has been incurred and the amount can be reasonably estimated. In March 2024, the Company recognized $65.2 million in IPR&D expense upon the achievement of a development milestone, which was paid during the second quarter of 2024 and recorded within investing activities in the Consolidated Statements of Cash Flows. As of September 30, 2025, no contingent payments have been accrued.

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 in which the outcome, 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 nine months ended September 30, 2025:

	Shares Underlying Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2024	9,283,331	$ 20.13	5.51	$ 16,408
Granted	1,434,200	20.50		
Exercised	(547,616)	16.20		3,790
Forfeited/canceled	(625,478)	29.03		
Outstanding at September 30, 2025	9,544,437	$ 19.83	5.89	$ 46,859
Vested and expected to vest at September 30, 2025	9,544,437	$ 19.83	5.89	$ 46,859
Exercisable at September 30, 2025	6,920,120	$ 20.66	4.83	$ 30,016

At September 30, 2025, unamortized stock compensation for stock options was $23.5 million, with a remaining weighted-average recognition period of 2.5 years.

Restricted Stock Units

Service Based Restricted Stock Units

The following table summarizes the Company's service based restricted stock unit activity during the nine months ended September 30, 2025:

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Unvested at December 31, 2024	3,517,263	$ 15.41
Granted	2,437,890	20.09
Vested	(1,265,220)	16.61
Forfeited/canceled	(234,788)	16.47
Unvested at September 30, 2025	4,455,145	$ 17.57

At September 30, 2025, unamortized stock compensation for service based restricted stock units was $60.5 million, with a remaining weighted-average recognition period of 2.6 years.

Performance Based Restricted Stock Units

The following table summarizes the Company's performance based restricted stock unit activity during the nine months ended September 30, 2025:

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Unvested at December 31, 2024	216,208	$ 18.34
Granted	88,000	20.46
Vested	(70,604)	27.54
Forfeited/canceled	—	—
Unvested at September 30, 2025	233,604	$ 16.35

At September 30, 2025, unamortized stock compensation for performance based restricted stock units was $0.1 million, with a remaining weighted-average recognition period of 0.6 years.

Share-Based Compensation

Total share-based compensation presented in the Consolidated Statements of Stockholders' Equity includes both continuing operations and discontinued operations. The following table sets forth share-based compensation for continuing operations for the three and nine months ended September 30, 2025 and 2024 (*in thousands*):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Research and development	$ 4,101	$ 3,321	$ 12,817	$ 10,752
Selling, general and administrative	7,162	4,700	20,587	16,946
Total share-based compensation	$ 11,263	$ 8,021	$ 33,404	$ 27,698

NOTE 15. INVENTORY

Inventory consisted of the following at September 30, 2025 and December 31, 2024 (*in thousands*):

	September 30, 2025	December 31, 2024
Raw materials	$ 25,299	$ 30,552
Work in process	1,986	7,625
Finished goods	10,262	3,679
Total inventory	$ 37,547	$ 41,856
Classified as:		
Current inventory	$ 5,548	$ 6,200
Long-term inventory	31,999	35,656
Total inventory	$ 37,547	$ 41,856

The balance classified as long-term inventory consists of raw materials, work in process and finished goods for both Thiola and FILSPARI as of September 30, 2025 and December 31, 2024. The Company maintains levels of these inventories beyond a one-year production plan to limit exposure to potential supply disruption.

NOTE 16. ACCOUNTS RECEIVABLE

Accounts receivable, net of reserves for prompt pay discounts and expected credit losses, was $83.0 million and $27.1 million at September 30, 2025 and December 31, 2024, respectively. The Accounts receivable balance as of September 30, 2025 includes the market access milestone of $40.0 million associated with the CSL Vifor License Agreement, which was received in the fourth quarter of 2025. 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

At-the-Market Equity Offering

In October 2024, the Company filed a prospectus supplement to the prospectus included in its registration statement on Form S-3 (File No. 333-281194), pursuant to which the Company may offer and sell, from time to time through Jefferies LLC, as agent ("Jefferies"), up to $100.0 million of common stock pursuant to an Amended and Restated Open Market Sale Agreement ("ATM Agreement") with Jefferies dated October 2024. As of September 30, 2025, the Company has not sold any shares under the ATM Agreement.

NOTE 18. DIVESTITURES

Discontinued Operations

Sale of Bile Acid Product Portfolio

In August 2023, the Company closed the sale of its bile acid business to Mirum Pharmaceuticals, Inc. (“Mirum”) pursuant to the terms of an asset purchase agreement dated July 16, 2023 (the "Purchase Agreement"). The Company is eligible to receive up to $235.0 million upon the achievement of certain milestones based on specified amounts of annual net sales (tiered from $125.0 million to $500.0 million) of Chenodal and Cholbam (also known as Kolbam, and together with Chenodal, the "Products"). The Company will recognize the contingent consideration receivable in earnings when the target annual sales for the milestones are met and the contingency is resolved. The Company determined that the divestiture represented a strategic shift that would have a major effect on the Company's operations and financial results, and has therefore reflected the bile acid business as a discontinued operation for all periods presented. The Company recognized loss from discontinued operations in the Consolidated Statements of Operations and Comprehensive Loss of $0.1 million and $0.9 million for the three and nine months ended September 30, 2024, respectively.

NOTE 19. SEGMENT INFORMATION

The Company operates in one business segment focused on identifying, developing and delivering life-changing therapies to people living with rare kidney and metabolic diseases. The determination of a single business segment is consistent with the consolidated financial information regularly provided to the Company’s chief operating decision maker (“CODM”), who is the President and Chief Executive Officer. The CODM uses net loss to monitor budget versus actual results in assessing segment performance and the allocation of resources. The Company’s CODM also utilizes the Company’s long-range plan as a strategic tool to allocate resources according to the Company’s strategic objectives.

The Company sells its products to specialty pharmacies and through direct-to-patient distributors worldwide, there were no significant product sales outside of the United States ("U.S.") for the three and nine months ended September 30, 2025 and 2024. License revenues outside of the U.S. were $42.4 million and $61.8 million for the three and nine months ended September 30, 2025, respectively. License revenues outside of the U.S. were not significant for the three and nine months ended September 30, 2024. Long-lived assets located outside the U.S. were immaterial as of both September 30, 2025 and December 31, 2024. The measure of segment assets is reported on the Consolidated Balance Sheets as total assets. The accounting policies of the segment are the same as those described in Note 2, Summary of Significant Accounting Policies.

The following table presents reportable segment loss, including significant expenses regularly provided to the CODM, attributable to the Company’s reportable segment for the three and nine months ended September 30, 2025 and 2024 (*in thousands*):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Revenue	$ 164,859	$ 62,898	$ 361,039	$ 158,388
Less:				
Cost of goods sold	1,585	1,626	7,786	5,191
Research and development:				
External research and development	27,837	31,587	77,653	89,443
Internal personnel costs	18,959	16,783	57,276	54,478
Other research and development	5,094	3,309	13,212	11,508
Total research and development	51,890	51,679	148,141	155,429
Selling, general and administrative	86,453	65,619	235,508	194,618
In-process research and development	—	—	—	65,205
Restructuring	—	123	—	1,035
Total other income, net	783	1,313	2,188	2,920
Income tax (provision) benefit on continuing operations	(8)	84	(67)	(192)
Loss from discontinued operations, net of tax	—	(59)	—	(919)
Net income (loss)	$ 25,706	$ (54,811)	$ (28,275)	$ (261,281)

NOTE 20. SUBSEQUENT EVENTS

In October 2025, Renalys announced that it has entered into a definitive stock purchase agreement with Chugai Pharmaceutical Co., Ltd. (“Chugai”) pursuant to which, upon the closing of the transaction, Chugai will acquire full ownership of Renalys and will gain exclusive rights to develop and commercialize sparsentan in Japan, South Korea, and Taiwan. As a minority shareholder in Renalys, Travere is entitled to receive a portion of the upfront payment at the closing of the transaction, and will be eligible to receive future payments upon the achievement of specified regulatory milestones for sparsentan and royalties on net sales in Japan, South Korea, and Taiwan.

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, 2024 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, 2024, filed with the Securities and Exchange Commission (SEC) on February 21, 2025. 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.

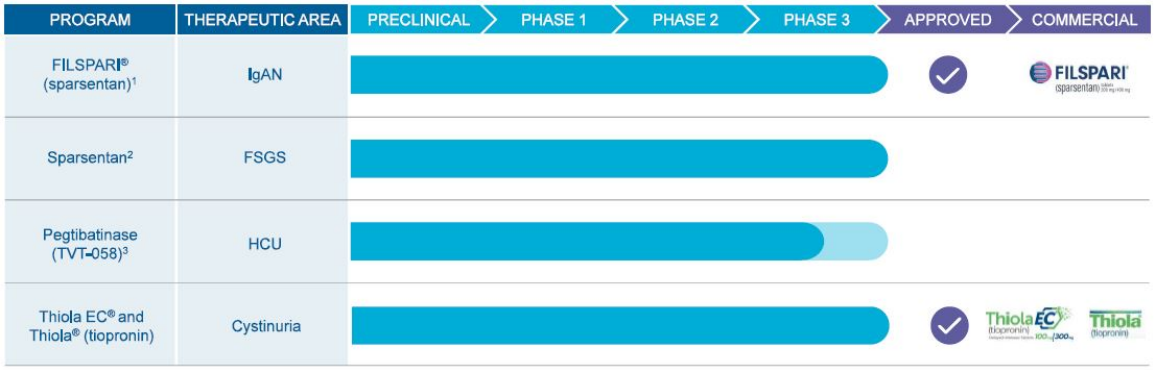
Overview

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 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 for over ten years.

Our Pipeline and Approved Products

We have a diversified pipeline designed to address areas of high unmet need in rare kidney 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.



1 On September 5, 2024, the FDA granted full approval of FILSPARI® (sparsentan) to slow kidney function decline in adults with primary IgAN who are at risk of disease progression. FILSPARI had previously been granted accelerated approval in February 2023.

2 In May 2025, we announced that the FDA accepted our sNDA for traditional approval of FILSPARI for the treatment of FSGS and assigned a PDUFA target action date of January 13, 2026.

3 In September 2024, we voluntarily paused enrollment in the Phase 3 HARMONY Study, as described below.

FILSPARI® (sparsentan)

On September 5, 2024, the FDA granted full approval of FILSPARI® (sparsentan) to slow kidney function decline in adults with primary Immunoglobulin A nephropathy (IgAN) who are at risk of disease progression. FILSPARI had previously been granted accelerated approval in February 2023 based on the surrogate marker of proteinuria. Full approval was based on positive long-term confirmatory results from the PROTECT Study demonstrating that FILSPARI significantly slowed kidney function decline over two years compared to irbesartan.

FILSPARI is the only oral, once-daily, non-immunosuppressive medication that directly targets glomerular injury in the kidney by blocking two critical pathways of IgAN disease progression (endothelin-1 and angiotensin II).

The two-year efficacy data contained in the FDA-approved label is a modified intention to treat (ITT) analysis and evaluates data from all patients regardless of treatment discontinuation. In the final analysis of the 404 randomized patients, FILSPARI significantly reduced the rate of decline in kidney function from baseline to Week 110 compared to irbesartan. In the ITT analysis included in the label, the mean eGFR slope from baseline to Week 110 was -3.0 mL/min/1.73 m^2/year for FILSPARI and -4.2 mL/min/1.73 m^2/year for irbesartan, corresponding to a statistically significant treatment effect of 1.2 mL/ min/1.73 m^2/year (p=0.0168). The positive treatment effects on proteinuria compared to the active control irbesartan that were observed at Week 36 were durable out to the two-year measurement period. Additional results from the PROTECT Study demonstrated the benefit of FILSPARI on absolute eGFR accrued over time and by Week 110 resulted in a 3.8 mL/min/1.73 m^2 difference in the mean change from baseline between FILSPARI and irbesartan.

Results from 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.

FILSPARI 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. FILSPARI has been granted seven years of Orphan Drug Exclusivity in the U.S. (running from the date of accelerated approval) for the reduction of proteinuria in adults with primary IgAN at risk of rapid disease progression, and has been granted a separate seven years of Orphan Drug Exclusivity in the U.S. (running from the date of full approval) to slow kidney function decline in adults with primary IgAN who are at risk for disease progression, excluding the use provided for in the aforementioned Orphan Drug Exclusivity granted in connection with the accelerated approval.

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 kidney failure within 15 years. FILSPARI is the first non-immunosuppressive therapy approved for IgAN and is the only oral, once-daily, non-immunosuppressive therapy approved for this condition that directly targets glomerular injury in the kidney by blocking two critical pathways of IgAN disease progression (endothelin-1 and angiotensin II). We estimate more than 70,000 patients in the United States to be addressable under FILSPARI's full approval indication.

Data to support the approval of FILSPARI was generated from the 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 that evaluated 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 angiotensin converting enzyme (ACE) inhibitor or angiotensin receptor blockers (ARB) therapy, and is currently ongoing in the open label extension phase of the study.

FILSPARI is available only through a risk evaluation and mitigation strategy (REMS) approved by the FDA for liver monitoring regarding potential risk of hepatotoxicity, as has been required for certain other approved endothelin antagonists. Initially, as part of the liver monitoring REMS, monthly monitoring of each patient was required for the first year a patient was on treatment, and quarterly thereafter. In August 2025, the FDA approved updated REMS labeling, reducing the frequency of liver monitoring to every three months from the onset of treatment and also removing the embryo-fetal toxicity monitoring requirement from the REMS.

In April 2024, we and our partner CSL Vifor announced that the European Commission had granted conditional marketing authorization (“CMA”) for FILSPARI (sparsentan) for the treatment of adults with primary IgAN with a urine protein excretion ≥1.0 g/day (or urine protein-to-creatinine ratio ≥0.75 g/g), and in April 2025, we and CSL Vifor announced that the European Commission has converted the CMA into a standard marketing authorization (“MA”) for FILSPARI for the treatment of adults with primary IgAN with a urine protein excretion ≥1.0 g/day (or urine protein-to-creatinine ratio ≥0.75 g/g). The MA is granted for all member states of the European Union, as well as in Iceland, Liechtenstein and Norway. As a result of the standard MA approval, we received a regulatory milestone payment of $17.5 million in May 2025 under the terms of the License Agreement. Additionally, in September 2025, we recognized a $40 million milestone for market access initiatives in certain countries; payment of the milestone was received in the fourth quarter of 2025. FILSPARI became commercially available in Europe under the CMA in August 2024, with an initial launch in Germany and Austria. In October 2024, we and CSL Vifor announced that Swissmedic has granted temporary marketing authorization for FILSPARI for the treatment of adults with primary IgAN with a urine protein excretion ≥1.0 g/day (or urine protein-to-creatinine ratio ≥0.75 g/g). In April 2025, the Medicines and Healthcare products Regulatory Agency (MHRA) in the UK converted its conditional approval of FILSPARI in IgAN to standard approval.

In January 2024, we announced our entry into an exclusive licensing agreement with Renalys Pharma, Inc. ("Renalys"), to bring sparsentan for the treatment of IgAN to patients in Japan and other countries in Asia. Renalys holds regional rights to sparsentan for Japan, South Korea, Taiwan, Brunei, Cambodia, Indonesia, Laos, Malaysia, Myanmar, the Philippines, Singapore, Thailand, and Vietnam. Following successful meetings with the Pharmaceuticals and Medical Devices Agency (PMDA) in 2023, in the second quarter of 2024 Renalys initiated an open label registration study of sparsentan in Japan to support potential approval of sparsentan in Japan. In July 2024, Renalys announced that the first patient was dosed in the study and in January 2025, Renalys announced achievement of full enrollment in the study. Results from the urine protein/creatinine ratio (UP/C) endpoint in the study are expected in the second half of 2025 to support a submission for approval to PMDA. In December 2024, Renalys announced that sparsentan received Orphan Drug Designation from the Japanese Ministry of Health, Labour and Welfare for the indication of primary IgA nephropathy as of November 27, 2024. In October 2025, Renalys announced that it had completed data collection for the primary endpoint in the Phase 3 clinical trial of sparsentan for IgAN and that it had reached an agreement with the PMDA regarding development plans for two new Phase 3 clinical trials of sparsentan, one investigating the use of sparsentan in focal segmental glomerulosclerosis (FSGS) and the other in Alport syndrome, in Japan. Under the terms of the licensing agreement, Renalys is responsible for development, regulatory matters, and commercialization in the licensed territories. Also in October 2025, Renalys announced that it has entered into a definitive stock purchase agreement with Chugai Pharmaceutical Co., Ltd. (“Chugai”) pursuant to which, upon the closing of the transaction, Chugai will acquire full ownership of Renalys and will gain exclusive rights to develop and commercialize sparsentan in Japan, South Korea, and Taiwan. As a minority shareholder in Renalys, Travere is entitled to receive a portion of the upfront payment at the closing of the transaction, and will be eligible to receive future payments upon the achievement of specified regulatory milestones for sparsentan and royalties on net sales in Japan, South Korea, and Taiwan.

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 the EEA.

FSGS is a leading cause of kidney failure 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). Following engagement with the FDA on the interim proteinuria analysis and a subsequent eGFR data-cut, we elected to forego the previously planned submission for accelerated approval and pursue a potential traditional approval upon completion of the DUPLEX Study.

In May 2023, we announced topline primary efficacy results from the pivotal Phase 3 DUPLEX Study of sparsentan in FSGS. The confirmatory primary endpoint of the DUPLEX Study designed to support traditional regulatory approval was the rate of change in eGFR over 108 weeks of treatment. 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 the results, including the pre-specified secondary endpoints on proteinuria and exploratory endpoints, including renal outcomes, which trended favorably for sparsentan. In addition, a review of the safety results through 108 weeks of treatment indicate sparsentan was generally well-tolerated and the overall safety profile in the study to date was generally consistent between treatment groups.

In December 2023, we announced that we had completed a planned Type C meeting with the FDA to discuss results from the Phase 3 DUPLEX Study of sparsentan in FSGS. The FDA acknowledged the high unmet need for approved therapies as well as the challenges in studying FSGS but indicated that the two-year results from the Phase 3 DUPLEX Study alone were not sufficient to support an sNDA submission. The FDA acknowledged the work being done by the larger nephrology community to better understand proteinuria and eGFR as endpoints in clinical trials of FSGS and indicated a willingness to continue to engage with us on a potential path forward for sparsentan in FSGS following our consideration of additional evidence. Subsequently, a collaborative international effort referred to as the PARASOL project was initiated with a goal to define the quantitative relationships between short-term changes in biomarkers (proteinuria and GFR) and long-term outcomes in order to support the use of alternative proteinuria-based endpoints as a basis for accelerated and traditional approval. The PARASOL project is led by several patient advocacy organizations focused on glomerular diseases, with participation from regulators and industry representatives. The principal finding from PARASOL was that in FSGS, reduction in proteinuria over 24 months is strongly associated with a reduction in the risk of kidney failure, and responder definitions based on thresholds of proteinuria are both biologically plausible and strongly supported by epidemiological data. Following the PARASOL public workshop in the fourth quarter of 2024, in which a multi-stakeholder group of rare kidney disease experts aligned around a potential proteinuria-based clinical trial endpoint for FSGS, we scheduled a Type C meeting with the FDA to discuss a potential regulatory pathway for a sparsentan FSGS indication. In February 2025, we announced that we had completed a Type C meeting with the FDA and in March 2025, we announced that we had submitted an sNDA to the FDA seeking traditional approval of FILSPARI for the treatment of FSGS. In May 2025, we announced that the FDA accepted the sNDA, assigned a Prescription Drug User Fee Act ("PDUFA") target action date of January 13, 2026, and initially indicated that it planned to hold an advisory committee meeting to discuss the application. In September 2025, following further review of the sNDA, the FDA informed us that an advisory committee meeting is no longer needed. The sNDA remains under review by the FDA with a PDUFA target action date of January 13, 2026.

The sNDA is supported by two of the largest and most rigorous head-to-head interventional studies conducted to date in FSGS, the Phase 3 DUPLEX Study and the Phase 2 DUET Study. In these studies, FILSPARI demonstrated rapid, superior and sustained reductions in proteinuria when compared with maximum labeled dose irbesartan across adult and pediatric patients. As published in the New England Journal of Medicine, DUPLEX showed statistically significant and clinically meaningful proteinuria remission at 36 weeks that was durable through 2 years. Patients who achieved partial or complete proteinuria remission in the DUPLEX Study, irrespective of the treatment arm, had a 67% to 77% lower risk of kidney failure, respectively, with the treatment effect of FILSPARI strengthened at more stringent thresholds down to complete remission. The results from these studies are in alignment with the findings of the independent PARASOL workgroup that support the importance of proteinuria in FSGS. If approved, FILSPARI could become the first and only FDA-approved medicine indicated for FSGS.

Together with CSL Vifor and Renalys, we continue to evaluate the potential for a regulatory pathway forward for sparsentan in FSGS in Europe and Asia.

Under the terms of our exclusive license to CSL Vifor, CSL Vifor is responsible for all commercialization activities in its licensed territories. We remain responsible for the clinical development of sparsentan in the applicable territories. If sparsentan receives marketing authorization in any of the territories covered by the exclusive license to Renalys, Renalys will be responsible for all development, regulatory matters, and commercialization activities in such licensed territories. We will retain all rights to sparsentan in the United States and rest of world outside of the territories licensed to CSL Vifor and Renalys, provided that CSL Vifor has a right of negotiation to expand the licensed territories into Canada and/or Mexico.

Pegtibatinase

Pegtibatinase 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. We estimate that there are approximately 7,000 to 10,000 addressable HCU patients globally. 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.

In December 2021, we announced positive topline results from 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. Pegtibatinase demonstrated dose-dependent reductions in total homocysteine (tHcy) during the 12 weeks of treatment, and in the highest dose cohort to date evaluating 1.5 mg/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.

In May 2023, we announced positive topline results from the sixth cohort of the Phase 1/2 COMPOSE Study, which was initiated to inform and refine formulation work for future development and commercial purposes and to further evaluate the dose response curve for pegtibatinase, and to further inform our pivotal development program to ultimately support potential approval of pegtibatinase for the treatment of HCU. In this cohort, five patients were randomized in a blinded fashion to receive 2.5 mg/kg of lyophilized pegtibatinase or placebo twice weekly (BIW), with four patients assigned to the treatment group. In this highest dose cohort to date, treatment with pegtibatinase resulted in rapid and sustained reductions in total homocysteine (tHcy), with a 67.1% mean relative reduction in tHcy from baseline, as well as maintenance of mean tHcy below the clinically meaningful threshold of 100 µmol, over weeks 6 to 12. In the double-blind period, pegtibatinase was generally well-tolerated, with no discontinuations due to treatment-related adverse events.

In December 2023, we initiated the pivotal Phase 3 HARMONY Study to support the potential approval of pegtibatinase for the treatment of classical HCU. The HARMONY Study is a global, randomized, multi-center, double-blind, placebo-controlled Phase 3 clinical trial designed to evaluate the efficacy and safety of pegtibatinase as a novel treatment to reduce total homocysteine (tHcy) levels. In the beginning of 2024, the first patients were dosed in the HARMONY Study.

In September 2024, we announced a voluntary pause of enrollment in the Phase 3 HARMONY Study. The voluntary enrollment pause enables us to work to address necessary process improvements in manufacturing scale-up to support commercial scale manufacturing as well as full enrollment in the HARMONY Study. Patients currently enrolled in pegtibatinase studies continue to receive study medication from small scale batches which are unaffected by the scale-up process. Currently enrolled patients will be able to continue on study medication as scheduled for the duration of the trials they are participating in. The voluntary enrollment pause was enacted following our determination that the desired drug substance profile was not achieved in the initial scale-up process. We have successfully manufactured the first commercial-scale batches and are engaging with regulators to restart enrollment in the Phase 3 HARMONY Study in 2026.

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

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 U.S. 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 U.S. that would be candidates for Thiola or Thiola EC.

In June 2019 we announced that the FDA approved 100mg and 300mg 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 100mg version of the original formulation of Thiola (tiopronin tablets) became available and in June 2022, a second option for the 100mg version of the original formulation of Thiola (tiopronin tablets) was approved. These generic versions of the original formulation of Thiola have impacted our sales, and these or additional generic versions of either formulation could have a material adverse impact on sales. To date, several generic options for the 100mg and 300mg versions of Thiola EC have been approved by the FDA and become available. Accordingly, Thiola EC is subject to generic competition.

Results of Operations

Results of operations for the three and nine months ended September 30, 2025 compared to the three and nine months ended September 30, 2024

Revenue

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
FILSPARI	$ 90,900	$ 35,619	$ 55,281	$ 218,668	$ 82,578	$ 136,090
Tiopronin products	22,250	25,382	(3,132)	65,184	70,583	(5,399)
Total net product sales	113,150	61,001	52,149	283,852	153,161	130,691
License and collaboration revenue	51,709	1,897	49,812	77,187	5,227	71,960
Total revenue	$ 164,859	$ 62,898	$ 101,961	$ 361,039	$ 158,388	$ 202,651

Net product sales

The increase in total net product sales for the three and nine months ended September 30, 2025 compared to the three and nine months ended September 30, 2024 was primarily due to growth in sales of FILSPARI.

License and collaboration revenue

The increase in license and collaboration revenue for the three months ended September 30, 2025 compared to the three months ended September 30, 2024 was primarily due to a market access milestone of $40.0 million associated with the CSL Vifor License agreement and license revenue of $9.3 million recognized upon the relinquishment of the Buyout Right in the Renalys License Agreement. The increase in license and collaboration revenue for the nine months ended September 30, 2025 compared to the nine months ended September 30, 2024 was primarily due to market access and regulatory milestones totaling $57.5 million associated with the CSL Vifor License agreement, license revenue of $9.3 million associated with the relinquishment of the Buyout Right in the Renalys License Agreement and the sale of $3.8 million active pharmaceutical ingredients to CSL Vifor in March of 2025.

Operating Expenses

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
Cost of goods sold - product sales	$ 1,308	$ 1,626	$ (318)	$ 4,083	$ 5,059	$ (976)
Cost of goods sold - license and collaboration	277	—	277	3,703	132	3,571
Total cost of goods sold	1,585	1,626	(41)	7,786	5,191	2,595
Research and development	51,890	51,679	211	148,141	155,429	(7,288)
Selling, general and administrative	86,453	65,619	20,834	235,508	194,618	40,890
In-process research and development	—	—	—	—	65,205	(65,205)
Restructuring	—	123	(123)	—	1,035	(1,035)
Total operating expenses	$ 139,928	$ 119,047	$ 20,881	$ 391,435	$ 421,478	$ (30,043)

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 our license and collaboration agreements, which currently consists of the sale of active pharmaceutical ingredients to our collaboration partners, at cost or at cost plus a margin.

Prior to the February 2023 FDA accelerated approval of FILSPARI (sparsentan), we expensed the production of active pharmaceutical ingredients purchased to support the commercial launch of FILSPARI, in research and development expenses. For the three and nine months ended September 30, 2025 and 2024, sales of FILSPARI primarily consisted of zero-cost inventories, and therefore cost of goods sold did not increase proportionally to the increase in product sales. As of September 30, 2025, we had $0.9 million of zero-cost inventory remaining, the majority of which we expect will be consumed in 2025. We began capitalizing inventory costs associated with FILSPARI following the February 2023 accelerated approval.

For the nine months ended September 30, 2025 compared to the nine months ended September 30, 2024, our cost of goods sold - license and collaboration increased by $3.6 million, primarily due to the sale of active pharmaceutical ingredients to CSL Vifor in March 2025.

Research and development expenses

Research and development costs include expenses related to sparsentan, pegtibatinase 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 four Phase 3 clinical trials in process that are in various 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 and year to year.

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 September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
External service provider costs:						
Sparsentan	$ 11,608	$ 14,525	$ (2,917)	$ 36,664	$ 44,204	$ (7,540)
Pegtibatinase	16,158	16,789	(631)	40,477	44,331	(3,854)
General and other product candidates	5,165	3,582	1,583	13,724	12,416	1,308
Total external service provider costs	32,931	34,896	(1,965)	90,865	100,951	(10,086)
Internal personnel costs	18,959	16,783	2,176	57,276	54,478	2,798
Total research and development	$ 51,890	$ 51,679	$ 211	$ 148,141	$ 155,429	$ (7,288)

For the three and nine months ended September 30, 2025 compared to the three and nine months ended September 30, 2024, our research and development expenses increased by $0.2 million and decreased by $7.3 million, respectively. External service provider costs decreased by $2.0 million and $10.1 million for the three and nine months ended September 30, 2025 compared to the three and nine months ended September 30, 2024, respectively, largely driven by a decrease in costs associated with the development of sparsentan as our Phase 3 programs advance towards completion and a decrease in costs associated with the development of pegtibatinase due to the pause of the HARMONY Study in September 2024. The increase in internal personnel costs of $2.2 million and $2.8 million for the three and nine months ended September 30, 2025 compared to the three and nine months ended September 30, 2024, respectively, is due to an increase in headcount.

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 and nine months ended September 30, 2025 compared to the three and nine months ended September 30, 2024, our selling, general and administrative expenses increased by $20.8 million and $40.9 million, respectively, primarily as a result of an increase in intangible asset amortization from capitalized FILSPARI royalties, an increase in commercial investment to support FILSPARI following full approval by the FDA in September 2024 and commercial spend in preparation for the potential launch of FSGS, if approved.

IPR&D expense

We did not recognize any in-process research and development (IPR&D) expense during the three and nine months ended September 30, 2025. In March 2024, we recognized $65.2 million in IPR&D expense upon the achievement of a development milestone associated with our treatment candidate pegtibatinase, which we acquired as part of the November 2020 acquisition of Orphan Technologies Limited.

Other Income/Expenses

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
Interest income	$ 3,047	$ 3,570	$ (523)	$ 10,129	$ 14,022	$ (3,893)
Interest expense	(2,751)	(2,777)	26	(8,452)	(8,365)	(87)
Other income (expense), net	487	520	(33)	511	(2,737)	3,248
Total other income, net	$ 783	$ 1,313	$ (530)	$ 2,188	$ 2,920	$ (732)

The change in our total other income, net for the three and nine months ended September 30, 2025 as compared to the three and nine months ended September 30, 2024 is partially attributable to changes in interest income, driven by a decrease in the overall balance of interest-bearing security investments held along with fluctuations in short-term interest rates on those investments. For the nine months ended September 30, 2024, we recognized $3.4 million in other expense in connection with our equity investment in Renalys, for the difference in basis between the carrying value and fair value of our proportionate share of the investee's net assets.

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, along with proceeds from license and collaboration agreements and the divestiture of our bile acid business. We experienced significant growth in recent years in the number of our employees and the scope of our operations. We also 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 commercial launch of FILSPARI in the United States for IgAN and for the potential commercial launch of FILSPARI in the United States for FSGS, if approved.

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 from the date of this filing. 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 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 manufacturing and selling these products.

We had the following balances and financial performance at September 30, 2025 and December 31, 2024 (*in thousands*):

	September 30, 2025	December 31, 2024
Cash and cash equivalents	$ 110,930	$ 58,535
Marketable debt securities, at fair value	$ 143,600	$ 312,166
Convertible debt	$ 311,370	$ 378,988
Accumulated deficit	$ (1,475,442)	$ (1,447,167)
Stockholders' equity	$ 73,564	$ 59,077
Net working capital*	$ 236,036	$ 215,951
Net working capital ratio**	2.75	2.08

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

As of September 30, 2025, we had cash and cash equivalents of $110.9 million and available-for-sale marketable debt securities of $143.6 million. Substantial sources of funds over the past year, as summarized further below, include net proceeds of $134.7 million from an underwritten public offering of our common stock in November 2024.

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, anticipated milestone payments, 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 ongoing launch of FILSPARI and expenses associated with the preparations for a potential commercial launch of FILSPARI in FSGS. 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, FILSPARI royalties and certain earned and potential milestone payments. We received payment of the FILSPARI market access initiative milestone of $40.0 million in the fourth quarter of 2025 and we anticipate achieving additional FILSPARI milestones with the potential for future payments depending on timing and outcomes of events over the next 12 months.

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, 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 2029 Notes, which mature on September 1, 2029. 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, 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 through cash purchases and/or exchanges for equity securities, in open market purchases, privately negotiated transactions or otherwise, and the amounts involved in such purchases and/or exchanges, individually or in the aggregate, may be material. We may not be able to successfully conduct financing or refinancing activity on favorable terms or at all.

Purchase Agreement Proceeds

Sale of Bile Acid Product Portfolio

In July 2023, we entered into the Purchase Agreement with Mirum, pursuant to which Mirum agreed to purchase substantially all of the assets primarily related to our business of development, manufacture and commercialization of the Products, which comprised our bile acid business. Upon the Closing of the transaction on August 31, 2023, we received an upfront cash payment of $210.0 million. Pursuant to the Purchase Agreement, we are eligible to receive up to $235.0 million upon the achievement of certain milestones based on specified amounts of annual net sales (tiered from $125.0 million to $500.0 million) of the Products.

Collaboration and License Proceeds

License and Collaboration Agreement with CSL Vifor

In September 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 FILSPARI in the licensed territories. Under the terms of the license agreement, we 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. Through September 30, 2025, we have received upfront and milestone payments totaling $72.5 million and have earned an additional

$40.0 million, which was received in the fourth quarter of 2025, associated with the license agreement. 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.

Licensing Agreement with Renalys

In January 2024, our license agreement with Renalys Pharma, Inc. came into effect. Under the terms of the agreement, we granted an exclusive license to Renalys for the development and commercialization of sparsentan in Japan and other specified countries in Asia. Pursuant to the terms of the agreement, we are eligible to receive up to $120.0 million in aggregate regulatory, development and sales-based milestone payments. We are also entitled to receive tiered double-digit to mid-20 percent royalties of annual net sales of sparsentan in the licensed territories. In addition, we received an option to purchase shares of common stock of Renalys, which we exercised in January 2024.

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

Equity Offerings

2024 Underwritten Public Offering of Common Stock

In November 2024, we sold an aggregate of approximately 9.0 million shares of our common stock in an underwritten public offering, at a price to the public of $16.00 per share of common stock. The net proceeds from the offering, after deducting the underwriting discounts and offering expenses, were approximately $134.7 million.

At-the-Market Equity Offering

In October 2024, we filed a prospectus supplement to the prospectus included in our registration statement on Form S-3 (File No. 333-281194), pursuant to which we may offer and sell, from time to time through Jefferies LLC, as agent (“Jefferies”), up to $100.0 million of our common stock pursuant to an Amended and Restated Open Market Sale Agreement ("ATM Agreement") with Jefferies dated October 2024. The Company has not sold any shares under the ATM Agreement.

Operating Leases

Future Minimum Rental Commitments

As of September 30, 2025, we have future minimum rental commitments totaling $20.2 million arising from our operating lease and sublease income totaling $3.8 million. 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 our 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 our financial statements.

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 FILSPARI (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 September 30, 2025, we have paid $47.2 million for contractual milestones achieved under the Ligand License Agreement. Pursuant to the terms of the Ligand License Agreement, we are obligated to pay to Ligand an escalating royalty between 15% and 17% of net sales of FILSPARI and any other products containing FILSPARI or related compounds, with payments due quarterly. We began incurring costs associated with such royalties following the February 2023 approval of FILSPARI.

The Ligand 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 Ligand 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 FILSPARI as described above or (iv) certain other conditions. We may terminate the Ligand 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.

Mission 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 ("Mission License Agreement"). Under the terms of the Mission License Agreement, as subsequently amended, which runs through May 2029, we are obligated to pay to Mission the greater of $2.1 million, representing the guaranteed minimum royalty, or 20% of our Thiola net sales generated globally during each calendar year.

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

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. We acquired Orphan by purchasing all of its 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 U.S. 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. We made a $65.0 million payment in the second quarter of 2024 following the achievement of a development milestone.

See Note 13 to our unaudited Consolidated Financial Statements for further discussion.

French Rebate Accrual

In October 2021, our distributor 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. In October 2024, we received an invoice from the government authority in the amount of approximately $6.2 million for reimbursement of amounts previously paid for Kolbam, which we paid in November 2024. We have appealed the pricing decision and will pursue an appeal of the amount owed with the Competent Administrative Court.

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.

See Note 10 to our unaudited Consolidated Financial Statements for further discussion.

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 from the date of this filing. We expect to use cash flows from operations and, when necessary, 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 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;
- payment obligations related to the 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;
- the potential impacts of actions taken by the current administration, including but not limited to tariffs and changes at the FDA and other government agencies; 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 from Continuing Operations

Cash Flows from Operating Activities

Cash used in operating activities from continuing operations for the nine months ended September 30, 2025 was $22.9 million compared to $201.4 million for the nine months ended September 30, 2024. The change was due to a $130.7 million increase in total net product sales, a regulatory milestone of $17.5 million and the timing of payments from normal operations.

Cash Flows from Investing Activities

Cash provided by investing activities for the nine months ended September 30, 2025 was $132.7 million compared to $179.6 million for the nine months ended September 30, 2024. The fluctuation in net cash used in investing activities resulted primarily from the timing differences in investment purchases, sales and maturities, and the fluctuation of our portfolio mix between cash equivalents and short-term investment holdings, an increase in intangible asset purchases, and a $65.0 million payment in the second quarter of 2024 following the achievement of a development milestone.

Cash Flows from Financing Activities

Cash used in financing activities from continuing operations for the nine months ended September 30, 2025 was $60.1 million compared to cash provided by of $0.3 million for the nine months ended September 30, 2024. The change was primarily due to repayment of the 2025 Notes, offset by proceeds from the exercise of stock options during the nine months ended September 30, 2025.

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, 2024 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 September 30, 2025, we had cash equivalents and marketable debt securities of approximately $254.5 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 continue to 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 as of September 30, 2025 would have had approximately a $0.3 million impact on the fair value of our investments at September 30, 2025.

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 Securities Exchange Act of 1934, as amended ("Exchange Act"), 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 Rule 13a-15(b) of the Exchange Act, 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 defined in Exchange Act Rule 13a–15(e) and 15d-15(e)), as of the end of the quarter covered by this report. Based on the foregoing, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

An evaluation was also performed under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, of any change to our internal control over financial reporting that occurred during the quarter covered by this report and that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting. Our evaluation did not identify any change 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 September 30, 2025 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II - OTHER INFORMATION

Item 1. Legal Proceedings

The information required by this Item is incorporated herein by reference to the Notes to the Unaudited 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, 2024 as filed with the Securities and Exchange Commission (SEC) on February 21, 2025.

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 slow kidney function decline in adults with primary IgAN who are at risk of disease progression, which was granted full approval by the FDA in September 2024. FILSPARI had previously been granted accelerated approval in February 2023 based on the surrogate marker of proteinuria. As a 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 had 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, the updated, full approval label, clinical practice guidelines and any future changes thereto, developments related to competitive products, and any future publications in an evolving treatment landscape. 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 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 in educating physicians and pharmacists on the benefits of 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.

We have granted exclusive licenses to third parties for the commercialization of sparsentan in certain territories outside of the United States, including Europe, Australia, New Zealand, Japan, South Korea, Taiwan, the ASEAN member states, Brazil, Chile, Israel and the Gulf Cooperation Council countries. If these third parties do not effectively engage or maintain their sales force for sparsentan if approved in the applicable territories, our ability to recognize milestone payments and royalties from the sales in such territories will be adversely affected.

We will need to continue to expend significant time and resources to train our sales forces to be credible 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 third parties for the successful commercialization of sparsentan in certain key territories outside of the United States, if approved, and such third parties' 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.*

We have granted exclusive licenses to third parties for the commercialization of sparsentan in certain territories outside of the United States, including Europe, Australia, New Zealand, Japan, South Korea, Taiwan, the ASEAN member states, Brazil, Chile, Israel and the Gulf Cooperation Council countries. Consequently, the commercial success of sparsentan in these territories will depend in significant part on the efforts of such third parties, 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. 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 territories licensed to CSL Vifor, 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 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 and Thiola, 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. Our product Thiola, and products from which we may receive milestone payments such as Cholbam, are subject to immediate competition from compounded and generic entrants, as the ANDA and/or NDA for these drug products have no remaining or current patent or non-patent exclusivity. In April 2021, a generic option for the 100mg 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 and 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 (100mg version of the original formulation). Additional generic versions of Thiola may be approved in the future. Several generic options for the 100mg and 300mg versions of Thiola EC have been approved by the FDA and become available. Our future net product revenues from Thiola and/or Thiola EC may 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, proposed "skinny label" legislation, 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.

In 2020, we 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 samples to certain generic manufacturers.

If additional generic versions of Thiola or Thiola EC, any generic versions of FILSPARI following the expiration of patent or regulatory exclusivity for the product, or generic versions of any 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 revenue and profitability. If generic versions of products from which we may receive milestone payments, such as Cholbam, are approved, our potential to receive milestone payments may be negatively impacted. 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.

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, 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.

The current administration has indicated that it plans to pursue additional policies aimed at lowering prescription drug costs. For example, in May 2025, the administration published an executive order regarding most favored nation (“MFN”) drug pricing, which is sometimes referred to as international reference pricing. This executive order directed the Secretary of Health and Human Services to communicate MFN price targets to pharmaceutical manufacturers, and

if significant progress towards MFN pricing is not delivered, to propose a rulemaking plan to impose MFN pricing. The scope, timing, and potential impact of current and future policy initiatives remain uncertain, and accordingly, we cannot predict how such legal and regulatory changes may affect our business, operations, or financial condition. If MFN pricing or other legal or regulatory changes are implemented in a way that is broadly applicable to our products, there could be a material negative impact.

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 are 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 currently sole source suppliers for manufacturing of FILSPARI and Thiola. The facilities used by our third-party manufacturers must be approved by the FDA and comparable foreign regulatory authorities. 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.

Based on the complex relationships between the United States and certain foreign countries, there is inherent risk that political, diplomatic and national security influences might lead to trade disputes and impacts and/or disruptions to our third-party manufacturers and product supply. There is currently significant uncertainty about the future relationship between the United States and Mexico, Canada, China and certain other countries, including potential changes with respect to trade policies, treaties, tariffs, taxes, and other limitations on cross-border operations. We currently source products and various materials that are necessary for the manufacturing of our products from countries that are subject to tariffs, and any changes in tariffs, trade barriers, and other regulatory requirements could lead to higher cost of goods, which would have an adverse effect on our business, financial condition and operating results, the extent of which cannot be predicted with certainty at this time.

We currently have no in-house distribution channels for FILSPARI or Thiola 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 and/or Thiola could become disrupted, resulting in lost revenues, provider dissatisfaction, and/or patient dissatisfaction.

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

Outside the United States, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies. In some countries, particularly EU Member States and EFTA countries, 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. The EU provides options for EU Member States to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. An EU Member State may approve a specific price for the medicinal product, it may refuse to reimburse a product at the price set by the manufacturer or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. Many EU Member States also periodically review their reimbursement procedures for medicinal products, which could have an adverse impact on reimbursement status.

Moreover, 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. This Health Technology Assessment ("HTA") of medicinal products is becoming an increasingly common part of the pricing and reimbursement procedures in some EU Member States, including those representing the larger markets. The HTA process is the procedure to assess therapeutic, economic and societal impact of a given medicinal product in the national healthcare systems of the individual country. The outcome of an HTA will often influence the pricing and reimbursement status granted to these medicinal products by the competent authorities of individual EU Member States. The extent to which pricing and reimbursement decisions are influenced by the HTA of the specific medicinal product currently varies between EU Member States.

In December 2021, Regulation No 2021/2282 on HTA amending Directive 2011/24/EU, was adopted in the EU. This Regulation entered into force in January 2022 and began to apply on January 12, 2025, through a phased implementation. The Regulation is intended to boost cooperation among EU Member States in assessing health technologies, including new medicinal products, and providing the basis for cooperation at EU level for joint clinical assessments in these areas. The Regulation permits EU Member States to use common HTA tools, methodologies, and procedures across the EU, working together in four main areas, including joint clinical assessment of the innovative health technologies with the most potential impact for patients, joint scientific consultations whereby developers can seek advice from HTA authorities, identification of emerging health technologies to identify promising technologies early, and continuing voluntary cooperation in other areas. Individual EU Member States will continue to be responsible for assessing non-clinical (e.g.,

economic, social, ethical) aspects of health technologies, and making decisions on pricing and reimbursement. If we or our partners are unable to maintain favorable pricing and reimbursement status in EU Member States for drug candidates that we or our partners may successfully develop and for which we or our partners may obtain regulatory approval, any anticipated revenue from and growth prospects for those products in the EU could be negatively affected. In light of the fact that the United Kingdom has left the EU, Regulation No 2021/2282 on HTA does not apply in the United Kingdom. However, the UK Medicines and Healthcare products Regulation Agency ("MHRA") is working with UK HTA bodies and other national organizations, such as the Scottish Medicines Consortium ("SMC"), the National Institute for Health and Care Excellence ("NICE"), and the All-Wales Medicines Strategy Group, to introduce new pathways supporting innovative approaches to the safe, timely and efficient development of medicinal products, including, effective as of March 31, 2025, relaunching the Innovative Licensing and Access Pathway with more predicable timelines and closer involvement of the National Health Service.

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 (which has since been divested) 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. Such notice was confirmed by a decision in October 2023, asserting percentages of our turnover owed for repayment. In April 2024, we filed an appeal with the Competent Administrative Court regarding this matter. In October 2024, we received an invoice from the government authority for approximately $6.2 million, which we paid while we continue to pursue an appeal of the decision and the amount paid. While we cannot predict the amount that we may ultimately need to repay following ongoing review and future potential appeal proceedings, 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 the EU, 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 in the United States (running from the date of accelerated approval) to reduce proteinuria in adults with primary IgAN at risk of rapid disease progression, and has been granted a separate seven years of Orphan Drug Exclusivity in the U.S. (running from the date of full approval) to slow kidney function decline in adults with primary IgAN who are at risk for disease progression, excluding the use provided for in the aforementioned Orphan Drug Exclusivity granted in connection with the accelerated approval. 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 the EU 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 the EU 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 the EU and the orphan drug designation may not result in orphan drug exclusivity in the United States for FSGS or the EU 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.

Guidelines and recommendations published by various organizations may impact the use of our products.

Government agencies promulgate regulations and guidelines directly applicable to us and to our products. However, professional societies, industry groups, practice management groups, insurance carriers, physicians, private foundations and other organizations involved in various diseases or conditions from time to time may also publish guidelines or recommendations to healthcare providers, administrators and payers, and patient communities. Recommendations by government agencies or those other groups/organizations may relate to such matters as clinical guidelines, usage and reimbursement of our products by government and private payers. Recommendations or guidelines that are followed by patients, healthcare providers and payers could impact the use of our products in positive or negative ways. In addition, recommendations or guidelines may not be followed by patients, healthcare providers or payers, and thus any such positive recommendations or guidelines may not have a positive impact on the use of our products. Any such recommendations or guidelines may be updated over time as the treatment landscape evolves, and future changes to guidelines or recommendations could have a material adverse impact on the use of our products. Any recommendations or guidelines, or changes thereto, that result in decreased use or reimbursement of our products could materially and adversely affect our product sales, business and operating results.

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, institutional review boards or ethics committees 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 or ethics committees 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, vary or terminate one or more of our clinical trials if we, regulators or institutional review boards or ethics committees determine that the participants are being exposed to unacceptable health risks;
- regulators, institutional review boards or ethics committees may require that we hold, suspend, vary 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 therapy, 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 have continued to engage with the FDA to explore a potential path forward for an sNDA, including through a recent Type C meeting, and while the FDA has accepted our sNDA seeking traditional approval of FILSPARI for FSGS and has indicated that an advisory committee meeting is not needed, there is no guarantee that the FDA will grant approval of FILSPARI for FSGS. In addition, a collaborative international effort referred to as the PARASOL project was initiated in late 2023 with a goal to define the quantitative relationships between short-term changes in biomarkers (proteinuria and GFR) and long-term outcomes in order to support the use of alternative proteinuria-based endpoints as a basis for accelerated and traditional approval. Even though representatives of regulatory agencies participated in the discussions, there is no guarantee that the outcome of those discussions will be reflected in any future formal determination by such regulatory agencies. There is no guarantee that the PARASOL group will achieve its intended goal, or that, even if it does, that sparsentan will be approved for FSGS.

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 authorities. 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.

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, 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 being tested in a mouse model of homocystinuria and the positive topline results we reported in December 2021 and May 2023 from the ongoing Phase 1/2 clinical trial of pegtibatinase may not be replicated in future studies. We cannot assure that any current or future clinical trials of sparsentan or pegtibatinase 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 under the Subpart H pathway for potential accelerated approval in the United States, and potential conditional marketing authorization in the EU, 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 May 2023, we announced that the DUPLEX Study did not achieve its two-year primary endpoint with statistical significance over the active control irbesartan. While we have continued to engage with the FDA to explore a potential path forward for an sNDA, including through a recent Type C meeting, and the FDA has accepted our sNDA seeking traditional approval of FILSPARI for FSGS and has indicated that an advisory committee is not needed, there is no guarantee that the FDA will grant approval of FILSPARI for FSGS. Similarly, there is no guarantee that our collaborator CSL Vifor will be able to establish a pathway to a potential submission of sparsentan for FSGS in Europe based on the results from the DUPLEX Study, that the EMA will support an application for sparsentan in FSGS, or that sparsentan will be approved for FSGS in Europe.

In December 2023, we initiated the pivotal Phase 3 HARMONY Study to support the potential approval of pegtibatinase for the treatment of classical HCU. The HARMONY Study is a global, randomized, multi-center, double-blind, placebo-controlled Phase 3 clinical trial designed to evaluate the efficacy and safety of pegtibatinase as a novel treatment to reduce total homocysteine (tHcy) levels. In September 2024, we announced a voluntary pause of enrollment in the HARMONY Study. The voluntary enrollment pause was enacted following our determination that the desired drug substance profile was not achieved in the initial scale-up process. While we have made process improvements and have manufactured material at the larger scale, and while we are engaging with regulatory authorities with a goal to restart enrollment in the Phase 3 HARMONY Study in 2026, there is no guarantee that regulators will agree with our assessment or that our pivotal Phase 3 HARMONY Study will resume on the anticipated timeline or be successful. Although the FDA has granted Fast Track and Breakthrough Therapy designations to pegtibatinase for the treatment of HCU, there is no guarantee that our pivotal Phase 3 HARMONY Study will resume on the anticipated timeline or be successful, or that pegtibatinase will be approved for HCU in the future.

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 authorities, 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 therapy, therapeutic 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.

In September 2024, the FDA granted full approval of FILSPARI to slow kidney function decline in adults with primary IgAN who are at risk of disease progression. 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 or comparable foreign regulatory authorities to approve pending applications or supplements to approved applications filed by us or our strategic partners, or suspension, variation 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. Further, we face risks relating to those post-marketing obligations, as well as the commercial acceptance of FILSPARI. If the regulatory approval for FILSPARI and/or Thiola are withdrawn for any reason, it would have a material adverse impact on our sales and profitability. Furthermore, if the regulatory approval for Chenodal and/or Cholbam are withdrawn for any reason, it would reduce the chance that we will receive any or all of the milestone payments from the sale of our bile acid product portfolio in August 2023.

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. 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;
- the 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 initially 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 certain 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. Initially, as part of the liver monitoring REMS, monthly monitoring of each patient was required for the first year a patient was on treatment, and quarterly thereafter. In August 2025, we announced that the FDA approved updated REMS labeling for FILSPARI, reducing the frequency of liver function monitoring to every three months from the onset of treatment with FILSPARI, and removing the embryo-fetal toxicity monitoring requirement from the REMS. 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 such efforts will be successful.

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.

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, suspend or vary 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 the original formulation of Thiola. 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"), certain generic manufacturers have been able to obtain "skinny-label" approvals of generic versions of tiopronin EC as described below. More generally, we may not be able to obtain additional issued patents relating to our technology or products. Even if issued, patents issued to us or our licensors may be challenged, narrowed, invalidated, held to be unenforceable or circumvented, which could limit our ability to stop competitors from marketing similar products or reduce the term of patent protection we may have for our products. In addition, in certain circumstances with respect to method of use patents, an ANDA applicant may certify 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. To date, several generic options for the 100mg and 300mg versions of Thiola EC have been approved by the FDA and become available. Accordingly, Thiola EC is subject to generic competition. 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. While we have additional pending U.S. and foreign patent applications directed to sparsentan and its uses, there is no guarantee that any pending or future patent applications will result in issued patents, issue on a timeline that provides material protection, or, if issued, contain claims of commercially meaningful scope.

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 Act ("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, companies 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. While we have filed an application for patent term extension of U.S. Patent No. 9,993,461, and subsequently filed a supplement to this application to reflect the FDA -determined regulatory review period for sparsentan, which, if granted could extend the term of U.S. Patent No. 9,993,461 to January 2033, there is no guarantee that such patent term extension will be granted to such date, or at all. In addition, while we intend to seek pediatric exclusivity for FILSPARI based on our ongoing development efforts, which, if granted, could extend the term of such patent by an additional six months, the granting of pediatric exclusivity requires a series of regulatory interactions to reach agreement with the FDA, and there is no guarantee that our pediatric development efforts will support a path to pediatric exclusivity or that pediatric exclusivity will be granted by the FDA on a timeline that confers benefit on the term of patent coverage or regulatory exclusivity for FILSPARI, or at all.

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 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 our licensees (including CSL Vifor and Renalys Pharma) could lose our rights to sparsentan. We have obtained a U.S. patent and European patent each covering the use of sparsentan for treating glomerulosclerosis, including FSGS, as well as a second U.S. patent and a second European patent each covering both the use of sparsentan for treating IgAN and the use of sparsentan for treating glomerulosclerosis, including FSGS. In 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 the EU 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 European Commission have granted orphan designation for sparsentan for the treatment of IgAN and FSGS, and pegtibatinase for the treatment of homocystinuria. 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 the EU, 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 and benefit from the potential regulatory exclusivity periods related to marketing authorizations granted to orphan products. The period of market exclusivity in the EU may, however, be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria on the basis of which it received orphan medicinal product destination, including where it can be demonstrated on the basis of available evidence that the original orphan medicinal product is sufficiently profitable not to justify maintenance of market exclusivity or where the prevalence of the condition has increased above the threshold. Additionally, a marketing authorization may be granted to a similar medicinal product with the same orphan indication during the 10 year period if: (i) the applicant consents to a second original orphan medicinal product application, (ii) the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities; or (iii) the second applicant can establish that its product, although similar, is safer, more effective or otherwise clinically superior to the original orphan medicinal product. A company may voluntarily remove a product from the register of orphan products.

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

In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the "PPACA"), was signed into law, which intended to, among other things, 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 and amendments to certain aspects of the PPACA. For example, on August 16, 2022, the Inflation Reduction Act of 2022 ("IRA") was signed 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 potential future healthcare reform measures of the current 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, on July 4, 2025, the annual reconciliation bill, the "One Big Beautiful Bill Act" ("OBBBA"), was signed into law which is expected to reduce Medicaid spending and enrollment by implementing work requirements for some beneficiaries, capping state-directed payments, reducing federal funding, and limiting provider taxes used to fund the program. OBBBA also narrows access to PPACA marketplace exchange enrollment and declines to extend the PPACA enhanced advanced premium tax credits, set to expire at the end of 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. Additionally, 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.

The current administration has indicated that it plans to pursue additional policies aimed at lowering prescription drug costs. For example, in May 2025, the administration published an executive order regarding MFN drug pricing, which is sometimes referred to as international reference pricing. This executive order directed the Secretary of Health and Human Services to communicate MFN price targets to pharmaceutical manufacturers, and if significant progress towards MFN pricing is not delivered, to propose a rulemaking plan to impose MFN pricing. The scope, timing, and potential impact of current and future policy initiatives remain uncertain, and accordingly, we cannot predict how such legal and regulatory changes may affect our business, operations, or financial condition. If MFN pricing or other legal or regulatory changes are implemented in a way that is broadly applicable to our products, there could be a material negative impact.

In addition, in the EU, in April 2023, the European Commission adopted a proposal for a new Directive and Regulation to revise the existing pharmaceutical legislation. In April 2024, the Parliament adopted its related position and on 4 June 2025 the European Council agreed on its position. The Council, the Parliament and the European Commission have begun trilogue negotiations with a view to reaching an agreement on the package. A decrease in data and market exclusivity opportunities for our product candidates in the EU could make them open to generic or biosimilar competition earlier than is currently the case with a related reduction in reimbursement status.

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. For example, the IRA, among other things (i) directs HHS to negotiate the price of certain high-expenditure, single-source drugs that have

been on the market for at least 7 years covered under Medicare (the "Medicare Drug Price Negotiation Program"), and (ii) imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation. These provisions began to take effect progressively starting in fiscal year 2023. On August 15, 2024, HHS announced the agreed-upon price of the first ten drugs that were subject to price negotiations, although the Medicare Drug Price Negotiation Program is currently subject to legal challenges. On January 17, 2025, HHS selected fifteen additional drugs covered under Part D for price negotiation in 2025. Each year thereafter more Part B and Part D products will become subject to the Medicare Drug Price Negotiation Program. HHS has and will continue to issue and update guidance as these programs are implemented. On December 8, 2023, the National Institute of Standards and Technology published for comment a Draft Interagency Guidance Framework for Considering the Exercise of March-In Rights which for the first time includes the price of a product as one factor an agency can use when deciding to exercise march-in rights. While march-in rights have not previously been exercised, it is uncertain if that will continue under the new framework. 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. For example, on January 5, 2024, the FDA approved Florida's Section 804 Importation Program (SIP) proposal to import certain drugs from Canada for specific state healthcare programs. It is unclear how this program will be implemented, including which drugs will be chosen, and whether it will be subject to legal challenges in the United States or Canada. Other states have also submitted SIP proposals that are pending review by the FDA. Any such approved importation plans, when implemented, may result in lower drug prices for products covered by those programs.

In addition, the current administration is pursuing policies to reduce regulations and expenditures across government including at the HHS, the FDA, Center for Medicare and Medicaid Services ("CMS") and related agencies and has made significant staff reductions at the FDA and other agencies. These actions, presently directed by executive orders or memoranda from the Office of Management and Budget, may propose policy changes that create additional uncertainty for our business. These actions and proposals include for example, (1) reducing agency workforce and cutting programs; (2) rescinding a Biden administration executive order tasking the Center for Medicare and Medicaid Innovation to consider new payment and healthcare models to limit drug spending; (3) eliminating the Biden administration's executive order that directed HHS to establish an AI task force and developing a strategic plan; (4) directing HHS and other agencies to lower prescription drug costs for Medicare through a variety of initiatives, including by improving upon the Medicare Drug Price Negotiation Program and establishing MFN pricing for pharmaceutical products; (5) imposing tariffs on imported pharmaceutical products; (6) directing certain federal agencies to enforce existing law regarding hospital and plan price transparency and by standardizing prices across hospitals and health plans; and (7) as part of the Make America Healthy Again Commission's recent Strategy Report, working across government agencies to increase enforcement on direct-to-consumer pharmaceutical advertising. These actions and policies may significantly reduce U.S. drug prices, potentially impacting manufacturers' global pricing strategies and profitability, while increasing their operational costs and compliance risks. Additionally, in its June 2024 decision in Loper Bright Enterprises v. Raimondo ("Loper Bright"), the U.S. Supreme Court overturned the longstanding Chevron doctrine, under which courts were required to give deference to regulatory agencies' reasonable interpretations of ambiguous federal statutes. The Loper Bright decision could result in additional legal challenges to current regulations and guidance issued by federal agencies applicable to our operations, including those issued by the FDA. Finally, Congress may introduce and ultimately pass health care related legislation that could impact the drug approval process and make changes to the Medicare Drug Price Negotiation Program created under the IRA. We cannot predict which additional measures may be adopted or the impact of current and additional measures on the marketing, pricing and demand for our products, which could have a material adverse effect on our business, financial condition and results of operations.

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 therapies. 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, particularly in light of the upcoming U.S. Presidential and Congressional elections.

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 or perceived as preferred relative to any of our products, or that obtain preferential formulary and reimbursement status. Factors affecting competition in the pharmaceutical and therapeutic 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 therapeutics. The industry in which we compete is characterized by extensive research and development efforts and rapid technological progress. In particular, the competitive landscape for IgAN is rapidly evolving and is expected to continue to evolve as multiple new modalities advance in development and potentially gain approval. For example, Novartis announced in April 2025 that it had received accelerated approval for atrasentan to reduce proteinuria in adults with primary IgAN at risk of rapid disease progression, generally a urine protein-to-creatinine ratio ≥1.5 g/g. Furthermore, 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 our products and product candidates noncompetitive.

Furthermore, competitors could enter the market with generic versions of our products. For example, a generic option for the 100mg version of the original formulation of Thiola (tiopronin tablets) was approved by the FDA in May 2021 and a second 100mg version of the original formulation of Thiola (tiopronin tablets) was approved by the FDA in June 2022. In addition, several generic options for the 100mg and 300mg versions of Thiola EC have been approved by the FDA and become available.

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 in general, and biologics in particular, 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.

In September 2024, we announced a voluntary pause of enrollment in the Phase 3 HARMONY Study evaluating pegtibatinase for the treatment of classical homocystinuria (HCU). The voluntary enrollment pause enables us to work to address necessary process improvements in manufacturing scale-up to support commercial scale manufacturing as well as full enrollment in the HARMONY Study. The voluntary enrollment pause was enacted following our determination that the desired drug substance profile was not achieved in the recent scale-up process. While we have made process improvements and have manufactured material at the larger scale, and while we are engaging with regulatory authorities with a goal to restart enrollment in the Phase 3 HARMONY Study in 2026, there is no guarantee that regulators will agree with our assessment or that the Phase 3 HARMONY Study will resume on the anticipated timeline or be successful. In addition, external factors including supply chain risks, geopolitical factors, and matters related to the staffing, resources and prioritization at the FDA and other government agencies, among others, could have impacts on our anticipated timeline.

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 and comparable foreign regulatory authority requirements. 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 to monitor and ensure compliance with cGMP. 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 shutdown of the third-party vendor or invalidation of drug product lots or processes, fines, injunctions, civil penalties, failure of regulatory authorities to grant marketing approval of our product candidates, delays, suspension, variation 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, inflation and global supply chain disruptions, as well as past disruptions related to COVID-19 and potential future disruptions related to a future health epidemic or pandemic, wars, armed conflicts, tariffs 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. Changes in legislation could potentially impact our ability to secure the materials we need for our products and product candidates. For example, the U.S. Senate passed a bill in October 2025 that could restrict business with Chinese biotech companies. If this bill becomes law, or if other new laws or regulations prohibiting us from dealing with suppliers in China, we may have to find alternative suppliers and our ability to secure the materials we need on our planned timelines could be adversely impacted. 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 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 processes. Additionally, in September 2024, we announced a voluntary pause of enrollment in the Phase 3 HARMONY Study to enable us to work to address necessary process improvements in manufacturing scale-up to support commercial scale manufacturing as well as full enrollment in the HARMONY Study. The voluntary enrollment pause was enacted following our determination that the desired drug substance profile was not achieved in the initial scale-up process. From time to time we continue to, and may in the future, face supply challenges or shortages of other materials necessary to manufacture pegtibatinase 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

International trade policies, including tariffs, sanctions and trade barriers may adversely affect our business, financial condition, results of operations and prospects.*

We operate in a global economy, and our business depends on a global supply chain for the development, manufacturing, and distribution of our pharmaceutical products, and for the advancement of our pre/nonclinical and clinical development programs. There is inherent risk, based on the complex relationships among the U.S. and the countries in which we conduct our business, that political, diplomatic, and national security factors can lead to global trade restrictions and changes in trade policies and export regulations that may adversely affect our business and operations. The current international trade and regulatory environment is subject to significant ongoing uncertainty.

We source quantities of active pharmaceutical ingredients (APIs), precursor chemicals, and specialized equipment from international suppliers, including from manufacturers in China, consistent with broader industry practices. While the impact of tariff policies on our business has been minimal to date, current or future tariff policies, particularly those affecting China and pharmaceutical products, could further increase our costs, and may affect profitability, particularly in formulary-based markets where pricing flexibility may be limited. Recent and potential future changes in international trade policies, particularly regarding U.S.-China trade relations and pharmaceutical-specific tariffs, present potential risks to our future operations and financial performance.

The ongoing trade tensions between the United States and other countries including China have resulted in multiple rounds of tariffs affecting pharmaceutical ingredients, manufacturing equipment, and related supplies. The evolving tariff and trade landscape contributes to planning challenges for global pharmaceutical operations. Changes in tariff classifications, country-of-origin requirements, or customs procedures can occur with limited notice. This uncertainty complicates our long-term investment decisions regarding manufacturing facilities, supply chain optimization, and research and development locations.

Recent policy discussions have included potential targeted tariffs or other trade measures specifically aimed at pharmaceutical products and ingredients as part of broader healthcare cost control or national security initiatives. Pharmaceuticals and biologics face regulatory and technical constraints that make rapid supply chain adjustments challenging, complex and costly. Identifying and qualifying a new alternative supplier with available capacity and capabilities— whether in the U.S. or in another country with a more favorable tariff regime—requires a substantial monetary investment and investment of personnel and

other resources, including those related to contracting, qualification, technology transfer, and regulatory approvals, and the process may take an extended period of time to complete.

While the impact of tariff policies on our business has been minimal to date, current or future tariffs may result in increased research and development expenses, including with respect to raw materials, APIs, laboratory equipment and research materials and components. Trade restrictions affecting the import of materials necessary for clinical trials could result in delays to development timelines. Increased costs and extended development timelines could affect competitiveness relative to companies operating in regions with more favorable trade relationships and could impact investor confidence.

The complexity of announced or future tariffs may also increase the risk of enforcement actions related to trade compliance. Foreign governments may adopt non-tariff measures, such as procurement preferences or informal disincentives to engage with, purchase from or invest in U.S. entities. These developments could affect our ability to compete internationally or engage with global suppliers, customers and partners. Retaliatory actions, such as changes to intellectual property protection, increased enforcement, or delays in regulatory approvals, could result in legal and operational risks.

In addition, the United States and other governments have imposed and may continue to impose additional sanctions, such as trade restrictions or trade barriers, which could restrict us from doing business directly or indirectly in or with certain countries or parties and may impose additional costs and complexity to our business.

Trade disputes, tariffs, restrictions and other political tensions between the United States and other countries may also exacerbate unfavorable macroeconomic conditions including inflationary pressures, foreign exchange volatility, financial market instability, and economic recessions or downturns. The ultimate impact of current or future tariffs and trade restrictions remains uncertain. While we actively monitor these risks and manage our supply chain accordingly, prolonged economic or geopolitical disruptions could adversely affect our business, ability to access the capital markets or other financing sources, results of operations, financial condition and prospects. In addition, tariffs and other trade developments have and may continue to heighten the risks related to the other risk factors described elsewhere in this report.

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. To appropriately manage for our future, we must continue to implement and improve our managerial, operational and financial systems, continue to recruit, train and retain qualified personnel as needed, and successfully integrate any changes 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, train and retain qualified personnel, including in connection with the ongoing commercial launch of FILSPARI in the United States. The management of changes to 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 or other changes in our organization 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 therapeutics, 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 in connection with our 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 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. New health epidemics or pandemics may emerge that result in similar or more severe disruptions to our business, which could adversely impact our business and operating results.

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 certain of our expenses will continue to increase, depending on factors including but not limited to: the continuation and cost of our clinical trials and the research and development of additional product candidates; the 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, costs related to our 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 therapies 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 the EU 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 or comparable foreign regulatory authority 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 ("D&O insurance"), 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 D&O insurance 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 potential 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 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 high interest rates and stock price volatility, actual or anticipated bank failures, new or increased tariffs, and ongoing issues arising global geopolitical tensions, including the wars and other armed conflicts, 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 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 resulting from generic entrants or health epidemics or pandemics;
- debt service obligations on the 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 has been in the past, and may be in the future, 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 high interest rates, actual or anticipated bank failures, new or increased tariffs, wars, armed conflicts and global geopolitical tensions;
- results of clinical trials conducted by others on therapies 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 not receive some or all of the potential milestone and/or royalty payments from our corporate and licensing transactions.

From time to time, we engage in corporate transactions and licensing transactions that include potential milestone payments and/or royalties. For example, on July 16, 2023, we entered into a definitive asset purchase agreement (the "Purchase Agreement") with Mirum Pharmaceuticals, Inc. ("Mirum"), pursuant to which we agreed to sell to Mirum, subject to the terms of the Purchase Agreement, our bile acid product portfolio including Chenodal and Cholbam (also known as Kolbam) (the "Products"). The closing of the transaction occurred on August 31, 2023. A portion of the consideration for the sale is in the form of milestone payments that will only be payable upon the achievement of certain milestones based on specified amounts of annual net sales of the Products. We are also party to license agreements with CSL Vifor and Renalys Pharma, Inc. pursuant to which we are entitled to receive certain payments contingent on the future achievement of specified milestones, and royalty payments based on potential future sales in specified licensed territories. There is a risk that any or all of the milestone events under these various agreements might not be achieved, that our licensees may not achieve sales that would entitle us to royalty payments, and that any or all of the consideration tied to the achievement of the milestone events and/or royalties might not be received.

In October 2025, our partner Renalys announced that it has entered into a definitive stock purchase agreement with Chugai Pharmaceutical Co., Ltd. ("Chugai") pursuant to which, upon the closing of the transaction, Chugai will acquire full ownership of Renalys and will gain exclusive rights to develop and commercialize sparsentan in Japan, South Korea, and Taiwan. If and when the closing occurs, Travere will be entitled to receive a portion of the upfront payment as a minority shareholder in Renalys, and will also be eligible to receive future payments upon the achievement of specified regulatory milestones for sparsentan and royalties on net sales in Japan, South Korea, and Taiwan. There is no guarantee that the transaction will close on the anticipated timeline, or at all, or that we will receive any or all of the consideration that is due upon closing or tied to the regulatory milestones or net sales.

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

We may in the future 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 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 authority 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 authority, including the FDA, may send enforcement letters, mandate labeling changes, suspend, vary or withdraw regulatory approval, suspend, vary or terminate 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 supranational, national, regional, state and local agencies and regulatory authorities, including but not limited to the FDA, the 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, and comparable foreign regulatory acts, 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 authorities. 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 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.

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.

If our operations are found to be in violation of any of the health regulatory laws described above or any other laws that apply to us, we may be subject to significant penalties, including imprisonment, criminal fines, civil monetary penalties, administrative penalties, disgorgement, exclusion from participation in federal healthcare programs, contractual damages, injunctions, recall or seizure of products, total or partial suspension of production, reputational harm, administrative burdens, additional oversight and reporting obligations if we become subject to a corporate integrity agreement or similar agreement to resolve allegation of non-compliance with these laws, diminished profits and future earnings, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

We are also subject to foreign requirements comparable to those established above. Outside the United States, interactions between pharmaceutical companies and health care professionals are also governed by strict laws, such as national anti-bribery laws of European countries, national sunshine rules, regulations, industry self-regulation codes of conduct and physicians' codes of professional conduct. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

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. Comparable requirements are applicable outside the United States.

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 or comparable foreign regulatory authority pre-approval inspection of the facility or facilities at which the product is manufactured to assess compliance with the FDA's cGMP regulations or comparable foreign regulatory authority requirements 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 suspended, varied or withdrawn, including for failure to comply with regulatory requirements or if clinical or manufacturing problems follow initial marketing.

We and the third parties with whom we work are subject to stringent and changing U.S. and foreign laws, regulations, and rules, contractual obligations, industry standards, policies and other obligations related to data privacy and security. Our (or the third parties with whom we work) actual or perceived failure to comply with such obligations could lead to regulatory investigations or actions; litigation (including class claims) and mass arbitration demands; 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 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 health data 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.

Numerous U.S. states have enacted comprehensive privacy laws that impose certain obligations on covered businesses, including providing specific disclosures in privacy notices and affording residents with certain rights concerning their personal data. As applicable, such rights include the right to access, correct, or delete certain personal data, and to opt-out of certain data processing activities, such as targeted advertising, profiling, and automated decision-making. The exercise of these rights may impact our business and ability to provide our products and services. Certain states also impose stricter requirements for processing certain personal data, including sensitive information, such as conducting data privacy impact assessments. These state laws allow for statutory fines for noncompliance. For example, the California Consumer Privacy Act of 2018 ("CCPA"), as amended, applies to personal data of consumers, business representatives, and employees who are California residents, and requires businesses to provide specific disclosures in privacy notices, and affords California residents certain privacy rights related to their personal data, such as those noted herein. The CCPA allows for fines for certain noncompliance and allows private litigants affected by certain data breaches to recover significant statutory damages. The CCPA and other U.S. comprehensive privacy laws exempt some data processed in the context of clinical trials, but these laws increase compliance costs and potential liability with respect to certain other personal data we maintain about residents in certain states. Similar laws are being considered in several other states, as well as at the local level, and we expect more jurisdictions to pass similar laws in the future.

In addition, numerous U.S. states—including but not limited to Connecticut, Nevada and Washington—have enacted new laws governing the privacy of consumer health data. For example, Washington's My Health My Data Act broadly defines consumer health data, places restrictions on processing consumer health data (including imposing stringent requirements for consents), provides consumers certain rights with respect to their health data, and creates a private right of action to allow individuals to sue for violations of the law. Other states are considering and may adopt similar laws.

Additionally, under various privacy laws and other obligations, we are required to obtain certain consents to process personal data. For example, some of our data processing practices may be challenged under wiretapping laws, since we obtain consumer information from third parties through various methods, including via third-party marketing pixels. These practices may be subject to increased challenges by class action plaintiffs. Our inability or failure to obtain consent for these practices could result in adverse consequences, including class action litigation and mass arbitration demands.

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") (EU GDPR and UK GDPR, collectively "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 GDPR imposes significant and complex burdens on processing personal data, which is relevant to our operations in the context of our conduct of clinical trials and is of interest to relevant regulators. Under the GDPR, government regulators can impose temporary or definitive bans on data processing, as well as fines of up to 20 million euros under the EU GDPR, 17.5 million pounds sterling under the UK GDPR or, in each case, or 4% of annual global revenue, whichever is greater. Further, under the 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, and may become subject to in the future. We are also bound by contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. Additionally, we publish privacy policies, marketing materials and other statements, such as compliance with certain certifications, regarding data privacy and security. Regulators in the United States are increasingly scrutinizing these statements. If these policies, materials or statements are found to be deficient, lacking in transparency, deceptive, unfair, misleading, 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 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 generally believes are inadequate. Other jurisdictions may adopt or have already adopted similarly stringent 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 are used to transfer personal data from the EEA and UK to the United States in compliance with these laws, such as the EU Standard Contractual Clauses ("EU SCCs"), the UK's International Data Transfer Agreement / International Data Transfer Addendum to the EU

SCCs, and the EU-U.S. Data Privacy Framework and the UK extension thereto (which allows for transfers to relevant U.S.-based organizations who self-certify compliance and participate in the applicable frameworks), these mechanisms may be subject to legal challenges, and there is no assurance that we can satisfy or rely on the Data Privacy Framework 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 third parties with whom we work (such as 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 (such as Europe) 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 activist 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.

Additionally, the U.S. Department of Justice issued a rule entitled the Preventing Access to U.S. Sensitive Personal Data and Government-Related Data by Countries of Concern or Covered Persons, which places additional restriction on certain data transactions involving countries of concern (e.g., China, Russia, Iran) and covered individuals (i.e., individuals and entities located in or controlled by individuals or entities located in those jurisdictions) that may impact certain business activities such as vendor engagements, sale or sharing of data, employment of certain individuals, and investor agreements. Violations of the rule could lead to significant civil and criminal fines and penalties. The rule applies regardless of whether data is anonymized, key-coded, pseudonymized, de-identified or encrypted, which presents particular challenges for companies like ours and may impact our ability to transfer data in connection with certain transactions or agreements.

In Europe, the Network and Information Security Directive ("NIS 2") regulates the cyber resilience and incident response capabilities of entities operating in a number of sectors, including the health sector. Although NIS 2 has not yet been transposed into domestic law in Ireland, we may be required to comply with its provisions. Achieving compliance with NIS 2 may require significant investment of our time and resources.

Our obligations related to data privacy and security (and consumers' data privacy expectations) are quickly changing in an increasingly stringent fashion, creating uncertainty. Additionally, these obligations are 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 with whom we work. 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 with whom we work 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 with whom we work 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 the third parties with whom we work 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, litigation (including class actions), or mass arbitration demands 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. In particular, plaintiffs have become increasingly more active in bringing privacy-related claims against companies, including class claims and mass arbitration demands. Some of these claims allow for the recovery of statutory damages on a per violation basis, and, if viable, carry the potential for monumental statutory damages, depending on the volume of data and the number of violations. Any 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. Moreover, clinical trial subjects and other individuals about whom we or the third parties with whom we work obtain personal data, as well as the third parties with whom we work who share this information with us, may limit our ability to collect, use and disclose the information. Claims that we have violated individuals' privacy rights, failed to comply with data protection laws, or breached our contractual obligations, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business.

If our information technology systems or data, or those of third parties with whom we work, 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 commercial operations, clinical trials or other operations; 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 the third parties with whom we work 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).

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, we and the third parties with whom we work 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 products. For example, we work with third parties to support our business located in unstable regions and regions experiencing (or expected to experience) geopolitical or other conflicts, including in Israel, where businesses have experienced an increase in cyberattacks in relation to the Israel/Hamas conflict. We and the third parties with whom we work are subject to a variety of other evolving threats, including, but not limited to, social-engineering attacks (including through deep fakes, which are increasingly more difficult to identify as fake, and phishing attacks), malicious code (such as viruses and worms), malware (including as a result of advanced persistent threat intrusions), denial-of-service attacks, 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, attacks enhanced or facilitated by artificial intelligence, 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, ability to provide our products, 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, hybrid and remote work has increased risks to our information technology systems and data, as our employees utilize network connections, computers, and devices outside our premises or network, including working at home, while in transit, and in public locations. Future or past business transactions (such as acquisitions or integrations) could also expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. Furthermore, we may discover security issues that were not found during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

We rely upon third parties and technologies to operate critical business systems to process sensitive information in a variety of contexts, including, without limitation, cloud-based infrastructure, encryption and authentication technology, employee email, and other functions. We also rely on third parties to provide certain products, including active pharmaceutical ingredients or API, 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 the third parties with whom we work 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 that of the third parties with whom we work have not been compromised.

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 designed to detect, mitigate and remediate vulnerabilities in our information security systems (such as our hardware and/or software, including that of third parties with whom we work), but we have not in the past and may not in the future be able to detect, mitigate, and remediate all such vulnerabilities including on a timely basis. Despite our efforts, there can be no assurance that these vulnerability mitigation measures will be effective. It may also be difficult and/or costly to detect, investigate, mitigate, contain, and remediate a security incident. Further, we have experienced, and may in the future experience, delays in developing and deploying remedial measures and patches designed to address identified vulnerabilities. If exploited, certain vulnerabilities can result in a security incident. We have experienced, and may in the future experience, security incidents involving our systems and the systems of third parties with whom we conduct business. Actions taken by us or the third parties with whom we work to detect, investigate, mitigate, contain, and remediate a security incident could result in outages, data losses, and disruptions of our business. Threat actors may also gain access to other networks and systems after a compromise of our networks and systems.

Certain of the previously identified or similar threats have in the past, and may in the future, cause a security incident or other interruption that could result in unauthorized, unlawful, or accidental acquisition, modification, destruction, loss, alteration, encryption, disclosure of, or access to our sensitive information or our information technology systems, or those of the third parties with whom we work. We have experienced, and may in the future experience, security incidents involving our systems and the systems of third parties with whom we conduct business. A security incident or other interruption could disrupt our ability (and that of third parties with whom we work) to provide our products. We expend resources and may have to 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.

Applicable data security and public company disclosure obligations require us, or we may voluntarily choose, to notify relevant stakeholders of certain security incidents, including affected individuals, customers, regulators and investors, or to take other actions in certain circumstances, such as providing credit monitoring and identity theft protection services. Whether a cybersecurity incident is reportable to our investors may not be straightforward, may take considerable time to determine, and may be subject to change as the investigation of the incident progresses, including changes that may significantly alter any initial disclosure that we provide. Such disclosures and related actions can be costly, and the disclosures or the failure to comply with such applicable requirements, could lead to adverse consequences. We have experienced, and may in the future experience, security incidents involving our systems and the systems of third parties with whom we conduct business. Any incidents or perceived incidents involving our systems (or those of a third party with whom we work) may lead to adverse consequences, such as 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; loss of customer, investor or partner confidence in the effectiveness of our cybersecurity measures; monetary fund diversions; the expenditure of significant capital and other resources; diversion of management attention; interruptions in our operations (including availability of data); financial loss and other similar harms. 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.

Some of our contracts do 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.

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. Sensitive information of us or our customers could also be leaked, disclosed, or revealed as a result of or in connection with our employee's, personnel's, or third parties with whom we work use of generative AI technologies.

Risks related to the use of artificial intelligence technologies could adversely affect our business, financial condition and/or operating results.*

Our employees and personnel use generative artificial intelligence, machine learning and other artificial intelligence technologies (together, "AI/ML") and/or automated decision-making technologies to perform their work, and the disclosure and use of personal data in AI/ML technologies is subject to various privacy laws and other privacy obligations. Governments have passed and are likely to pass additional laws regulating AI/ML and/or automated decision-making technologies. Our use of this technology could result in additional compliance costs, regulatory investigations and actions, and lawsuits. If we are unable to use AI/ML and/or automated decision-making technologies in the future, it could make our business less efficient and result in competitive disadvantages. Any sensitive information (including confidential, competitive, proprietary, or personal data) that we input into a third-party generative AI/ML platform could be leaked or disclosed to others, including if sensitive information is used to train the third parties' AI/ML model. Additionally, where an AI/ML model ingests personal data and makes connections using such data, those technologies may reveal other personal or sensitive information generated by the model. Moreover, AI/ML models may create flawed, incomplete, or inaccurate outputs, some of which may appear correct. This may happen if the inputs that the model relied on were inaccurate, incomplete or flawed (including if a bad actor "poisons" the AI/ML with bad inputs or logic), or if the logic of the AI/ML is flawed (a so-called "hallucination"). We may use AI/ML outputs to make certain decisions. Due to these potential inaccuracies or flaws, the model could be biased and could lead us to make decisions that could bias certain individuals (or classes of individuals), and adversely impact their rights, employment, and ability to obtain certain pricing, products, services, or benefits.

Several jurisdictions around the globe, including Europe and certain U.S. states, have proposed, enacted, or are considering laws governing AI/ML, including the EU's AI Act and the Colorado AI Act. For example, the EU AI Act sets out a risk-based framework, subjecting certain AI technologies to numerous compliance obligations, including transparency, conformity and risk assessment, monitoring and human oversight requirements. Under the EU AI Act, non-compliant companies may be subject to administrative fines of up to 35 million Euros or 7% of a company's total worldwide annual turnover for the preceding financial year, whichever is the higher. Certain of our activities subject us to the EU AI Act and depending on how the EU AI Act is implemented and interpreted, we may have to adapt our business practices, contractual arrangements, and services to comply with such obligations. We expect other jurisdictions will adopt similar laws. Additionally, certain privacy laws extend rights to consumers (such as the right to delete certain personal data) and regulate automated decision making, which may be incompatible with our use of AI/ML. These obligations may make it harder for us and our employees and personnel to use AI/ML, lead to regulatory fines or penalties, require us to change our business practices or make changes to the AI/ML that we use, or prevent or limit our use of AI/ML. For example, the FTC has required other companies to turn over (or disgorge) valuable insights or trainings generated through the use of AI/ML where they allege the company has violated privacy and consumer protection laws. If we cannot use AI/ML or that use is restricted, our business may be less efficient, or we may be at a competitive disadvantage.

Additionally, sensitive information of the Company or our employees or other individuals could be leaked, disclosed, or revealed as a result of or in connection with our employee's, personnel's, or vendor's use of AI/ML technologies.

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.

The U.S. government recently enacted the OBBBA, that (along with other recent U.S. federal tax reform) has resulted in significant changes to the taxation of business entities including, among other changes, changes to the taxation of income derived from international operations, changes in the deduction and amortization of research and development expenditures, and limitations on the deductibility of business interest. Future guidance from the Internal Revenue Service and other tax authorities with respect to any legislation may affect us, and certain aspects of such legislation could be repealed or modified or sunset in future years.

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.

Our U.S. 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 NOL carryforwards in a taxable year is limited to 80% of taxable income in such year. As of December 31, 2024, we had federal NOL carryforwards of $223.8 million. 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. We continue to evaluate potential historical ownership changes and 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. For example, California imposed limits on the usability of California state net operating losses to offset taxable income in tax years beginning after 2023 and before 2027. 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 or regulatory authorities 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 therapies 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, including beginning on October 1, 2025, 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. In addition, there have been significant staff reductions at the FDA and other agencies, some of which are or may be subject to legal challenges. 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. Comparable considerations may be applicable in relation to foreign regulatory authorities.

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

In recent years, there has been an increased 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. Various policymakers within and outside of the U.S., including the State of California and the European Union, among others, have adopted (or are considering adopting) requirements for certain companies to undertake various actions, including disclosures, on climate-related or other ESG-related matters. These requirements are not uniform and may not be interpreted or applied uniformly, which may increase the cost and complexity of compliance and any associated risks. The ESG landscape has been evolving rapidly, and it can be difficult to predict future developments. For example, in March 2024, the SEC adopted rules designed to enhance and standardize climate-related disclosures, which were stayed pending judicial review, and the SEC subsequently voted to cease its defense of the climate-related disclosure rules, effectively halting their implementation. If other climate-related disclosure rules or other ESG rules become effective or become applicable to us, they may 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.

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.*

The UK withdrawal from the EU on January 31, 2020, commonly referred to as Brexit, has changed the regulatory relationship between the UK and the EU. The MHRA is now the UK's standalone regulator for medicinal products and medical devices. The United Kingdom is no longer subject to EU regulations. Northern Ireland continues to follow certain limited EU regulatory rules, but not in relation to medicinal products.

The UK regulatory framework in relation to clinical trials is governed by the Medicines for Human Use (Clinical Trials) Regulations 2004, as amended, which is derived from the CTD, as implemented into UK national law through secondary legislation. On January 17, 2022, the MHRA launched an eight-week consultation on reframing the UK legislation for clinical trials. The UK Government published its response to the consultation on March 21, 2023 confirming that it would bring forward changes to the legislation, and such changes were laid before Parliament on December 12, 2024. and signed into law on April 11, 2025. The changes include risk-proportionate regulation of clinical trials, with low-risk trials able to receive faster approval through automatic authorisation, a streamlined approval process that integrates both regulatory and ethics committee approvals, leading to a single UK decision for clinical trials, and new legal obligations mandating the registration of clinical trials in public registries and the publication of trial results within 12 months of trial conclusion.

Marketing authorizations in the UK are governed by the Human Medicines Regulations (SI 2012/1916), as amended. Since January 1, 2021, an applicant for the EU centralized procedure marketing authorization can no longer be established in the UK. As a result, since this date, companies established in the UK cannot use the EU centralized procedure and instead must follow one of the UK national authorization procedures or one of the remaining post-Brexit international cooperation procedures to obtain a marketing authorization to market products in the UK. All existing EU marketing authorizations for centrally authorized products were automatically converted or grandfathered into UK marketing authorization, effective in Great Britain only, free of charge on January

1, 2021, unless the marketing authorization holder opted-out of this possibility. Northern Ireland remained within the scope of EU authorizations in relation to centrally authorized medicinal products until January 1, 2025. However, on January 1, 2025, a new arrangement as part of the so-called "Windsor Framework" came into effect and reintegrated Northern Ireland under the regulatory authority of the MHRA with respect to medicinal products. The Windsor Framework also removes EU licensing processes and EU labelling and serialization requirements in relation to Northern Ireland and introduces a UK-wide licensing process for medicines.

The MHRA has also introduced changes to national marketing authorization procedures. This includes introduction of procedures to prioritize access to new medicines that will benefit patients, including a 150-day assessment route, a rolling review procedure and the International Recognition Procedures which entered into application on January 1, 2024. Since January 1, 2024, the MHRA may rely on the International Recognition Procedure, or IRP, when reviewing certain types of marketing authorization applications. This procedure is available for applicants for marketing authorization who have already received an authorization for the same product from a reference regulator. These include the FDA, the EMA, and national competent authorities of individual EEA countries. A positive opinion from the EMA and CHMP, or a positive end of procedure outcome from the mutual recognition or decentralized procedures are considered to be authorizations for the purposes of the IRP.

There is no pre-marketing authorization orphan designation for medicinal products in the UK. Instead, the MHRA reviews applications for orphan designation in parallel to the corresponding marketing authorization application. The criteria are essentially the same as those in the EU, but have been tailored for the market. This includes the criterion that prevalence of the condition in the United Kingdom, rather than the EU, must not be more than five in 10,000. Upon the grant of a marketing authorization with orphan status, the medicinal product will benefit from up to 10 years of market exclusivity from similar products in the approved orphan indication. The start of this market exclusivity period will be set from the date of first approval of the product in 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, 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, or a 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 products or materials that 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.

If material weaknesses in our internal control over financial reporting are discovered or occur in the future, our consolidated financial statements may contain material misstatements and we could be required to restate our financial results, which could adversely affect our stock price and result in an inability to maintain compliance with applicable stock exchange listing requirements.

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. If material weaknesses in our internal control over financial reporting are discovered or occur in the future, or if we are unable to maintain effective internal control over financial reporting or disclosure controls and procedures for any reason, 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 any 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. 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.

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 concerns or uncertainty relating to tariffs and their potential impact on the economy, levels of 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. 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 the end of the period covered by this report, we had approximately $316 million of total debt outstanding, all of which is 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 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 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 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 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 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 2029 Notes or pay cash upon their conversion.*

Noteholders may require us to repurchase their 2029 Notes following a fundamental change at a cash repurchase price generally equal to the principal amount of the 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 2029 Notes or pay the cash amounts due upon conversion of the 2029 Notes. In addition, applicable law, regulatory authorities and the agreements governing our future indebtedness may restrict our ability to repurchase the 2029 Notes or pay the cash amounts due upon conversion of the 2029 Notes. Our failure to repurchase the 2029 Notes or to pay the cash amounts due upon conversion of the 2029 Notes when required will constitute a default under the base and supplemental indentures that govern the 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 2029 Notes.

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

If an event of default under the 2029 Notes occurs, the principal amount of 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 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 2029 Notes, unless cured or waived, may have a material adverse effect on our results of operations.

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

Certain provisions of the 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 2029 Notes will have the right, at their option, to require us to repurchase all of their 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 2029 Notes.*

To the extent we issue shares of common stock upon conversion of the 2029 Notes, the conversion of some or all of the 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 2029 Notes may encourage short selling by market participants because the conversion of the 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 high interest rates, bank failures, tariffs, other restrictive trade policies, wars, armed conflicts and global geopolitical tension, and may experience disruptions in the future. These disruptions can result in severely diminished liquidity and credit availability, increased inflation, declines in consumer confidence, declines in economic growth, increases in unemployment rates, recessions 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 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.

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

Trading Arrangements

During the fiscal quarter ended September 30, 2025, none of our directors and/or officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted or terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement," as those terms are defined in Regulation S-K, Item 408.

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 the Company, 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).
31.1	Chief Executive Officer's Certification pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Chief Financial Officer's Certification pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Chief Executive Officer's Certification pursuant to Section 906 of the Sarbanes Oxley Act of 2002
32.2	Chief Financial Officer's Certification pursuant to Section 906 of the Sarbanes Oxley Act of 2002
101.INS	Inline XBRL Instance Document
101.SCH	Inline XBRL Taxonomy Extension Schema Document
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Taxonomy Extension Presentation Linkbase Document
104	The cover page to this Quarterly Report on Form 10-Q has been formatted in Inline XBRL

SIGNATURES

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

Date: October 30, 2025

TRAVERE THERAPEUTICS, INC.

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

By: /s/ Christopher Cline
Name: Christopher Cline
Title: Chief Financial Officer

Exhibit 31.1

CERTIFICATION OF CHIEF EXECUTIVE OFFICER

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

I, Eric 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: October 30, 2025

/s/ Eric Dube

Eric 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: October 30, 2025

/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 September 30, 2025 (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: October 30, 2025

/s/ Eric Dube

Eric 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 September 30, 2025 (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: October 30, 2025

/s/ Christopher Cline

Christopher Cline
Chief Financial Officer
(Principal Financial Officer)