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, 2026

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 28, 2026 was 95,992,155.

TRAVERE THERAPEUTICS, INC.

Form 10-Q
For the Fiscal Quarter Ended September 30, 2026

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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, 2025, 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.
- 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.
- 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.
- 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.
- 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.
- 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.
- International trade policies, including tariffs, sanctions and trade barriers may adversely affect our business, financial condition, results of operations and prospects.
- 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, 2026	December 31, 2025
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 123,542	$ 93,035
Marketable debt securities, at fair value	348,276	229,761
Accounts receivable, net	107,528	80,134
Inventory	10,516	5,875
Prepaid expenses and other current assets	43,584	28,760
Total current assets	633,446	437,565
Long-term inventory	33,287	30,280
Property and equipment, net	2,724	4,022
Operating lease right of use assets	7,807	10,576
Intangible assets, net	164,108	113,868
Other assets	6,857	8,880
Total assets	$ 848,229	$ 605,191
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 15,284	$ 24,800
Accrued expenses	187,216	126,035
Operating lease liabilities, current portion	6,312	5,875
Other current liabilities	2,100	3,194
Total current liabilities	210,912	159,904
Convertible debt	603,510	311,724
Operating lease liabilities, less current portion	6,333	11,134
Other non-current liabilities	4,890	7,601
Total liabilities	825,645	490,363
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, 2026 and December 31, 2025	—	—
Common stock $0.0001 par value; 200,000,000 shares authorized; 95,673,465 and 90,922,868 issued and outstanding as of September 30, 2026 and December 31, 2025, respectively	9	9
Additional paid-in capital	1,621,570	1,588,721
Accumulated deficit	(1,596,949)	(1,472,713)
Accumulated other comprehensive loss	(2,046)	(1,189)
Total stockholders' equity	22,584	114,828
Total liabilities and stockholders' equity	$ 848,229	$ 605,191

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Net product sales	$ 220,196	$ 113,150	$ 506,040	$ 283,852
License and collaboration revenue	4,367	51,709	15,305	77,187
Total revenue	224,563	164,859	521,345	361,039
Operating expenses:				
Cost of goods sold	2,867	1,585	6,991	7,786
Research and development	70,304	51,890	187,680	148,141
Selling, general and administrative	98,414	71,046	274,779	194,041
Royalty expense	7,968	15,407	39,846	41,467
In-process research and development	113,115	—	113,115	—
Total operating expenses	292,668	139,928	622,411	391,435
Operating (loss) income	(68,105)	24,931	(101,066)	(30,396)
Other income (expense), net:				
Interest income	3,704	3,047	9,643	10,129
Interest expense	(2,140)	(2,751)	(6,575)	(8,452)
Other (expense) income, net	(391)	487	(650)	511
Inducement expense	—	—	(40,008)	—
Total other income (expense), net	1,173	783	(37,590)	2,188
(Loss) income from continuing operations before income tax	(66,932)	25,714	(138,656)	(28,208)
Income tax benefit (provision) on continuing operations	5,895	(8)	6,220	(67)
(Loss) income from continuing operations, net of tax	(61,037)	25,706	(132,436)	(28,275)
Income from discontinued operations, net of tax	8,700	—	8,200	—
Net (loss) income	$ (52,337)	$ 25,706	$ (124,236)	$ (28,275)
Per share data				
Basic and diluted:				
Net (loss) income from continuing operations, basic	$ (0.64)	$ 0.29	$ (1.42)	$ (0.32)
Net (loss) income from continuing operations, diluted	$ (0.64)	$ 0.28	$ (1.42)	$ (0.32)
Net income from discontinued operations, basic and diluted	$ 0.09	$ —	$ 0.09	$ —
Net (loss) income per common share, basic	$ (0.55)	$ 0.29	$ (1.33)	$ (0.32)
Net (loss) income per common share, diluted	$ (0.55)	$ 0.28	$ (1.33)	$ (0.32)
Weighted average common shares outstanding, basic	94,627,788	89,230,420	93,335,461	88,847,209
Weighted average common shares outstanding, diluted	94,627,788	102,618,560	93,335,461	88,847,209
Comprehensive (loss) income:				
Net (loss) income	$ (52,337)	$ 25,706	$ (124,236)	$ (28,275)
Foreign currency translation gain (loss)	450	(284)	1,367	(702)
Unrealized (loss) gain on marketable debt securities	(1,379)	42	(2,224)	(272)
Comprehensive (loss) income	$ (53,266)	$ 25,464	$ (125,093)	$ (29,249)

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, 2026						Three Months Ended September 30, 2025					
	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount					Shares	Amount				
Balance - June 30	**94,174,767**	**$ 9**	**$1,570,242**	**$ (1,117)**	**$ (1,544,612)**	**$ 24,522**	**89,102,347**	**$ 9**	**$1,534,698**	**$ (812)**	**$ (1,501,148)**	**$ 32,747**
Share based compensation	—	—	22,885	—	—	22,885	—	—	10,901	—	—	10,901
Issuance of common stock under the equity incentive plan and proceeds from exercise	1,498,698	—	27,891	—	—	27,891	354,279	—	4,090	—	—	4,090
Employee stock purchase program expense	—	—	552	—	—	552	—	—	362	—	—	362
Foreign currency translation adjustments	—	—	—	450	—	450	—	—	—	(284)	—	(284)
Unrealized (loss) gain on marketable debt securities	—	—	—	(1,379)	—	(1,379)	—	—	—	42	—	42
Net (loss) income	—	—	—	—	(52,337)	(52,337)	—	—	—	—	25,706	25,706
Balance - September 30	**95,673,465**	**$ 9**	**$1,621,570**	**$ (2,046)**	**$ (1,596,949)**	**$ 22,584**	**89,456,626**	**$ 9**	**$1,550,051**	**$ (1,054)**	**$ (1,475,442)**	**$ 73,564**

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, 2026						Nine Months Ended September 30, 2025					
	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount					Shares	Amount				
Balance - December 31	**90,922,868**	**$ 9**	**$1,588,721**	**$ (1,189)**	**$ (1,472,713)**	**$ 114,828**	**87,452,835**	**$ 9**	**$1,506,315**	**$ (80)**	**$ (1,447,167)**	**$ 59,077**
Share based compensation	—	—	59,893	—	—	59,893	—	—	32,410	—	—	32,410
Issuance of common stock under the equity incentive plan and proceeds from exercise	4,671,815	—	60,461	—	—	60,461	1,883,440	—	8,796	—	—	8,796
Employee stock purchase program purchase and expense	78,782	—	3,835	—	—	3,835	120,351	—	2,530	—	—	2,530
Induced conversion on 2029 Notes	—	—	(91,340)	—	—	(91,340)	—	—	—	—	—	—
Foreign currency translation adjustments	—	—	—	1,367	—	1,367	—	—	—	(702)	—	(702)
Unrealized loss on marketable debt securities	—	—	—	(2,224)	—	(2,224)	—	—	—	(272)	—	(272)
Net loss	—	—	—	—	(124,236)	(124,236)	—	—	—	—	(28,275)	(28,275)
Balance - September 30	**95,673,465**	**$ 9**	**$1,621,570**	**$ (2,046)**	**$ (1,596,949)**	**$ 22,584**	**89,456,626**	**$ 9**	**$1,550,051**	**$ (1,054)**	**$ (1,475,442)**	**$ 73,564**

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

	For the Nine Months Ended September 30,	
	2026	**2025**
Cash Flows From Operating Activities:		
Net loss	$ (124,236)	$ (28,275)
Net income from discontinued operations	8,200	—
Net loss from continuing operations	(132,436)	(28,275)
Adjustments to reconcile net loss to net cash provided by (used in) operating activities:		
Depreciation and amortization	34,645	42,606
Share based compensation	61,372	33,404
Foreign currency remeasurement impact	1,362	(2,466)
Inducement expense	40,008	—
Acquired in-process research and development	113,115	—
Other	(3,620)	(1,500)
Changes in operating assets and liabilities:		
Accounts receivable	(42,505)	(55,641)
Inventory	(6,054)	3,839
Prepaid expenses and other current and non-current assets	(12,466)	(8,104)
Accounts payable	(3,525)	(6,332)
Accrued expenses	44,546	11,501
Deferred revenue	—	(2,815)
Other current and non-current liabilities	(2,033)	(9,111)
Net cash provided by (used in) operating activities - continuing operations	92,409	(22,894)
Net cash provided by operating activities - discontinued operations	23,500	—
Net cash provided by (used in) operating activities	115,909	(22,894)
Cash Flows From Investing Activities:		
Proceeds from the sale and maturity of marketable debt securities	164,060	241,175
Purchase of marketable debt securities	(283,310)	(69,217)
Purchase of fixed assets	—	(375)
Purchase of intangible assets	(71,999)	(38,918)
Acquisition of in-process research and development	(113,115)	—
Other	—	66
Net cash (used in) provided by investing activities	(304,364)	132,731
Cash Flows From Financing Activities:		
Payment of guaranteed minimum royalty	(1,575)	(1,575)
Proceeds from issuance of 2032 convertible senior notes	525,000	—
Payment of debt issuance costs	(16,348)	—
Repayment of 2025 convertible senior notes	—	(68,904)
Repurchase of 2029 convertible senior notes including premium	(350,885)	—
Proceeds from exercise of stock options	60,461	8,796
Proceeds from issuances under the employee stock purchase plan	2,356	1,537
Net cash provided by (used in) financing activities	219,009	(60,146)
Effect of exchange rate changes on cash	(47)	2,704
Net increase in cash and cash equivalents	30,507	52,395
Cash and cash equivalents, beginning of year	93,035	58,535
Cash and cash equivalents, end of period	**$ 123,542**	**$ 110,930**

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)

Treatment of IgAN

In September 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).

Treatment of FSGS

On April 13, 2026, the FDA granted full (traditional) approval of FILSPARI (sparsentan) to reduce proteinuria in adult and pediatric patients aged 8 years and older with focal segmental glomerulosclerosis (“FSGS”) without nephrotic syndrome. In the Phase 3 DUPLEX Study patients treated with FILSPARI in the overall study population experienced a significant reduction in proteinuria compared to those treated with maximum labeled dose irbesartan.

FILSPARI is the first and only medicine approved by the FDA for the treatment of FSGS.

License and Collaboration Agreements

In September 2021, the Company entered into a license and collaboration agreement (the "CSL Vifor 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 ("UPCR") ≥0.75 g/g), and in April 2025, the Company and CSL Vifor announced that the European Commission had 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 UPCR ≥0.75 g/g). The MA is granted for all member states of the European Union, as well as in Iceland, Liechtenstein and Norway.

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. 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 in November 2024. In the fourth quarter of 2025, Renalys announced positive topline results from its Phase 3 study of sparsentan in Japanese patients with IgAN. Renalys also announced that it had reached an agreement with the Pharmaceuticals and Medical Devices Agency ("PMDA") regarding development plans for two other Phase 3 clinical trials of sparsentan, one investigating the use of sparsentan in FSGS and the other in Alport syndrome, in Japan. In the fourth quarter of 2025, Renalys was acquired by and merged into Chugai Pharmaceutical Co., Ltd. (“Chugai”). Through the acquisition, Chugai gained exclusive rights to develop and commercialize sparsentan in Japan, South Korea, and Taiwan. As a minority shareholder in Renalys, Travere received $10.2 million at the closing of the transaction in the fourth quarter of 2025, and Travere is also eligible to receive multiple milestones according to the progress of sparsentan regulatory approval, and consideration linked to sparsentan's net sales in the applicable territory. Under the terms of the licensing agreement, Chugai 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:

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 was enacted following the Company's determination that the desired drug substance profile was not achieved in the initial scale-up process, and it enabled the Company to address necessary process improvements in manufacturing scale-up to support initial commercial scale manufacturing as well as full enrollment in the HARMONY Study. Following process improvements, the Company restarted enrollment activities in the first quarter of 2026 and dosed the first new patient following study restart in April 2026.

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

Civorebrutinib

Civorebrutinib is an investigational oral, covalent reversible Bruton's tyrosine kinase ("BTK") inhibitor designed to provide differentiated efficacy, safety and convenience for patients with rare, immune-mediated kidney diseases, with planned evaluation in primary membranous nephropathy ("PMN"), FSGS, minimal change disease ("MCD") and potentially additional indications. BTK is a key mediator of B-cell receptor signaling and plays an important role in B-cell activation, maturation, proliferation, and differentiation into antibody-producing cells.

In immune-mediated kidney diseases, B-cell activation and autoantibody production are believed to contribute directly to kidney injury. Civorebrutinib has demonstrated proof of concept in a Phase 1/2 clinical trial of patients with PMN. The previously reported Phase 1/2 data demonstrated rapid and sustained reductions in anti-PLA2R autoantibodies and proteinuria, with high rates of immunologic and clinical remission and stable kidney function through 52 weeks of follow-up. Civorebrutinib has been generally well tolerated throughout the development program to date.

In June 2026, the Company entered into a license and collaboration agreement (the "Everest Agreement") with Everest Medicines (Singapore) Pte. Ltd. ("Everest"), pursuant to which the Company obtained an exclusive license to develop, manufacture and commercialize civorebrutinib in the United States and global markets excluding Greater China and certain countries in East and Southeast Asia (the "Territory"). The Everest Agreement became effective in July 2026 following the satisfaction of customary closing conditions.

The Company and Everest are collaborating on global clinical development, including sharing development costs for global clinical trials. Except for certain global development activities, the Company is responsible for development and commercialization of civorebrutinib in the Territory, while Everest retains responsibility for its territory.

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, 2025, filed with the SEC on February 19, 2026. 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, 2025 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. The Company revised the presentation of amortization expense of certain royalty and milestone payments associated with license agreements acquired in prior asset acquisitions that were accounted for under the asset accumulation method. Historically, the royalty amortization expense was included within selling, general and administrative expenses in the consolidated statements of operations. Beginning in the current year, the Company presents these amounts separately as royalty expense to better reflect the nature of these costs as consideration payable to licensors based on sales of licensed products and regulatory milestone payments. As a result of this change in presentation, $15.4 million and $41.5 million of amortization expense previously included within selling, general and administrative expenses has been reclassified to royalty expense for the three and nine months ended September 30, 2025, respectively. This reclassification had no impact on total operating expenses, loss from operations, net loss, net loss per share, or cash flows for any period presented.

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").

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 ("APIs") 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, 2026 and 2025 (*in thousands*):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Cost of goods sold - product sales	$ 2,867	$ 1,308	$ 6,991	$ 4,083
Cost of goods sold - license and collaboration	—	277	—	3,703
Total cost of goods sold	$ 2,867	$ 1,585	$ 6,991	$ 7,786

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) in IgAN, the Company expensed the production of APIs purchased to support the commercial launch of FILSPARI, in research and development expenses. For the three and nine months ended September 30, 2025 sales of FILSPARI primarily consisted of zero-cost inventories. 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, 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, costs to 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 three Phase 3 clinical trials in process that are in varying stages of activity, with ongoing non-clinical support trials. As such, clinical trial expenses will vary depending on all the factors set forth above and may fluctuate significantly from quarter to quarter 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.

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 and RSUs using the treasury stock method. The treasury stock method reduces the number of shares issuable by the shares 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/(loss) per share calculation. 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 was 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, 2026 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.

Royalty expense includes amortization of royalties and milestones paid that are capitalized to the intangible asset under the asset accumulation model as well as royalties expensed after the estimated useful life of the license agreement has been reached.

The following table summarizes royalty expense for the three and nine months ended September 30, 2026 and 2025 (*in thousands*):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Amortization of intangibles - royalties and milestones	$ 4,802	$ 15,407	$ 33,458	$ 41,467
Royalties expensed	3,166	—	6,388	—
Total royalty expense	$ 7,968	$ 15,407	$ 39,846	$ 41,467
Royalties and milestones capitalized	$ 37,408	$ 19,239	$ 83,831	$ 46,372

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 Sheets 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.

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.

Recently Adopted Accounting Pronouncements

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, including interim periods within those years. The Company adopted this standard effective January 1, 2026. The adoption did not have a material impact on the Company's consolidated financial statements.

In November 2024, the FASB issued ASU No. 2024-04, Debt with Conversion and Other Options (Subtopic 470-20): Induced Conversions of Convertible Debt 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, including interim periods within those years. The Company adopted this standard effective January 1, 2026 on a prospective basis. See Note 10 for further discussion of accounting for convertible debt transactions during 2026.

In September 2025, the FASB issued ASU 2025-07, Derivatives and Hedging (Topic 815) and Revenue from Contracts with Customers (Topic 606): Derivatives Scope Refinements and Scope Clarification for Share-Based Noncash Consideration from a Customer in a Revenue Contract. The update provides a derivative scope refinement and scope clarification for share-based noncash consideration from a customer in a revenue contract. The amendments are effective for annual reporting periods beginning after December 15, 2026, including interim reporting periods within those annual periods. Early adoption is permitted. The Company early adopted this standard effective January 1, 2026 on a prospective basis. The adoption did not have a material impact on the Company's consolidated financial statements and related disclosures.

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 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 standard on the Company's accounting policies and the financial statements.

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.

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 99% of the Company's net product sales.

The Company sells FILSPARI to three 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, 2026 and 2025, adjustments to net product revenue related to performance obligations satisfied in previous periods were $1.9 million and $1.0 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, 2026 and 2025 (*in thousands*):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
FILSPARI	$ 200,192	$ 90,900	$ 446,422	$ 218,668
Tiopronin products	20,004	22,250	59,618	65,184
Total net product sales	$ 220,196	$ 113,150	$ 506,040	$ 283,852

NOTE 4. COLLABORATION AND LICENSE AGREEMENTS

License Agreement with CSL Vifor

In September 2021, the Company entered into the CSL Vifor License Agreement, 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 expand the license to cover additional countries (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.

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

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

The Company determined the initial transaction price under the CSL Vifor License Agreement totaled $55.0 million, consisting of the fixed non-refundable upfront payment. The variable regulatory and access related milestones were considered variable consideration and excluded from the transaction price given the substantial uncertainty at inception related to their achievement, based on the most likely amount method. Sales-based milestone payments and royalties on net sales were excluded from the transaction price and are 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 re-evaluates the transaction price for each reporting period and as uncertainties are resolved.

The Company concluded that CSL Vifor represented a customer and applied relevant guidance from ASC 606 to evaluate the accounting under the CSL Vifor License Agreement. In accordance with this guidance, the Company concluded at inception that the promise to grant the license is distinct from the promise to provide clinical development services resulting in two performance obligations. As a result, the Company allocated $12.0 million of the transaction price, based on the performance obligations' relative standalone selling prices, to the license, which was recognized in full in 2021. The remaining $43.0 million of the transaction price was allocated to the clinical development activities and recorded as deferred revenue, which was recognized over the development period based upon the ratio of costs incurred to date to the total estimated costs through June 30, 2025.

For the three months ended September 30, 2025, the Company recognized a market access milestone in the amount of $40.0 million. For the nine months ended September 30, 2025, the Company recognized a total of $57.5 million, including $17.5 million for the achievement of a regulatory milestone as a result of the European Commission granting standard MA approval. These milestones were included in the transaction price under the most likely amount method and recognized in full.

For the three months ended September 30, 2026 and 2025, the Company recognized $4.1 million and $42.4 million, respectively, in license and collaboration revenue associated with the CSL Vifor License Agreement. For the nine months ended September 30, 2026 and 2025, the Company recognized $10.0 million and $67.9 million, respectively, in license and collaboration revenue associated with the CSL Vifor License Agreement.

Licensing Agreement with Chugai

In January 2024, the Company entered into an exclusive licensing agreement ("Chugai License Agreement") with Renalys, pursuant to which, the Company granted an exclusive license to Renalys (which has since been merged with and into Chugai) for the development and commercialization of sparsentan in Japan and other specified Asian countries. Under the terms of the Chugai 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 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 Chugai License Agreement, Chugai is responsible for all development and commercialization activities in the licensed territories. The Company will retain all rights to sparsentan in the United States and rest of world outside of the licensed territories and the CSL Vifor Licensed Territories, provided that CSL Vifor has a right of negotiation to expand the licensed territories into Canada and/or Mexico. The Chugai License Agreement will remain in effect, unless terminated earlier, until the expiration of all royalty terms for sparsentan in the licensed territories. Each party has the right to terminate the Chugai License Agreement for the other party’s uncured material breach or insolvency, or if the time required for performance under the Chugai License Agreement by the other party is extended due to a force majeure event that continues for nine months or more. Chugai may terminate the Chugai License Agreement for any reason upon prior written notice to the Company. The Company may terminate the Chugai License Agreement, in whole or with respect to Japan or South Korea if Chugai abandons development of all licensed products in the applicable country (or both countries) prior to the first commercial sale.

The Company assessed the Chugai License Agreement and determined that it meets both criteria to be considered a collaborative agreement within the scope of ASC 808 which are 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 Chugai 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 under the most likely amount method. 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 Chugai represents a customer and applied relevant guidance from ASC 606 to evaluate the accounting under the Chugai 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 functionality at contract inception. The Buyout Right precluded 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 Chugai License Agreement as a financing arrangement. The transaction price was recorded in other current liabilities as a result of the Buyout Right. During the year ended December 31, 2025, the Buyout Right was relinquished, and the Company recognized the Renalys deferred revenue balance of $9.3 million in license and collaboration revenue.

See Note 6 for further discussion of VIEs.

For the three and nine months ended September 30, 2026, the Company recognized $0.3 million and $5.3 million, respectively, in license and collaboration revenue associated with the Chugai License Agreement, including a $5.0 million regulatory milestone. For the three and nine months ended September 30, 2025, the Company recognized $9.3 million in license and collaboration revenue associated with the Chugai License Agreement.

License Agreement with Everest

In June 2026, the Company entered into the Everest Agreement, granting the Company an exclusive license to develop, manufacture and commercialize civorebrutinib, an investigational oral, covalent reversible BTK inhibitor, and products containing civorebrutinib for any and all prophylactic, diagnostic and therapeutic uses and treatment of diseases and disorders in all countries outside of Greater China and certain countries in East and Southeast Asia (the "Territory"). The agreement became effective in July 2026 following the satisfaction of customary closing conditions.

Under the terms of the agreement, the Company paid Everest an upfront payment of $112.5 million in July 2026. Pursuant to the agreement, Everest is eligible to receive up to $1.0 billion in additional development, regulatory and commercial milestone payments, as well as tiered royalties ranging from high single-digit to double-digit percentages based on annual net sales of licensed products in the Territory. The Everest Agreement will remain in effect, unless terminated earlier, until the expiration of the royalty term for the licensed products in the Territory.

The Company and Everest have agreed to collaborate on global clinical development, including sharing development costs for global clinical trials. Except for certain global development activities, the Company is responsible for development and commercialization of civorebrutinib in the Territory, while Everest retains responsibility for its territory.

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

The Company determined that the acquisition constitutes an asset acquisition. The primary asset acquired is the in-process research and development (IPR&D) intangible asset related to civorebrutinib and its multiple indications, which collectively have no alternative future use. Accordingly, the upfront consideration and direct transaction costs totaling $113.1 million were expensed as IPR&D at Closing. Contingent milestone payments will be recognized when probable and reasonably estimable and developmental milestones prior to FDA approval will be expensed as IPR&D. Royalties and milestones after regulatory approval will be capitalized and amortized over their estimated useful life.

NOTE 5. MARKETABLE DEBT SECURITIES

The Company's marketable debt securities as of September 30, 2026 and December 31, 2025 were composed of available-for-sale commercial paper and corporate and government debt securities and municipal bonds. 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, 2026	December 31, 2025
Marketable debt securities:		
Commercial paper	$ 49,524	$ 62,988
Corporate debt securities	262,905	151,777
Securities of government sponsored entities	19,783	9,993
Municipal bonds	16,064	5,003
Total available-for-sale marketable debt securities	$ 348,276	$ 229,761

The following is a summary of short-term marketable debt securities classified as available-for-sale as of September 30, 2026 (*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	$ 49,618	$ —	$ (94)	$ 49,524
Corporate debt securities	Less than 1	110,932	2	(338)	110,596
Securities of government-sponsored entities	Less than 1	10,000	—	(64)	9,936
Municipal bonds	Less than 1	9,827	—	(50)	9,777
Total maturity less than 1 year		180,377	2	(546)	179,833
Corporate debt securities	1 to 2	153,838	—	(1,529)	152,309
Securities of government-sponsored entities	1 to 2	9,944	—	(97)	9,847
Municipal bonds	1 to 2	6,355	—	(68)	6,287
Total maturity 1 to 2 years		170,137	—	(1,694)	168,443
Total available-for-sale marketable debt securities		$ 350,514	$ 2	$ (2,240)	$ 348,276

The following is a summary of short-term marketable debt securities classified as available-for-sale as of December 31, 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	$ 62,974	$ 20	$ (6)	$ 62,988
Corporate debt securities	Less than 1	77,414	12	(33)	77,393
Municipal bonds	Less than 1	5,000	3	—	5,003
Total maturity less than 1 year		145,388	35	(39)	145,384
Corporate debt securities	1 to 2	74,389	69	(74)	74,384
Securities of government-sponsored entities	1 to 2	10,000	—	(7)	9,993
Total maturity 1 to 2 years		84,389	69	(81)	84,377
Total available-for-sale marketable debt securities		$ 229,777	$ 104	$ (120)	$ 229,761

For the three and nine months ended September 30, 2026 and 2025, realized gains and losses on marketable debt securities were immaterial. As of September 30, 2026 and December 31, 2025, the accrued interest receivable related to the Company's marketable debt securities was $2.8 million and $1.3 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 aggregated by investment category and length of time those individual securities have been in a continuous unrealized loss position reported as of September 30, 2026 (*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	$ 49,524	$ 94	$ —	$ —	$ 49,524	$ 94
Corporate debt securities	259,896	1,867	—	—	259,896	1,867
Securities of government-sponsored entities	19,783	161	—	—	19,783	161
Municipal bonds	16,064	118	—	—	16,064	118
Total	$ 345,267	$ 2,240	$ —	$ —	$ 345,267	$ 2,240

The following is a summary of available-for-sale marketable debt securities in an unrealized loss position with no credit losses aggregated by investment category and length of time those individual securities have been in a continuous unrealized loss position reported as of December 31, 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
Commercial paper	$ 12,317	$ 6	$ —	$ —	$ 12,317	$ 6
Corporate debt securities	118,443	107	—	—	118,443	107
Securities of government-sponsored entities	9,993	7	—	—	9,993	7
Total	$ 140,753	$ 120	$ —	$ —	$ 140,753	$ 120

As of September 30, 2026 and December 31, 2025, the amortized cost of the available-for-sale marketable debt securities in an unrealized loss position was $347.5 million and $140.9 million, respectively.

As of September 30, 2026 and December 31, 2025, the Company does not intend to sell these investments and it is not more likely than not that the Company will be required to sell the investments before recovery of their amortized cost basis. The increase in unrealized losses for the nine months ended September 30, 2026 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

Licensing Agreement with Chugai

In January 2024, the Chugai License Agreement between the Company and Renalys (which has since been acquired by and merged into Chugai) came into effect and the Company exercised its option to purchase shares of common stock of Renalys. The Company determined that Renalys was a VIE as they could require additional funding to support development and commercial activities. The Company had variable interests in Renalys, including an equity interest, Buyout Right and performance-related payments under the Chugai 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 was not the primary beneficiary as it did not have the power to direct the activities that most significantly impact the economic performance of Renalys and did not have an obligation to absorb losses.

During the year ended December 31, 2025, the Buyout Right was relinquished and the Company recognized the deferred revenue balance of $9.3 million in license and collaboration revenue. In the fourth quarter of 2025, Renalys was acquired by and merged into Chugai. The Company sold its equity interest in Renalys to Chugai as part of the acquisition.

NOTE 7. LEASES

As of September 30, 2026, 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 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, 2026 (*in thousands*):

	September 30, 2026
2026 (remaining)	$ 1,703
2027	6,978
2028	4,782
2029 and thereafter	—
Total undiscounted future minimum payments	13,463
Present value discount	(818)
Total lease liability	12,645
Unamortized lease incentives	(1,825)
Cash payments in excess of straight-line lease expense	(3,013)
Total right-of-use asset	$ 7,807

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

	September 30, 2026	December 31, 2025
Weighted-average remaining lease term in years	1.9	2.7
Weighted-average discount rate	6.48 %	6.48 %

For the three and nine months ended September 30, 2026, the Company recorded $1.1 million and $3.3 million in expense related to operating leases, including amortized tenant improvement allowances. For the three and nine months ended September 30, 2025, the Company recorded $1.1 million and $3.3 million in expense related to operating leases, including amortized tenant improvement allowances.

NOTE 8. FAIR VALUE MEASUREMENTS

The Company utilizes a fair value hierarchy that prioritizes the inputs to valuation techniques used to measure fair value. The hierarchy gives the highest priority to unadjusted quoted prices in active markets for identical assets or liabilities (Level 1 measurements) and the lowest priority to unobservable inputs (Level 3 measurements). The three levels of the fair value hierarchy are described below:

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

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

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

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

Financial instruments with carrying values approximating fair value include cash and cash equivalents, accounts receivable, and accounts payable, due to their short-term nature. As of September 30, 2026 and December 31, 2025, the fair value of the Company's 2.25% Convertible Senior Notes due 2029 was $184.1 million and $448.3 million, respectively. As of September 30, 2026 the fair value of the Company's 0.50% Convertible Senior Notes due 2032 was $619.2 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, 2026 (*in thousands*):

	As of September 30, 2026			
	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	$ 123,542	$ 123,542	$ —	$ —
Marketable debt securities, available-for-sale	348,276	—	348,276	—
Total	$ 471,818	$ 123,542	$ 348,276	$ —

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, 2025 (*in thousands*):

	As of December 31, 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	$ 93,035	$ 93,035	$ —	$ —
Marketable debt securities, available-for-sale	229,761	—	229,761	—
Total	$ 322,796	$ 93,035	$ 229,761	$ —

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, 2026 the Company has capitalized $48.7 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) 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 IgAN. For the three and nine months ended September 30, 2026, the Company capitalized royalties owed on net sales of FILSPARI to intangible assets of $37.4 million and $80.8 million, respectively and $15.6 million and $35.9 million for the three and nine months ended September 30, 2025, respectively. The cost of historical milestones paid and royalty payments are being amortized to royalty expense on a straight-line basis through the estimated useful life.

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 capitalized a total of $188.4 million related to the Thiola intangible asset. The cost of the milestone payments and royalty payments were fully amortized to royalty expense on a straight-line basis through March 31, 2026. During the second quarter of 2026, the Company began expensing royalty payments during the period incurred.

The following table sets forth amortizable intangible assets as of September 30, 2026 and December 31, 2025 (*in thousands*):

	September 30, 2026	December 31, 2025
Finite-lived intangible assets	$ 377,121	$ 293,467
Less: accumulated amortization	(213,766)	(180,352)
Net carrying value	$ 163,355	$ 113,115

Amortization expense for the three and nine months ended September 30, 2026 was $4.8 million and $33.5 million, respectively and $15.4 million and $41.5 million, for the three and nine months ended September 30, 2025, respectively, and is recorded in royalty expense.

NOTE 10. CONVERTIBLE DEBT

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

	September 30, 2026	December 31, 2025
0.50% convertible senior notes due 2032	$ 525,000	$ —
2.25% convertible senior notes due 2029	94,875	316,250
Unamortized debt issuance costs - 0.50% convertible senior notes due 2032	(15,328)	—
Unamortized debt issuance costs - 2.25% convertible senior notes due 2029	(1,037)	(4,526)
Total convertible senior notes, net of unamortized debt issuance costs	$ 603,510	$ 311,724

Convertible Senior Notes Due 2032

On May 11, 2026, the Company completed a registered underwritten public offering of $525.0 million aggregate principal amount of 0.50% Convertible Senior Notes due 2032 ("2032 Notes"), which includes $50.0 million aggregate principal amount of 2032 Notes sold pursuant to the full exercise of the underwriters' over-allotment option. The Company issued the 2032 Notes under an indenture, dated as of May 11, 2026, as supplemented by the first supplemental indenture, dated as of May 11, 2026 (collectively, the "2032 Indenture"). The Company received net proceeds from the issuance of the 2032 Notes of $509.3 million, after deducting commissions and offering expenses of $16.3 million. The 2032 Notes will mature on May 15, 2032, unless earlier repurchased, redeemed, or converted. The 2032 Notes are senior unsecured obligations of the Company and bear interest at an annual rate of 0.50%, payable semi-annually in arrears on May 15 and November 15 of each year, beginning on November 15, 2026.

At September 30, 2026, accrued interest on the 2032 Notes of $1.0 million is included in accrued expenses in the accompanying Consolidated Balance Sheets. The 2032 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, including the 2029 Notes; (ii) senior in right of payment to the Company's existing and future indebtedness that is expressly subordinated to the 2032 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, of the Company's subsidiaries.

Holders may convert their 2032 Notes at their option only in the following circumstances: (1) during any calendar quarter commencing after the calendar quarter ending on September 30, 2026 (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 2032 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 2032 Notes for redemption; and (5) at any time from, and including, February 17, 2032 until the close of business on the second 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 2032 Notes is 15.4078 shares of the Company's common stock per $1,000 principal amount of 2032 Notes, which represents an initial conversion price of approximately $64.90 per share. If a "make-whole fundamental change" (as defined in the 2032 Indenture) occurs, then the Company will, in certain circumstances, increase the conversion rate for a specified period of time.

The 2032 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 May 21, 2029 and, in the case of any partial redemption, on or before the 30th scheduled trading day before the maturity date, at a cash redemption price equal to the principal amount of the 2032 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 2032 Notes unless at least $75.0 million aggregate principal amount of 2032 Notes are outstanding and not called for redemption as of the time the Company sends the related redemption notice. In addition, calling any 2032 Note for redemption will constitute a make-whole fundamental change with respect to that 2032 Note, in which case the conversion rate applicable to the conversion of that 2032 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 2032 Indenture) occurs, then, except as described in the 2032 Indenture, holders may require the Company to repurchase their 2032 Notes at a cash repurchase price equal to the principal amount of the 2032 Notes to be repurchased, plus accrued and unpaid interest, if any, to, but excluding, the fundamental change repurchase date.

The Company incurred approximately $16.3 million of debt issuance costs relating to the issuance of the 2032 Notes, which were recorded as a reduction to the 2032 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 2032 Notes using the effective interest method. The Company determined the expected life of the debt is equal to the six-year term of the 2032 Notes. The effective interest rate on the 2032 Notes is 1.0%.

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 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. 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.

At September 30, 2026, accrued interest on the 2029 Notes of $0.2 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, including the 2032 Notes; (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, of the Company's subsidiaries.

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.

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%.

On May 11, 2026, the Company completed its repurchase of $221.4 million aggregate principal amount of 2029 Notes for cash, including accrued and unpaid interest, for a total of $350.9 million. The Company evaluated the transaction in accordance with amended accounting guidance under ASU 2024-04 and accounted for the repurchase as an induced conversion. As a result of the repurchase, the Company recognized inducement expense of $40.0 million, which was recorded as other expense in the Consolidated Statements of Operations for the nine months ended September 30, 2026. The inducement expense primarily represents the difference between the if-converted value of the 2029 Notes and the fair value of the 2029 Notes at the time that the debt repurchase terms were accepted by noteholders. The remaining $91.3 million of the premium, which represents the difference between the if-converted value and carrying value of the repurchased 2029 Notes and $2.8 million unamortized debt issuance costs related to the repurchased 2029 Notes were accounted for as reductions to additional paid-in capital in the Consolidated Balance Sheets. After giving effect to the repurchase, the total remaining principal amount outstanding under the 2029 Notes was $94.9 million.

The 2029 Notes and 2032 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 and 2032 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 and 2032 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 or 2032 Notes at September 30, 2026.

The 2029 Notes and 2032 Notes are classified as long-term convertible debt on the Company's Consolidated Balance Sheets at September 30, 2026.

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.

Interest Expense

The following table sets forth total interest expense recognized related to the 2025, 2029 and 2032 Notes (*in thousands*):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Contractual interest expense	$ 1,190	$ 2,138	$ 4,407	$ 6,557
Amortization of debt issuance costs	808	420	1,703	1,286
Total interest expense	$ 1,998	$ 2,558	$ 6,110	$ 7,843

Total interest expense recognized for the three and nine months ended September 30, 2026 was $2.1 million and $6.6 million, respectively, and $2.8 million and $8.5 million for the three and nine months ended September 30, 2025, respectively.

NOTE 11. ACCRUED EXPENSES

Accrued expenses at September 30, 2026 and December 31, 2025 consisted of the following (*in thousands*):

	September 30, 2026	December 31, 2025
Sales discounts, rebates, and allowances	$ 68,038	$ 30,595
Accrued royalties	41,099	22,703
Compensation related costs	33,928	41,515
Research and development	22,097	16,027
Selling, general and administrative	14,025	9,818
Miscellaneous accrued expenses	8,029	5,377
Total accrued expenses	$ 187,216	$ 126,035

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.

The Company's potentially dilutive shares, which include outstanding stock options, restricted stock units, and shares issuable upon conversion of the 2025 Notes, repaid in September 2025, 2029 Notes and 2032 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 loss per share is calculated as follows (*in thousands, except share and per share amounts*):

	Three Months Ended			Nine Months Ended		
	September 30, 2026					
	Shares	Net Loss	EPS	Shares	Net Loss	EPS
Continuing operations	94,627,788	$ (61,037)	$ (0.64)	93,335,461	$ (132,436)	$ (1.42)
Discontinued operations	94,627,788	$ 8,700	$ 0.09	93,335,461	$ 8,200	$ 0.09
Basic and diluted loss per share	94,627,788	$ (52,337)	$ (0.55)	93,335,461	$ (124,236)	$ (1.33)

	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)

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,	
	2026	2025	2026	2025
Convertible debt	11,065,704	—	10,521,096	11,593,869
Options	7,577,397	5,948,756	8,296,057	9,947,928
Restricted stock units	5,346,596	55,566	5,440,841	4,368,415
Total anti-dilutive shares	23,989,697	6,004,322	24,257,994	25,910,212

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 "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. Through September 30, 2026, the Company has paid $65.2 million for contractual milestones achieved under the stock purchase agreement.

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. As of September 30, 2026, no contingent cash 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, 2026:

	Shares Underlying Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2025	8,241,429	$ 19.91	5.89	$ 150,796
Granted	1,371,230	$ 34.26		
Exercised	(2,933,914)	$ 20.61		$ 90,724
Forfeited/canceled	(103,494)	$ 22.41		
Outstanding at September 30, 2026	6,575,251	$ 22.56	6.88	$ 238,842
Vested and expected to vest at September 30, 2026	6,575,251	$ 22.56	6.88	$ 238,842
Exercisable at September 30, 2026	4,011,269	$ 20.37	5.74	$ 154,459

As of September 30, 2026, excluding amounts associated with the CEO transition discussed below, unrecognized share-based compensation for stock options was $28.8 million, which is expected to be recognized over a remaining weighted-average period of 2.6 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, 2026:

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Unvested at December 31, 2025	4,567,367	$ 18.02
Granted	2,447,920	$ 34.98
Vested	(1,508,401)	$ 18.43
Forfeited/canceled	(254,245)	$ 22.95
Unvested at September 30, 2026	5,252,641	$ 25.57

As of September 30, 2026, excluding amounts associated with the CEO transition discussed below, unrecognized share-based compensation for service based restricted stock units was $95.6 million, which is expected to be recognized over a remaining weighted-average period of 2.4 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, 2026:

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Unvested at December 31, 2025	233,604	$ 16.35
Granted	215,730	$ 24.94
Vested	(229,500)	17.77
Forfeited/canceled	—	—
Unvested at September 30, 2026	219,834	$ 23.30

As of September 30, 2026, excluding amounts associated with the CEO transition discussed below, unrecognized share-based compensation for performance based restricted stock units was $0.9 million, which is expected to be recognized over a weighted-average period of 1.1 years.

Share-Based Compensation

The following table sets forth share-based compensation for continuing operations for the three and nine months ended September 30, 2026 and 2025 (*in thousands*):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Research and development	$ 7,121	$ 4,101	$ 19,553	$ 12,817
Selling, general and administrative	16,316	7,162	41,819	20,587
Total share-based compensation	$ 23,437	$ 11,263	$ 61,372	$ 33,404

During the three months ended September 30, 2026 the Company announced the planned transition of its President and Chief Executive Officer, Dr. Eric Dube. In connection with his planned transition, the Company and Dr. Dube entered into a Transition and Separation Agreement addressing, among other matters, transition services to be provided by Dr. Dube following the CEO transition. Certain terms of the agreement resulted in modification accounting of the stock options and restricted stock units held by Dr. Dube. These modifications resulted in incremental stock-based compensation expense of $31.3 million, which is being recognized over Dr. Dube's remaining CEO service period from September 21, 2026 to December 1, 2026. The Company recognized $4.4 million during the three and nine months ended September 30, 2026 and expects to recognize the remaining $26.9 million during the fourth quarter of 2026.

NOTE 15. INVENTORY

Inventory consisted of the following at September 30, 2026 and December 31, 2025 (*in thousands*):

	September 30, 2026	December 31, 2025
Raw materials	$ 18,766	$ 24,169
Work in process	12,377	2,348
Finished goods	12,660	9,638
Total inventory	$ 43,803	$ 36,155
Classified as:		
Current inventory	$ 10,516	$ 5,875
Long-term inventory	33,287	30,280
Total inventory	$ 43,803	$ 36,155

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, 2026 and December 31, 2025. 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 $107.5 million and $80.1 million at September 30, 2026 and December 31, 2025, respectively. The accounts receivable balance as of December 31, 2025 includes the $25.0 million milestone payment due from Mirum Pharmaceuticals, Inc. (“Mirum”), received in April 2026. The accounts receivable balance as of September 30, 2026 includes the additional $10.0 million milestone payment due from Mirum. See Note 18 for further discussion. 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. As of September 30, 2026, 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 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. Mirum achieved the first such milestone based on its annual net sales in 2025, and Travere recognized $25.0 million for the year ended December 31, 2025 in discontinued operations. The $25.0 million is included in accounts receivable as of December 31, 2025 and payment was received in April 2026. Mirum achieved an additional milestone based on its annual net sales in 2026, and Travere recognized $10.0 million in milestone revenue for the three and nine months ended September 30, 2026 in discontinued operations. The $10.0 million is included in accounts receivable as of September 30, 2026. Income from discontinued operations, net of tax for the three and nine months ended September 30, 2026 was $8.7 million and $8.2 million, respectively and zero for both the three and nine months ended September 30, 2025.

The Company corrected an immaterial presentation error from a prior period in its consolidated statement of cash flows for the nine months ended September 30, 2026. The previously presented cash flows for the six months ended June 30, 2026 incorrectly included cash flows of $24.5 million in cash provided by operating activities - continuing operations and should have been reflected in cash provided by operating activities - discontinued operations. The correction would have resulted in cash used in operating activities - continuing operations of $6.8 million and cash provided by operating activities - discontinued operations of $24.5 million for the six months ended June 30, 2026. The correction does not affect total cash flows from operating activities or the reported net change in cash and cash equivalents. The Company evaluated the materiality of the error in its statement of cash flows, including its effect on the previously issued consolidated financial statements and the financial statements taken as a whole, and concluded that the error was not material to the previously issued consolidated financial statements. Accordingly, the Company has corrected the presentation of the cash flows during the nine months ended September 30, 2026.

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.

Long-lived assets located outside the U.S. were immaterial as of both September 30, 2026 and December 31, 2025. 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.

See Note 3 for further discussion of net product sales and Note 4 for discussion of license and collaboration revenues.

The following table presents reportable segment (loss) income, including significant expenses regularly provided to the CODM, attributable to the Company's reportable segment for the three and nine months ended September 30, 2026 and 2025 (*in thousands*):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue	$ 224,563	$ 164,859	$ 521,345	$ 361,039
Less:				
Cost of goods sold	2,867	1,585	6,991	7,786
Research and development:				
External research and development	38,646	27,837	97,589	77,653
Internal personnel costs	25,731	18,959	74,217	57,276
Other research and development	5,927	5,094	15,874	13,212
Total research and development	70,304	51,890	187,680	148,141
Selling, general and administrative	98,414	71,046	274,779	194,041
Royalty expense	7,968	15,407	39,846	41,467
In-process research and development	113,115	—	113,115	—
Total other income (expense), net	1,173	783	(37,590)	2,188
Income tax benefit (provision)	5,895	(8)	6,220	(67)
Income from discontinued operations, net of tax	8,700	—	8,200	—
Net (loss) income	$ (52,337)	$ 25,706	$ (124,236)	$ (28,275)

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, 2025 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, 2025, filed with the Securities and Exchange Commission ("SEC") on February 19, 2026. 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.

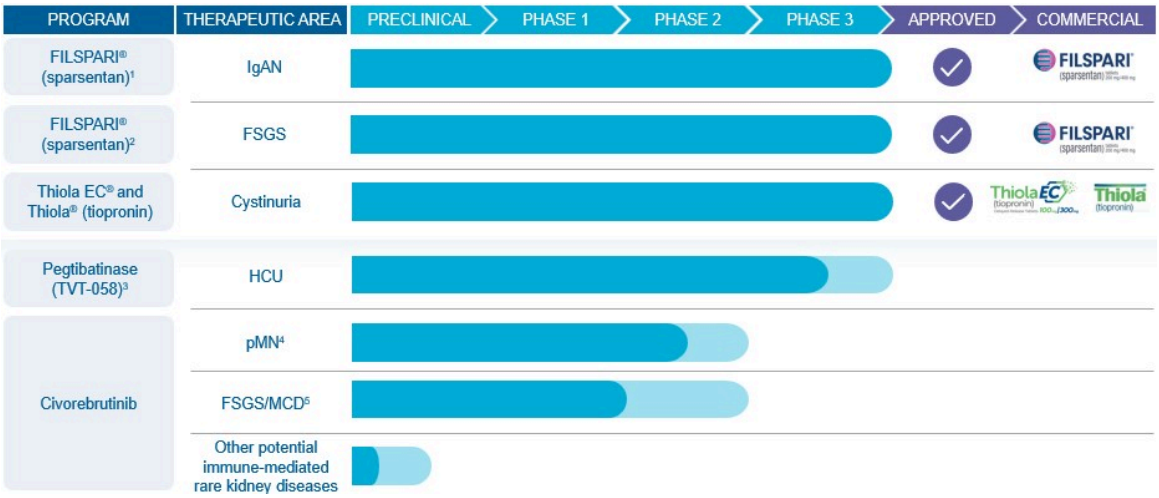
Overview

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 Immunoglobulin A nephropathy ("IgAN") who are at risk of disease progression. FILSPARI had previously been granted accelerated approval in February 2023.

2 On April 13, 2026, the FDA granted full (traditional) approval of FILSPARI to reduce proteinuria in adult and pediatric patients aged 8 years and older with focal segmental glomerulosclerosis ("FSGS") without nephrotic syndrome.

3 Following a voluntary pause in enrollment, we restarted enrollment activities for the pivotal Phase 3 HARMONY Study in the first quarter of 2026.

4 Current Everest Medicines studies.

5 As part of the ongoing basket study conducted by Everest Medicines.

FILSPARI® (sparsentan) for the treatment of IgAN

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 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. 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 had 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 was 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 CSL Vifor License Agreement. 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 the fourth quarter of 2025, we received a $40.0 million market access milestone payment from CSL Vifor.

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. 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 the fourth quarter of 2025, Renalys announced positive topline results from its Phase 3 study of sparsentan in Japanese patients with IgAN. Renalys has also announced that it has reached an agreement with the Pharmaceuticals and Medical Devices Agency ("PMDA") regarding development plans for two other Phase 3 clinical trials of sparsentan, one investigating the use of sparsentan in FSGS and the other in Alport syndrome, in Japan. In the fourth quarter of 2025, Renalys was acquired by and merged into Chugai Pharmaceutical Co., Ltd. (“Chugai”). Through the acquisition, Chugai gained exclusive rights to develop and commercialize sparsentan in Japan, South Korea, and Taiwan. As a minority shareholder in Renalys, we received $10.2 million at the closing of the transaction and we are also eligible to receive multiple milestones according to the progress of sparsentan regulatory approval, and consideration linked to sparsentan's net sales in the applicable territory. Under the terms of the licensing agreement, Chugai is responsible for development, regulatory matters, and commercialization in the licensed territories.

FILSPARI (sparsentan) for the treatment of FSGS

On April 13, 2026, the FDA granted full (traditional) approval of FILSPARI (sparsentan) to reduce proteinuria in adult and pediatric patients aged 8 years and older with FSGS without nephrotic syndrome.

FILSPARI is the first and only medicine approved by the FDA for the treatment of FSGS.

FSGS is a leading cause of kidney failure and nephrotic syndrome. Prior to the approval of FILSPARI in April 2026, there were 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.

People with FSGS who do not have nephrotic syndrome span across types of FSGS and represent a population aligned with the KDIGO guidelines for treating glomerular diseases. Nephrotic syndrome is commonly defined as the presence of three concurrent criteria: proteinuria greater than 3.5 g/24h, edema, and albumin less than 3.0 g/dL. We estimate that the addressable population in the U.S. is more than 30,000 individuals with FSGS who do not have nephrotic syndrome.

In the Phase 3 DUPLEX Study, the largest head-to-head interventional study in FSGS to date, patients treated with FILSPARI in the overall study population experienced a statistically significant 46% reduction in proteinuria from baseline to Week 108 compared to 30% for those treated with maximum labeled dose irbesartan (nominal p-value, 0.0299). In patients without nephrotic syndrome, FILSPARI demonstrated even greater improvements compared to maximum labeled dose irbesartan across proteinuria and eGFR. Those without nephrotic syndrome who were treated with FILSPARI experienced a 48% reduction in proteinuria from baseline to Week 108 compared to 27% for those treated with irbesartan, which was statistically significant (nominal p-value, 0.0075). FILSPARI-treated patients without nephrotic syndrome also demonstrated a benefit in eGFR with a treatment difference of 1.1 mL/min/1.73 m2 based on mean change from baseline to Week 108 (-11.3 mL/min/1.73 m2 for FILSPARI compared to -12.4 mL/min/1.73 m2 for maximum labeled dose irbesartan). Across both adult and pediatric patients, FILSPARI was generally well tolerated, with a safety profile comparable to irbesartan and consistent across clinical programs.

The Phase 3 DUPLEX Study was a global, randomized, multicenter, double-blind, parallel-arm, active-controlled Phase 3 clinical trial that assessed the efficacy and safety of FILSPARI in 371 patients ages 8 to 75 years with biopsy-proven or genetic FSGS. After a two-week washout period, patients were randomized 1:1 to receive either FILSPARI or irbesartan, the active control, and subsequently dose titrated to the maximum dose of 800 mg of sparsentan or 300 mg of irbesartan, as tolerated. In the study, nephrotic syndrome was defined as (a) documentation of nephrotic syndrome in the medical history, or (b) the concurrent presence of proteinuria >3.5 g/24 hours (adults) or UPCR >2.0 g/g (pediatric patients <18 years of age), serum albumin <3.0 g/dL, and edema at baseline. The primary efficacy endpoint at the final analysis was the rate of change in eGFR from baseline to Week 108. The two-year results from the study were published in the *New England Journal of Medicine*. Patients who completed the DUPLEX double-blind portion of the study on treatment were eligible to participate in the open-label extension of the trial.

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

As noted above in more detail, FILSPARI is available only through a REMS approved by the FDA for liver monitoring regarding potential risk of hepatotoxicity, as has been required for certain other approved endothelin antagonists.

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

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 Chugai, Chugai 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 Chugai, provided that CSL Vifor has a right of negotiation to expand the licensed territories into Canada and/or Mexico.

Clinical-Stage Programs:

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 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 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 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 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 was enacted following our determination that the desired drug substance profile was not achieved in the initial scale-up process, and it enabled us to address necessary process improvements in manufacturing scale-up to support initial commercial scale manufacturing as well as full enrollment in the HARMONY Study. Following successful process improvements, the Company restarted enrollment activities in the first quarter of 2026 and dosed the first new patient following study restart in April 2026, with topline data anticipated in the second half of 2027.

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

Civorebrutinib

Civorebrutinib is an investigational oral, covalent reversible Bruton's tyrosine kinase ("BTK") inhibitor designed to provide differentiated efficacy, safety and convenience for patients with rare, immune-mediated kidney diseases, with planned evaluation in primary membranous nephropathy ("PMN"), FSGS, minimal change disease ("MCD") and potentially additional indications. BTK is a key mediator of B-cell receptor signaling and plays an important role in B-cell activation, maturation, proliferation, and differentiation into antibody-producing cells.

In immune-mediated kidney diseases, B-cell activation and autoantibody production are believed to contribute directly to kidney injury. Civorebrutinib has demonstrated proof of concept in a Phase 1/2 clinical trial of patients with PMN. The previously reported Phase 1/2 data demonstrated rapid and sustained reductions in anti-PLA2R autoantibodies and proteinuria, with high rates of immunologic and clinical remission and stable kidney function through 52 weeks of follow-up. Civorebrutinib has been generally well tolerated throughout the development program to date.

In June 2026, we entered into a license and collaboration agreement (the "Everest Agreement") with Everest Medicines (Singapore) Pte. Ltd. ("Everest"), pursuant to which we obtained an exclusive license to develop, manufacture and commercialize civorebrutinib in the United States and global markets excluding Greater China and certain countries in East and Southeast Asia (the "Territory"). The Everest Agreement became effective in July 2026 following the satisfaction of customary closing conditions.

We and Everest are collaborating on global clinical development, including sharing development costs for global clinical trials. Except for certain global development activities, we are responsible for development and commercialization of civorebrutinib in the Territory, while Everest retains responsibility for its territory.

Under the Everest Agreement, we paid Everest an upfront payment of $112.5 million in July 2026 and Everest is eligible to receive up to approximately $1.0 billion in additional development, regulatory and commercial milestone payments, as well as tiered royalties ranging from high single-digit to double-digit percentages on net sales of civorebrutinib products in the Territory.

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, 2026 compared to the three and nine months ended September 30, 2025

Revenue

The following table provides information regarding revenue, including net product sales and license and collaboration revenue (*in thousands*):

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2026	2025	Change	2026	2025	Change
FILSPARI	$ 200,192	$ 90,900	$ 109,292	$ 446,422	$ 218,668	$ 227,754
Tiopronin products	20,004	22,250	(2,246)	59,618	65,184	(5,566)
Total net product sales	220,196	113,150	107,046	506,040	283,852	222,188
License and collaboration revenue	4,367	51,709	(47,342)	15,305	77,187	(61,882)
Total revenue	$ 224,563	$ 164,859	$ 59,704	$ 521,345	$ 361,039	$ 160,306

Net product sales

The increase in total net product sales for the three and nine months ended September 30, 2026 compared to the three and nine months ended September 30, 2025 was primarily due to growth in sales of FILSPARI.

License and collaboration revenue

The decrease in license and collaboration revenue for the three months ended September 30, 2026 compared to the three months ended September 30, 2025 was primarily due to recognition of a $40.0 million market access milestone associated with the CSL Vifor License Agreement and license revenue of $9.3 million recognized upon the relinquishment of the Buyout Right in the Chugai License Agreement during the three months ended September 30, 2025. The decrease in license and collaboration revenue for the nine months ended September 30, 2026 compared to the nine months ended September 30, 2025 was primarily due to recognition of $57.5 million in milestones associated with the CSL Vifor License Agreement, license revenue of $9.3 million recognized upon the relinquishment of the Buyout Right in the Chugai License Agreement and the sale of $3.8 million in active pharmaceutical ingredients ("APIs") during the nine months ended September 30, 2025, offset by recognition of a $5.0 million regulatory milestone associated with the Chugai License Agreement during the nine months ended September 30, 2026.

Operating Expenses

We revised the presentation of amortization expense of certain royalty and milestone payments associated with license agreements. Historically, these royalties were included within selling, general and administrative expenses in the consolidated statements of operations. Beginning in the current year, we present these amounts separately as royalty expense to better reflect the nature of these costs as consideration payable to licensors based on sales of licensed products and regulatory milestone payments.

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2026	2025	Change	2026	2025	Change
Cost of goods sold - product sales	$ 2,867	$ 1,308	$ 1,559	$ 6,991	$ 4,083	$ 2,908
Cost of goods sold - license and collaboration	—	277	(277)	—	3,703	(3,703)
Total cost of goods sold	2,867	1,585	1,282	6,991	7,786	(795)
Research and development	70,304	51,890	18,414	187,680	148,141	39,539
Selling, general and administrative	98,414	71,046	27,368	274,779	194,041	80,738
Royalty expense	7,968	15,407	(7,439)	39,846	41,467	(1,621)
In-process research and development	113,115	—	113,115	113,115	—	113,115
Total operating expenses	$ 292,668	$ 139,928	$ 152,740	$ 622,411	$ 391,435	$ 230,976

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 APIs to our collaboration partners, at cost or at cost plus a margin.

Prior to the February 2023 FDA accelerated approval of FILSPARI (sparsentan) in IgAN, we expensed the production of APIs purchased to support the commercial launch of FILSPARI, in research and development expenses. For the three and nine months ended September 30, 2025 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. We began capitalizing inventory costs associated with FILSPARI following the February 2023 accelerated approval.

For the nine months ended September 30, 2026 compared to the nine months ended September 30, 2025, our cost of goods sold - license and collaboration decreased by $3.7 million, due to the sale of API in 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, costs to 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 three 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,		
	2026	2025	Change	2026	2025	Change
External service provider costs:						
Sparsentan	$ 9,728	$ 11,608	$ (1,880)	$ 26,198	$ 36,664	$ (10,466)
Pegtibatinase	28,918	16,158	12,760	71,391	40,477	30,914
General and other product candidates	5,927	5,165	762	15,874	13,724	2,150
Total external service provider costs	44,573	32,931	11,642	113,463	90,865	22,598
Internal personnel costs	25,731	18,959	6,772	74,217	57,276	16,941
Total research and development	$ 70,304	$ 51,890	$ 18,414	$ 187,680	$ 148,141	$ 39,539

For the three and nine months ended September 30, 2026 compared to 2025, our research and development expenses increased by $18.4 million and $39.5 million, respectively. External service provider costs increased by $11.6 million and $22.6 million, respectively, for the three and nine months ended September 30, 2026 compared to 2025, primarily driven by an increase in costs associated with pegtibatinase due to restarting enrollment activities for the HARMONY Study in 2026, as well as additional manufacturing costs, offset by a decrease in costs associated with the development of sparsentan as our Phase 3 programs advanced towards completion. The increase in internal personnel costs of $6.8 million and $16.9 million, respectively, for the three and nine months ended September 30, 2026 compared to 2025, is primarily due to an increase in headcount to support additional pipeline development.

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, 2026 compared to 2025, our selling, general and administrative expenses increased by $27.4 million and $80.7 million, respectively, primarily as a result of an increase in commercial investments to support FILSPARI in IgAN and the ongoing launch of FSGS, including an expansion of our field force to support both indications.

Royalty expense

Royalty expense includes amortization of royalties and milestones paid that are capitalized to the intangible asset under the asset accumulation model as well as royalties expensed after the estimated useful life of the license agreement has been reached.

For the three months ended September 30, 2026 compared to 2025, our royalty expense decreased by $7.4 million due to the Thiola intangible asset reaching the end of its estimated useful life on March 31, 2026. For the nine months ended September 30, 2026 compared to 2025, royalty expense decreased by $1.6 million, primarily due to elevated amortization of the Thiola intangible asset in the first quarter of 2026 as it reached the end of its estimated useful life on March 31, 2026, with no further amortization after this date as royalties are expensed in the period incurred.

In-process research and development expense

In July 2026, we recognized $113.1 million of in-process research and development (IPR&D) expense, consisting of upfront consideration and direct transaction costs related to the acquisition of the civorebrutinib IPR&D intangible asset from Everest.

Other Income/Expenses

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2026	2025	Change	2026	2025	Change
Interest income	$ 3,704	$ 3,047	$ 657	$ 9,643	$ 10,129	$ (486)
Interest expense	(2,140)	(2,751)	611	(6,575)	(8,452)	1,877
Other (expense) income, net	(391)	487	(878)	(650)	511	(1,161)
Inducement expense	—	—	—	(40,008)	—	(40,008)
Total other income (expense), net	$ 1,173	$ 783	$ 390	$ (37,590)	$ 2,188	$ (39,778)

The change in our total other income (expense), net for the nine months ended September 30, 2026 compared to 2025 primarily reflects a $40.0 million inducement expense related to the partial repurchase of the 2.25% Convertible Senior Notes due 2029 ("2029 Notes") during the nine months ended September 30, 2026. This expense was partially offset by a decrease in interest expense following the September 2025 maturity of our 2.50% Convertible Senior Notes due 2025, the partial repurchase of the 2029 Notes and issuance of 0.50% Convertible Senior Notes due 2032 ("2032 Notes") at a lower interest rate.

Income Taxes

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2026	2025	Change	2026	2025	Change
Income tax benefit (provision)	$ 5,895	$ (8)	$ 5,903	$ 6,220	$ (67)	$ 6,287

The Company's income tax provision is calculated using an estimated annual effective tax rate, which may fluctuate based on changes in forecasted annual pretax income or loss. The change from income tax expense in the prior-year periods to an income tax benefit for the three and nine months ended September 30, 2026, primarily reflects changes in the estimated annual effective tax rate resulting from differences in projected annual operating results.

The Company's effective tax rate differs from the federal statutory rate primarily due to the utilization of net operating loss carryforwards for which a valuation allowance had previously been recognized. In addition, the Company expects an increase in state taxes related to jurisdictions where the Company expects to generate current taxable income with limited or no remaining net operating loss carryforwards. The Company continues to maintain a full valuation allowance against its U.S. deferred tax assets due to its history of cumulative losses.

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 commercial launch of FILSPARI in the United States for FSGS.

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 and 2032 Notes, on or before maturity. Though we generate revenues from product sales, 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, 2026 and December 31, 2025 (*in thousands*):

	September 30, 2026	December 31, 2025
Cash and cash equivalents	$ 123,542	$ 93,035
Marketable debt securities, at fair value	$ 348,276	$ 229,761
Convertible debt	$ 603,510	$ 311,724
Accumulated deficit	$ (1,596,949)	$ (1,472,713)
Stockholders' equity	$ 22,584	$ 114,828
Net working capital*	$ 422,534	$ 277,661
Net working capital ratio**	3.00	2.74

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

As of September 30, 2026, we had cash and cash equivalents of $123.5 million and available-for-sale marketable debt securities of $348.3 million. Substantial sources of funds over the past year, as summarized further below, include net proceeds of $158.4 million from the issuance of 2032 Notes after giving effect to the partial repurchase of 2029 Notes, a milestone payment of $25.0 million from Mirum and $10.2 million from the sale of Renalys stock to Chugai.

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 commercialization of FILSPARI in IgAN and expenses associated with the 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.

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 March 1, 2029, and 2032 Notes, which mature on May 15, 2032. 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 in August 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. Mirum achieved the first such milestone based on its annual net sales in 2025, and we received a payment of $25.0 million in April 2026, as a result of such achievement. Mirum achieved an additional milestone based on its annual net sales in 2026, resulting in the recognition of $10.0 million in milestone revenue. We expect to receive payment in the second quarter of 2027.

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, 2026, we have received milestone payments totaling $57.5 million 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 Chugai

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. In the fourth quarter of 2025, Renalys was acquired by and merged into Chugai. Through the acquisition, Chugai gained exclusive rights to develop and commercialize sparsentan in Japan, South Korea, and Taiwan. As a minority shareholder in Renalys, we received $10.2 million at the closing of the transaction and we are also eligible to receive multiple milestones according to the progress of sparsentan regulatory approval, and consideration linked to sparsentan's net sales in the applicable territory. Under the terms of the licensing agreement, Chugai is responsible for development, regulatory matters, and commercialization in the licensed territories.

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

Equity Offerings

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. We have not sold any shares under the ATM Agreement.

Operating Leases

Future Minimum Rental Commitments

As of September 30, 2026, we have future minimum rental commitments totaling $13.5 million arising from our operating lease and sublease income totaling $2.5 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, 2026, we have paid $48.7 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.

Everest License Agreement

In 2026, we entered the Everest Agreement, pursuant to which we obtained an exclusive license to develop, manufacture and commercialize civorebrutinib, an investigational oral, covalent reversible BTK inhibitor, and products containing civorebrutinib for any and all prophylactic, diagnostic and therapeutic uses and treatment of diseases and disorders in all countries outside of Greater China and certain countries in East and Southeast Asia (the "Territory"). The agreement became effective in July 2026 following the satisfaction of customary closing conditions.

Under the terms of the agreement, the Company paid Everest an upfront payment of $112.5 million in July 2026. Pursuant to the agreement, Everest is eligible to receive up to $1.0 billion in additional development, regulatory and commercial milestone payments, as well as tiered royalties ranging from high single-digit to double-digit percentages based on net sales of licensed products in the Territory. The Everest Agreement will remain in effect, unless terminated earlier, until the expiration of the royalty term for the licensed products in the Territory.

See Note 4 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. Through September 30, 2026, we have paid $65.2 million for contractual milestones achieved under the stock purchase agreement.

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 2032

On May 11, 2026, we completed a registered underwritten public offering of $525.0 million aggregate principal amount of our 2032 Notes. We issued the 2032 Notes under an indenture dated as of May 11, 2026, as supplemented by the first supplemental indenture, dated as of May 11, 2026 (collectively, the “2032 Indenture”). The 2032 Notes will mature on May 15, 2032, unless earlier repurchased, redeemed, or converted. The 2032 Notes are senior unsecured obligations of ours and bear interest at an annual rate of 0.50%, payable semi-annually in arrears on May 15 and November 15 of each year, beginning on November 15, 2026. The 2032 Notes do not contain any financial or operating covenants or any restrictions on the payment of dividends, the issuance of other indebtedness or the issuance or repurchase of securities by us.

Convertible Senior Notes Due 2029

On March 11, 2022, we completed a registered underwritten public offering of $316.3 million aggregate principal amount of our 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. On May 11, 2026, coinciding with the issuance of the 2032 Notes, we completed our repurchase of $221.4 million aggregate principal amount of 2029 Notes for cash. After giving effect to the repurchase, the total remaining principal amount outstanding under the 2029 Notes was $94.9 million. 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 FSGS, and to obtain regulatory approval for, and successfully commercialize our other or future product candidates;
- increases or decreases in revenue from our marketed products, including decreases in revenue resulting from generic entrants, changes in reimbursement or rebates, and/or health epidemics or pandemics;
- payment obligations related to the 2029 Notes and 2032 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 provided by operating activities from continuing operations for the nine months ended September 30, 2026 was $92.4 million compared to cash used of $22.9 million for the nine months ended September 30, 2025. The change was due to a $222.2 million increase in total net product sales, offset by an increase in operational expenses and the timing of payments from normal operations.

Cash Flows from Investing Activities

Cash used in investing activities for the nine months ended September 30, 2026 was $304.4 million compared to cash provided of $132.7 million for the nine months ended September 30, 2025. The change resulted primarily from the investment of the net proceeds of the 2032 Notes and partial repurchase of the 2029 Notes, acquisition of $113.1 million IPR&D from Everest, as well as timing differences in investment purchases, sales and maturities, and the fluctuation of our portfolio mix between cash equivalents and short-term investment holdings and an increase in intangible asset purchases.

Cash Flows from Financing Activities

Cash provided by financing activities for the nine months ended September 30, 2026 was $219.0 million compared to cash used of $60.1 million for the nine months ended September 30, 2025. The change was primarily due to net proceeds of $158.4 million from the issuance of the 2032 Notes and the partial repurchase of the 2029 Notes and an increase in proceeds from the exercise of stock options during the nine months ended September 30, 2026.

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, 2025 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, 2026, we had cash equivalents and marketable debt securities of approximately $471.8 million, consisting of money market funds, U.S. government agency debt, municipal bonds, 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. A change in interest rates of 100 basis points as of September 30, 2026 would have had approximately a $2.9 million impact on the fair value of our investments at September 30, 2026.

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 rates 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. During the three months ended September 30, 2026, the Company completed its implementation of a new enterprise resource planning ("ERP") system. The new ERP system integrates various financial and operational processes to enhance accuracy, efficiency, and internal controls. A standardized internal control framework was used during the implementation and monitoring controls have been established. Management will continue to assess and monitor the new ERP system to ensure the ongoing effectiveness of internal controls.

Other than the ERP system implementation, there have been no material changes in the Company's internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Securities Exchange Act of 1934) during the quarter ended September 30, 2026 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, 2025 as filed with the Securities and Exchange Commission ("SEC") on February 19, 2026.

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, and FILSPARI (sparsentan) to reduce proteinuria in adult and pediatric patients aged 8 years and older with focal segmental glomerulosclerosis ("FSGS") without nephrotic syndrome, which was granted full (traditional) approval by the FDA in April 2026. FILSPARI had previously been granted accelerated approval for IgAN in February 2023 based on the surrogate marker of proteinuria. The successful launch and commercialization of FILSPARI in IgAN and FSGS 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, in particular as it relates to the launch and commercialization in FSGS. 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, and because FILSPARI is the first and only medicine approved by the FDA for the treatment of FSGS, it is difficult to accurately 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 and FSGS patients. For example, if the addressable patient population suffering from primary IgAN or FSGS 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. Although we have gained experience in recent years regarding physician, patient and payer acceptance of FILSPARI, the degree of future adoption and utilization of FILSPARI may be affected by numerous factors, including its pricing, the recent approval in FSGS, the details of the approved labeling, the evolving clinical practice guidelines and treatment paradigms, payer coverage and reimbursement policies, developments related to existing and future 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 IgAN in February 2023, and in FSGS in April 2026, no one in our sales force had promoted FILSPARI for the treatment of IgAN or FSGS patients, respectively. 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, 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, 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. Similarly, in the fourth quarter of 2025, Renalys was acquired by and merged into Chugai Pharmaceutical Co., Ltd. ("Chugai"). While our agreements with these partners remain in place following the acquisitions, there is no guarantee that our collaboration with these partners will not be affected, adversely or otherwise, by the changes in ownership. Moreover, in connection with the acquisitions and related restructurings, substantially less resources could be devoted to the commercialization of sparsentan in the territories licensed to such parties, 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 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.

Pegtibatinase, if approved, is expected to be administered by patients or caregivers through self-administration using a delivery device. While we have not observed material issues related to self-administration in clinical studies to date, patient acceptance, proper use of the device, and adherence in broader or longer-term real-world use may differ from clinical trial experience, which could adversely affect utilization, outcomes, or commercial success.

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 abbreviated new drug application ("ANDA") seeking approval of a generic copy of an approved innovator product or a new drug application ("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 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, 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") generally requires an ANDA applicant seeking FDA approval of its proposed generic product prior to the expiration of a patent listed in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the "Orange Book") 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. If the required patent information was submitted to the FDA before the ANDA was submitted and 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, subject to applicable exceptions and adjustments, 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 applicable 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 stating 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, civorebrutinib, 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. As a result, certain pharmaceutical manufacturers have entered agreements with the administration that require the pharmaceutical manufacturers to offer, through a direct-to-consumer platform. U.S. patients and Medicaid programs prescription drug Most-Favored Nation pricing equal to or lower than those paid in other developed nations, with additional mandates for direct-to-patient discounts and repatriation of foreign revenues. As part of these MFN pricing initiatives, the administration is implementing or considering various CMS Center for Medicare and Medicaid Innovation models, under which manufacturers may owe rebates for certain prescription drugs under Medicaid or Medicare if the price of a drug included in an applicable model exceeds an international benchmark calculated based on prices available in certain reference countries. These include the finalized Global Benchmark for Efficient Drug Pricing Model (GLOBE) Model for certain Medicare Part B drugs; and the proposed Guarding United States Medicare Against Rising Drug Costs (GUARD) Model for certain Medicare Part D drugs, among others. 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, medicinal products may be subject to Health Technology Assessments (HTA) at the EU and national levels. This HTA is a multidisciplinary process that summarizes information regarding the medical, patient and social aspects, as well as economic and ethical issues, related to the use of a health, and is becoming an increasingly common part of the pricing and reimbursement procedures in some EU Member States, including those representing the larger markets. 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.

At the EU level, on January 12, 2025, Regulation No 2021/2282 entered into application through a phased implementation. The Regulation initially applies to new active substances for oncology and ATMPs. It will be expanded to orphan medicinal products in January 2028, and to all centrally authorized medicinal products as of 2030. 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 establishes a framework for EU-level joint clinical assessments, joint scientific consultations, and the early identification of emerging health technologies. It enables EU Member States to use common tools, methodologies, and procedures and requires them to take into account EU-level joint clinical assessment reports for the clinical components of their national HTA evaluations. 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. As implementation of the HTA Regulation is phased in and key methodological and procedural guidance continues to evolve, there remains uncertainty regarding the evidence requirements, timing, and impact of joint clinical assessments on national reimbursement processes. The new framework may result in additional or differently structured evidentiary expectations, misalignment between assessment and regulatory timelines, or delays in national decisions. Any adverse or delayed HTA outcomes, or divergent national reimbursement decisions, could negatively affect our ability to obtain or maintain favorable pricing and reimbursement status. 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 predictable 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 classical homocystinuria ("HCU"), we may not be able to maintain it in the EU and the orphan drug designation may not result in orphan drug exclusivity for pegtibatinase if approved, or FSGS in 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. In addition, the EU orphan medicinal product framework is subject to change under the pending overhaul of the EU general pharmaceutical legislative framework described below, which may modify the length of orphan market exclusivity and the manner in which market exclusivity is awarded to medicinal products with more than one approved orphan indication.

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. 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 vary 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.

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, 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, pegtibatinase or civorebrutinib 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 European Medicines Agency ("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.

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 have restarted enrollment activities for the Phase 3 HARMONY Study, and while 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 be conducted or completed 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, and in April 2026, the FDA granted full (traditional) approval of FILSPARI to reduce proteinuria in adult and pediatric patients aged 8 years and older with FSGS without nephrotic syndrome. 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 certain milestone payments from the sale of our bile acid product portfolio in August 2023. On July 28, 2026, we received an untitled letter from FDA's Office of Prescription Drug Promotion ("OPDP") regarding claims made in a professional visual aid for FILSPARI. The letter asserts that the professional visual aid includes certain false or misleading claims about the safety and efficacy of FILSPARI. We responded within the requested timeframe, and we are engaging with the FDA in an attempt to resolve the matter.

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, including but not limited to IgAN, FSGS, PMN and HCU, are relatively rare. Our estimates and projections of both the number of people who have a particular disease or condition, as well as the subset of people who have the potential to benefit from treatment with our product candidates, may not be accurate.

Currently, most reported estimates of the prevalence of such diseases or conditions are based on studies of small subsets of the population of specific geographic areas, which are then extrapolated to estimate the prevalence in the broader world population. As new studies are performed, the estimated prevalence of a particular disease or condition may change. There can be no assurance that the prevalence of any such disease or condition in study populations accurately reflect the prevalence in the broader world population.

If our estimates of the prevalence of a particular disease or condition, or of the number of patients who may benefit from treatment with sparsentan, pegtibatinase or civorebrutinib 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 submit a section viii statement 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 was covered by U.S. Patent No. 6,638,937, which expired in 2019 and to which we had 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 has an original expiration date in 2030, subject to the patent term extension described below, covers the use of sparsentan for treating IgAN as well as glomerulosclerosis, including FSGS. We have also been granted U.S. Patent No. 12,714,697, directed to certain methods of using sparsentan in IgAN. While we have additional pending U.S. and foreign patent applications directed to sparsentan and its uses, including an allowed U.S. patent application (U.S. Patent Application No. 19/253,120) directed to certain methods of using sparsentan in FSGS, there is no guarantee that any pending, allowed or future patent applications will result in issued patents, issue on a timeline that provides material protection, or, if issued, be listed in the Orange Book or 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. 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. The regulatory protection associated with 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. In September 2026, the U.S. Patent and Trademark Office ("USPTO") issued a final determination that U.S. Patent No. 9,993,461 is eligible for a patent term extension of 1,025 days, to January 17, 2033. We expect the USPTO to issue a certificate of extension reflecting this determination. In addition, while we intend to seek pediatric exclusivity for FILSPARI based on our ongoing development efforts, which, if granted, could provide an additional six months of regulatory exclusivity after such patent expires, 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 provides additional regulatory protection 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 for which we have obtained FDA approval in the areas 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 Chugai) 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 EPO. In March 2026, following an appeal hearing at the Technical Boards of Appeal of the EPO, the opposing party prevailed and the ‘277 EP Patent was revoked. Accordingly, previously granted supplementary protection certificates, or SPCs, based on the ‘277 EP Patent have been or will be invalidated. As a result of this outcome with respect to the ‘277 EP Patent and associated SPCs, we expect to rely on the data and/or marketing exclusivity that may be available in the EU. In addition, future changes to EU legislation may affect the availability, scope, or duration of rights granted after such changes take effect.

We have a license and collaboration agreement with Everest Medicines for certain rights to develop and commercialize civorebrutinib, including in the United States, and rely on licenses and sublicenses to certain patents and know-how owned or controlled by Everest Medicines and third parties. If we were to default on our obligations under the agreement or applicable upstream third-party agreements, or if the agreement were terminated, we could lose our rights to civorebrutinib and our ability to develop, manufacture or commercialize civorebrutinib could be adversely affected.

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. In addition, the EU orphan medicinal product framework is subject to change under the pending overhaul of the EU general pharmaceutical legislative framework described below, which may modify the length of orphan market exclusivity and the manner in which market exclusivity is awarded to medicinal products with more than one approved orphan indication.

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. There have been executive, judicial, Congressional, and political challenges and amendments to certain aspects of the PPACA. For example, on July 4, 2025, the One Big Beautiful Bill Act, or OBBBA, was signed into law, which narrowed access to PPACA marketplace exchange enrollment and declined to extend the PPACA enhanced advanced premium tax credits that expired at the end of 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. The OBBBA also 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. Congress is considering proposed legislation intended to further reduce healthcare costs with alternatives to replace the expired PPACA subsidies. 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.

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.

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 Health 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, HHS imposes rebates on many Medicare Part B and Medicare Part D products to penalize price increases that outpace inflation. In addition, HHS has been empowered to negotiate the price of certain single-source biologics that have been on the market for at least 11 years covered under Medicare as part of the Medicare Drug Price Negotiation Program. Each year up to twenty (20) products will be selected by HHS for the Medicare Drug Price Negotiation Program. Products subject to the Medicare Drug Price Negotiation Program are expected to experience a significant reduction in reimbursement from the Medicare program on a per unit basis. If coverage and adequate reimbursement are not available, or are available only to limited levels, we may not be able to successfully commercialize our current and any future product candidates that we develop, which could have an adverse effect on our operating results and our overall financial condition.

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 June 15, 2026, the FDA approved Colorado's Section 804 Importation Program ("SIP") proposal to import certain drugs from Canada for specific state healthcare programs. It is unclear how this and Florida's similar program, approved by the FDA in 2024, will be implemented and whether they will overcome potential legal, regulatory, or industry challenges in the United States and/or Canada. 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, Centers for Medicare & 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. For example, the current administration has announced agreements with certain pharmaceutical companies that require the drug manufacturers to offer, through a direct-to-consumer platform, U.S. patients and Medicaid programs prescription drug Most-Favored Nation pricing equal to or lower than those paid in other developed nations, with additional mandates for direct-to-patient discounts and repatriation of foreign revenues. Other recent actions and proposals include for example, (1) directing agencies to reduce agency workforce and cut programs; (2) 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; (3) imposing tariffs on certain imported pharmaceutical products; and (4) 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. Additionally, the current administration recently called on Congress to enact "The Great Healthcare Plan," to codify and expand Most-Favored Nation pricing, lower government subsidies to private insurance companies, increase healthcare price transparency, expand pharmaceutical drugs available for over-the-counter purchase, and enact restrictions on pharmacy benefit manager payment methodologies, among other things. 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. In June 2024, the U.S. Supreme Court's Loper Bright decision greatly reduced judicial deference to regulatory agencies, which could increase successful legal challenges to federal regulations affecting our operations. 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. 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.

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 $35 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 begin to gain approval. While the competitive landscape for FSGS is limited at this point in time, we expect that it will evolve as other companies advance their programs toward potential approvals in the future. 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 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 HCU. The voluntary enrollment pause enabled us to address necessary process improvements in manufacturing scale-up to support the initial 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. While we have made process improvements and have manufactured material at the larger scale, and while we restarted enrollment activities for the pivotal Phase 3 HARMONY Study in the first quarter of 2026, there is no guarantee that the Phase 3 HARMONY Study will be conducted or completed 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 or biologic substances used in our nonclinical and clinical studies and for the commercial supply of our products. 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. If our manufacturers are unable to obtain these materials, our nonclinical and clinical studies, product testing, regulatory activities, manufacturing and commercial supply of our products and product candidates could be delayed or interrupted. If our manufacturers or we are unable to purchase these materials for our commercial products or after regulatory approval has been obtained for any of our product candidates, commercial supply could be interrupted, commercial launches could be delayed, or shortages could occur, which could materially affect our ability to meet clinical trial timelines, and/or generate revenues from the sale of our products . 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. We also may encounter manufacturing process or supply challenges. For example, in September 2024, we announced a voluntary pause of enrollment in the Phase 3 HARMONY Study to enable us to address necessary process improvements in manufacturing scale-up; these process improvements have been achieved and enrollment has restarted. 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.

Changes in legislation could also potentially impact our ability to secure the materials we need for our products and product candidates. For example, the United States passed legislation in December 2025, namely the BIOSECURE Act (the "BIOSECURE Act"), to prohibit U.S. federal executive agencies from procuring or obtaining any biotechnology equipment or service produced or provided by a "biotechnology company of concern" or entering into or renewing a contract, loan, or grant with an entity that uses such biotechnology equipment or services. The BIOSECURE Act prohibits the U.S. government from procuring or obtaining biotechnology equipment or services produced or provided by a "biotechnology company of concern" ("BCC"); entering into, extending, or renewing government contracts with an entity that directly or indirectly uses biotechnology equipment or services from a BCC in performance of that federal contract; and/or issuing grants or loans to purchase, obtain, or use biotechnology equipment or services produced by a BCC. The BIOSECURE Act also prohibits U.S. government loan and grant recipients from using federal loan or grant money to enter into contracts with entities that use equipment from BCCs in the performance of any federal prime contract or subcontract. Companies designated as a BCC include those that are identified on the U.S.

Department of Defense's annual List of Chinese Military Companies, also known as the 1260H List, and the U.S. government also has the ability to designate entities as BCCs through a separate designation process. There is a "safe harbor" provision providing that the restrictions do not apply to equipment or services that were formerly but are no longer provided by a BCC, as well as a "grandfathering" provision providing that the prohibitions shall not apply for a five-year period to biotechnology equipment or services produced or provided under a contract or agreement entered into before the applicable effective date. Given the BIOSECURE Act, we may be restricted in our ability to work with certain Chinese biotechnology companies to the extent we would contract with, or otherwise receive funding from, the U.S. government. In June 2026, the Section 1260H list was expanded to include our current primary API supplier for sparsentan. In August 2026, a federal court preliminarily enjoined the Department of Defense from enforcing that designation while litigation proceeds. As a result, the designation cannot currently serve as the basis for the supplier's designation as a BCC under the BIOSECURE Act. As part of our regular supply continuity planning, we have taken steps over the past several years to identify, secure and qualify alternative sources of sparsentan API; however, there can be no assurance that any alternative source will be successfully qualified on a timely basis, obtain any necessary regulatory approvals, manufacture API at commercial scale, or supply API in sufficient quantities or on commercially reasonable terms. We may ultimately be required, or choose to transition away from our current primary API supply relationship for sparsentan if and when the BIOSECURE Act's restrictions become applicable to our primary API supplier and prior to or upon the expiration of the expected five-year grandfathering period. Any such transition could increase costs or adversely affect the commercial supply of sparsentan if we are unable to complete it successfully and on a timely basis. In addition, BCC designations, implementing regulations or other changes under the BIOSECURE Act could affect supply arrangements for certain of our other products or product candidates. As a result, we or our licensors, manufacturers or other supply chain partners may be required to identify and qualify alternative suppliers, which could increase costs or delay development, manufacturing or commercialization activities. For certain products and product candidates, there may be a limited number of suppliers and manufacturers with the required technical capabilities, manufacturing capacity or regulatory qualifications.

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. The Bureau of Industry and Security, U.S. Department of Commerce, initiated an investigation to determine whether pharmaceutical ingredients, including finished drug product, manufactured outside the United States pose a national security risk and should be subject to additional tariffs. Following that investigation, the President announced a proclamation which imposes 100% tariffs on certain patented pharmaceutical products and associated pharmaceutical ingredients. The proclamation provides a potential pathway to a 0% tariff rate for orphan drugs that address urgent U.S. health needs. Relevant agencies must interpret and implement the proclamation and determine which products qualify. Until that process is complete, it remains uncertain whether the 0% rate will apply to FILSPARI. We will continue to monitor developments and assess any potential impact on our business.

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.

Many of the trade, tariff, pricing, and related policy actions described herein remain subject to significant uncertainty, including with respect to their scope, duration, implementation, and enforceability. Such measures may be modified, delayed, suspended, repealed, or invalidated through administrative action, changes in policy priorities, or judicial challenges, and it is uncertain whether, when, or to what extent any such measures will ultimately be implemented or apply to our products, suppliers, or operations. Although we have taken and may continue to take certain actions intended to reduce potential exposure to supply chain or cost impacts, including efforts to diversify suppliers, there can be no assurance that such actions will be effective, timely, or sufficient to mitigate any adverse effects arising from these policies.

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

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

We have experienced significant growth over the past five years in the number of our employees and the scope of our operations. We have expanded our sales and marketing, compliance and legal functions in addition to expansion of all functions to support a commercial organization. We have also expanded our operations in connection with the commercial launch of FILSPARI in the United States, including by adding additional members to our sales force, and in connection with our development plans for civorebrutinib, which we licensed in from Everest Medicines in June 2026. 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 other parts of the world where it is approved or may be approved in the future. 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 from 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 and 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, and to obtain regulatory approval for, and successfully commercialize, our other or future product candidates;
- increases or decreases in revenue from our marketed products, including decreases resulting from generic entrants or health epidemics or pandemics;
- debt service obligations on the 2029 Notes and 2032 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 Chugai 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 the fourth quarter of 2025, our partner Renalys was acquired by and merged into Chugai, and as a result of the acquisition, Chugai gained exclusive rights to develop and commercialize sparsentan in Japan, South Korea, and Taiwan. We received a portion of the upfront payment due to the fact that it was a minority shareholder in Renalys prior to the acquisition, and we are also 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 we will receive any or all of the consideration that is 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. For example, in June 2026, we signed a license and collaboration agreement (the "Everest Agreement") with Everest Medicines (Singapore) Pte. Ltd. ("Everest"), pursuant to which Everest granted us an exclusive license for the development and commercialization of civorebrutinib and products containing civorebrutinib for any and all prophylactic, diagnostic and therapeutic uses and treatment of diseases and disorders in the U.S. and global markets excluding Greater China and certain countries in East and Southeast Asia. The Everest Agreement became effective in July 2026.

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.

Our ability to realize the anticipated benefits of in-licensed products and collaboration arrangements depends on several factors, many of which are outside of our control.*

We have entered into, and may in the future enter into, in-license, collaboration and other strategic agreements to acquire rights to develop and commercialize product candidates. For example, in June 2026 we entered into an exclusive license and collaboration agreement with Everest Medicines for the development and commercialization of civorebrutinib. Our ability to realize the anticipated benefits of these types of arrangements depends on a number of factors, many of which are outside of our control.

Under the terms of these agreements, we may rely on our licensors, collaborators or other counterparties to perform obligations that are important to the successful development and commercialization of licensed product candidates, including technology transfer activities, manufacturing support, the conduct of certain development activities, regulatory support and other responsibilities allocated to such parties. These agreements are often complex and require ongoing coordination among the parties. As a result, disagreements may arise regarding the interpretation of contractual terms, the parties' respective rights and obligations, development strategy or execution, intellectual property matters or differing business priorities. Delays or failures by these counterparties in performing their obligations, disagreements regarding development strategy or execution, differing business priorities, changes in a counterparty's financial condition or strategic focus, or other disputes between the parties could delay development, regulatory submissions or commercialization of licensed product candidates, increase our costs or otherwise adversely affect the value of the applicable license or collaboration. For example, under our agreement with Everest, we rely on Everest to perform certain technology transfer, development and other obligations with respect to civorebrutinib.

In addition, our rights to in-licensed products and product candidates are subject to the terms and conditions of the applicable license or collaboration agreements and may be subject to additional upstream license agreements. Accordingly, our rights may depend, in part, on agreements to which we are not a party and over which we have limited or no control. Disputes involving our licensors and their upstream licensors, or differing interpretations of applicable agreements, could adversely affect the scope or availability of rights that are important to our business. Furthermore, our ability to fully exploit these products and product candidates may be subject to contractual restrictions, diligence obligations, milestone and royalty requirements and other contractual provisions, including developmental cost-sharing obligations and reimbursements, and our failure, or our counterparty's failure, to comply with applicable contractual obligations could adversely affect our rights under these arrangements. If any such agreement is modified or terminated, whether as a result of a material breach, insolvency, failure to satisfy contractual obligations or otherwise, we could lose some or all of our rights to the applicable product or product candidate, be required to transfer or provide rights to certain regulatory filings or other assets developed under the license or collaboration, lose the benefit of investments we have made in the program, or incur additional costs in pursuing alternative development or licensing arrangements.

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, 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 certain regulatory licenses to manufacture or distribute our products commercially and/or 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;
- the FDA or comparable regulatory authorities determine that data from clinical trials conducted outside the United States, including studies conducted solely in China, are not adequate to support regulatory approval or require additional nonclinical, clinical or other studies before such data may be relied upon;
- our inability to obtain or maintain authorization to conduct clinical trials, including FDA clearance of an IND, on a timely basis or at all;
- 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. For certain of our product candidates, including civorebrutinib, clinical development to date has been conducted outside of the United States. Although the FDA may accept data from foreign clinical trials under applicable regulations, it may require additional studies, including bridging or U.S. clinical studies, or determine that such data are insufficient to support approval. In addition, the FDA's approach to the use of clinical data generated in China may continue to evolve. If the FDA requires us to generate additional data or repeat studies, our development timelines could be delayed, our costs could increase and our ability to obtain regulatory approval could be adversely affected.

On December 11, 2025, the Parliament and the Council of the EU reached a political agreement on a comprehensive overhaul of EU pharmaceutical legislation (the "Pharma Package"). The reform has been under negotiation since the European Commission submitted its proposal in April 2023. The Pharma Package, comprising a new directive and regulation to replace existing legislation, aims to modernize the EU framework. The Council formally adopted its first-reading position on the Pharma Package on September 28, 2026. The legislation is still subject to formal approval by the European Parliament, following which it will be published in the Official Journal of the European Union and enter into force. The new framework is expected to enter into force in late 2026 and to be subject to transitional arrangements, with general application not anticipated before 2028. The Pharma Package encompasses a broad range of measures, including changes to regulatory data and market protection periods, including an eight-year regulatory data protection period and a one-year market protection period, with opportunities for additional periods of market protection subject to specified conditions and a maximum combined regulatory protection period of eleven years; changes to the orphan medicinal product framework, including a reduction in the standard period of orphan market exclusivity from ten to nine years and an eleven-year period of orphan market exclusivity for certain "breakthrough" orphan medicinal products; and an expansion of the "Bolar" exemption to permit certain activities relating to generic and biosimilar medicines while relevant intellectual property rights remain in force, including submissions for procurement tenders. Any reduction in regulatory protection or orphan market exclusivity available for our products or product candidates in the EU, combined with the expanded Bolar exemption, could facilitate earlier generic or biosimilar competition, potentially impacting reimbursement status and the commercial prospects of our products and product candidates.

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 (collectively, "sensitive information"). 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, impose 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 personal information, such as conducting data privacy impact assessments or risk 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 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 have passed, 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 cookies or 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, 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 persons 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 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 APIs, 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. 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. For example, threat actors may use an initial compromise of one part of our environment to gain access to other parts of our environment, or leverage a compromise of our networks or systems to gain access to the networks or systems of third parties with whom we work, such as through phishing or supply chain attacks.

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. 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 and regulations 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.

If we fail to establish or maintain successful collaborations or licensing arrangements, including cross-border collaborations, our business could be adversely affected.*

Our current and future collaborations, partnerships, licensing and other arrangements may be affected by evolving U.S. and foreign laws, regulations and government policies governing cross-border investments, technology transfers, licensing arrangements and collaborations involving biotechnology and pharmaceutical companies. In particular, U.S. legislative and regulatory proposals have sought to expand governmental review of certain investments, licensing transactions and other business arrangements involving companies with operations or significant activities in China. Although these proposals remain subject to legislative and regulatory developments, if enacted or implemented, they could delay, restrict, increase the cost of, or otherwise adversely affect our ability to enter into, maintain or perform collaborations, licensing arrangements or other strategic transactions involving existing or prospective partners. Any such developments could require modifications to transaction structures, delay development timelines, increase compliance costs or limit our ability to realize the anticipated benefits of our collaborations.

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.

In July 2025, the OBBBA was signed into law, and it (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, 2025, we had federal NOL carryforwards of $232.6 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 in the fourth calendar quarter of 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 EMA 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 potential 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. Some of these initiatives have been subject to legal challenges, delays, stays, or changes in enforcement posture, and their ultimate scope, applicability, and timing remain uncertain. The ESG landscape has been evolving rapidly, and it can be difficult to predict future developments. 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 authorization, 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 exceeds 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 Financial Condition

Our indebtedness could adversely affect our financial condition.*

As of the end of the period covered by this report, we had approximately $620 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 and 2032 Notes if the notes are not converted into 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 and 2032 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 and 2032 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 and 2032 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 the 2029 Notes, the 2032 Notes or any other indebtedness.

We may be unable to raise the funds necessary to repurchase the 2029 Notes and 2032 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 and 2032 Notes or pay cash upon their conversion.*

Noteholders may require us to repurchase their 2029 Notes or 2032 Notes, as applicable, following a fundamental change at a cash repurchase price generally equal to the principal amount of the 2029 Notes and 2032 Notes to be repurchased, plus accrued and unpaid interest to, but excluding, the fundamental change repurchase date. In addition, upon conversion of the 2029 Notes or the 2032 Notes, we may be required or elect to satisfy all or a portion of our conversion obligation in cash, shares of our common stock or a combination thereof, depending on the terms of the applicable notes and any settlement election we make.

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 2032 Notes, or pay the cash amounts due upon conversion thereof. In addition, applicable law, regulatory authorities and the agreements governing our future indebtedness may restrict our ability to repurchase the 2029 Notes or 2032 Notes or pay the cash amounts due upon conversion thereof. Our failure to repurchase the 2029 Notes or 2032 Notes or to pay the cash amounts due upon conversion thereof when required will constitute a default under the base and supplemental indentures that govern the 2029 Notes and 2032 Notes, which we refer to collectively as the “indenture.” We may not have sufficient funds to satisfy all amounts due under the 2029 Notes, the 2032 Notes and any other indebtedness.

A default under the 2029 Notes or 2032 Notes may have a material adverse effect on our financial condition.*

If an event of default under the 2029 Notes or 2032 Notes occurs, the principal amount of the 2029 Notes or 2032 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 under the indentures governing the 2029 Notes and the 2032 Notes include, among other things:

- certain failures to make required payments of principal or interest;
- certain failures to comply with conversion, notice or other covenant obligations;
- specified defaults or accelerations with respect to other indebtedness; and
- certain bankruptcy, insolvency or reorganization events.

Accordingly, the occurrence of a default under the 2029 Notes or 2032 Notes, unless cured or waived, may have a material adverse effect on our results of operations.

Provisions of the 2029 Notes and 2032 Notes could discourage an acquisition of us by a third party.*

Certain provisions of the 2029 Notes and 2032 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, under the applicable indenture governing the 2029 Notes and 2032 Notes, holders of the 2029 Notes or 2032 Notes, as the case may be, will have the right, at their option, to require us to repurchase all of their 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 of the 2029 Notes and/or 2032 Notes, as applicable, in connection with certain make-whole fundamental changes.

Conversion of the 2029 Notes or 2032 Notes may dilute the ownership interest of existing stockholders, including holders who had previously converted their 2029 Notes or 2032 Notes.*

To the extent we issue shares of common stock upon conversion of the 2029 Notes or the 2032 Notes, the conversion of some or all of such notes may 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 and 2032 Notes may encourage short selling by market participants because the conversion of the notes could be used to satisfy short positions, which 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. While we cannot predict the broader consequences, conflicts 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, 2026, certain 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, as set forth below. Each 10b5-1 plan listed below is part of such individual's long-term asset diversification, tax and/or financial planning strategy, and has been entered into in accordance with the terms of our Insider Trading Policy. Under the terms of the plans, once the plans are in effect, the individuals listed below have no discretion or control over the timing or effectuation of any transactions in our securities pursuant to the plans, and sales will be made only if the stock meets the minimum price thresholds and/or other requirements specified in the applicable plan. Each such director and/or officer continues to be subject to our stock ownership guidelines, and the execution of all potential sales under the plan would not by themselves cause any such individual to be out of compliance with the requirements of our stock ownership guidelines.

Trading Arrangements Adopted:

Name & Title	Date Adopted	Character of Trading Arrangement (1)	Aggregate Number of Shares of Common Stock to be Sold Pursuant to Trading Arrangement	Expiration Date (2)
Roy Baynes member of our Board of Directors	August 14, 2026	Rule 10b5-1 Trading Arrangement	Up to 36,000 shares	August 6, 2027
Ron Squarer member of our Board of Directors	August 14, 2026	Rule 10b5-1 Trading Arrangement	Up to 27,500 shares (3)	April 21, 2027

1 Each trading arrangement marked as a "Rule 10b5-1 Trading Arrangement" is intended to satisfy the affirmative defense of Rule 10b5-1(c) under the Exchange Act (the "Rule").

2 Each trading arrangement permits transactions through and including the earlier to occur of (a) the completion of all sales and (b) the date listed in the table. Each trading arrangement marked as a "Rule 10b5-1 Trading Arrangement" only permits transactions upon expiration of the applicable mandatory cooling-off period under the Rule.

3 Consists of shares underlying stock options expiring in April 2027 and May 2028.

Trading Arrangements Terminated:

None.

Item 6. Exhibits

(a) Exhibits

3.1	Certificate of Incorporation of the Company (incorporated by reference to Exhibit 3.1 to Amendment No. 2 to the Company's General Form for Registration of Securities on Form 10-12G, filed with the SEC on October 28, 2010).
3.2	Certificate of Amendment of Certificate of Incorporation of the Company (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K, filed with the SEC on June 11, 2015).
3.3	Certificate of Amendment of Certificate of Incorporation of the Company (incorporated by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K, filed with the SEC on November 16, 2020).
3.4	Certificate of Amendment of Certificate of Incorporation of the Company (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K, filed with the SEC on May 18, 2021).
3.5	Amended and Restated Bylaws of the Company (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K, filed with the SEC on November 16, 2020).
3.6	Certificate of Amendment of Bylaws of 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).
10.1†	Transition and Separation Agreement, dated September 21, 2026, between the Company and Eric Dube (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on September 21, 2026).
10.2†	Employment Agreement, dated September 21, 2026 and effective as of December 1, 2026, between the Company and Bradley L. Campbell (incorporated by reference to Exhibit 10.2 to the Company's Current Report on Form 8-K, filed with the SEC on September 21, 2026).
31.1	Chief Executive Officer's Certification pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Chief Financial Officer's Certification pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Chief Executive Officer's Certification pursuant to Section 906 of the Sarbanes Oxley Act of 2002
32.2	Chief Financial Officer's Certification pursuant to Section 906 of the Sarbanes Oxley Act of 2002
101.INS	Inline XBRL Instance Document
101.SCH	Inline XBRL Taxonomy Extension Schema Document
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Taxonomy Extension Presentation Linkbase Document
104	The cover page to this Quarterly Report on Form 10-Q has been formatted in Inline XBRL

† Indicates management contract or compensatory plan.

SIGNATURES

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

Date: November 2, 2026

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: November 2, 2026

/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: November 2, 2026

/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, 2026 (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: November 2, 2026

/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, 2026 (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: November 2, 2026

/s/ Christopher Cline

Christopher Cline

Chief Financial Officer

(Principal Financial Officer)