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DELTA REPORT

10-Q

TVTX - TRAVERE THERAPEUTICS, INC

10-Q - SEPTEMBER 30, 2024 COMPARED TO 10-Q - JUNE 30, 2024

The following comparison report has been automatically generated

TOTAL DELTAS	1644
CHANGES	242
DELETIONS	216
ADDITIONS	1186

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

FORM 10-Q

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

For the quarterly period ended June 30, 2024 September 30, 2024

or

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

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

TRAVERE THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

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

3611 Valley Centre Drive, Suite 300

San Diego, CA 92130

(Address of Principal Executive Offices)

(888) 969-7879

(Registrant's Telephone number including area code)

N/A

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

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

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

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☑ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☑ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☑	Accelerated filer	☐
Non-accelerated filer	☐	Smaller reporting company	☐
		Emerging growth company	☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☑

The number of shares of outstanding common stock, par value $0.0001 per share, of the Registrant as of July 29, 2024 October 28, 2024 was 76,491,485. 78,049,928.

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TRAVERE THERAPEUTICS, INC.

Form 10-Q
For the Fiscal Quarter Ended June 30, 2024 September 30, 2024

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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, 2023, and in this Quarterly Report on Form 10-Q. You are urged not to place undue reliance on these forward-looking statements, which speak only as of the date of this report.

In addition, statements that "we believe" and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this Quarterly Report on Form 10-Q, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain and you are cautioned to not unduly rely upon these statements.

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

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

Risk Factor Summary

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

- Our future prospects are highly dependent upon our ability to successfully develop and execute commercialization strategies for our products, including FILSPARI, (sparsentan) to reduce proteinuria in adults with primary Immunoglobulin A nephropathy (IgAN), and to attain market acceptance among physicians, patients and healthcare payers.

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- 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 clinical trials does not guarantee any particular outcome from or timeline for regulatory review, and expedited regulatory review pathways may not actually lead to faster development or approval.
- In order to operate our business and increase adoption and sales of our products, we need to continue to develop our commercial organization, including maintaining a highly experienced and skilled workforce with qualified sales representatives.
- Interim, topline and preliminary data from our clinical trials that we announce or publish may change materially as more patient data become available and audit and verification procedures are completed.
- We face substantial generic and other competition, and our operating results will suffer if we fail to compete effectively.
- Healthcare reform initiatives, unfavorable pricing regulations, and changes in reimbursement practices of third-party payers or patients' access to insurance coverage could affect the pricing of and demand for our products.
- We are dependent on third parties to manufacture and distribute our products.
- The market opportunities for our products and product candidates may be smaller than we believe they are.
- Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval or commercialization.
- We do not currently have patent protection for certain of our commercial products. If we are unable to obtain and maintain intellectual property relating to our technology and products, their value may be adversely affected.
- We expect to rely on orphan drug status to develop and commercialize certain of our product candidates, but our orphan drug designations may not confer marketing exclusivity or other expected commercial benefits.
- We will likely experience fluctuations in operating results and could incur substantial losses, and the market price for shares of our common stock may be volatile.

- Negative publicity regarding any of our products could impair our ability to market any such product and may require us to spend time and money to address these issues.
- We may need substantial funding and may be unable to raise capital when needed. Our indebtedness could adversely affect our financial condition.
- We may 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)

		June 30, 2024	December 31, 2023		September 30, 2024	December 31, 2023
Assets	**Assets**	(unaudited)		**Assets**	(unaudited)	
Current assets:	Current assets:			Current assets:		
Cash and cash equivalents						
Marketable debt securities, at fair value						
Accounts receivable, net						
Inventory						
Prepaid expenses and other current assets						
Total current assets						
Total current assets						
Total current assets						

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Long-term inventory		
Property and equipment, net		
Operating lease right of use assets		
Intangible assets, net		
Other assets		
Total assets		
Total assets		
Total assets		
Liabilities and Stockholders' Equity		
Liabilities and Stockholders' (Deficit) Equity		
Current liabilities:	Current liabilities:	Current liabilities:
Accounts payable		
Accrued expenses		
Convertible debt, current portion		
Deferred revenue, current portion		
Operating lease liabilities, current portion		
Other current liabilities		
Total current liabilities		
Total current liabilities		
Total current liabilities		
Convertible debt		
Deferred revenue, less current portion		
Convertible debt, less current portion		
Operating lease liabilities, less current portion		
Operating lease liabilities, less current portion		
Operating lease liabilities, less current portion		
Other non-current liabilities		
Total liabilities		
Total liabilities		
Total liabilities		
	Commitments and Contingencies (See Note	Commitments and Contingencies (See
Commitments and Contingencies (See Note 13)	13)	Note 13)
Stockholders' Equity:		
Preferred stock $0.0001 par value; 20,000,000 shares authorized; no shares issued and outstanding as of June 30, 2024 and December 31, 2023		
Common stock $0.0001 par value; 200,000,000 shares authorized; 76,456,562, and 75,367,117 issued and outstanding as of June 30, 2024 and December 31, 2023, respectively		
Stockholders' (Deficit) Equity:		
Preferred stock $0.0001 par value; 20,000,000 shares authorized; no shares issued and outstanding as of September 30, 2024 and December 31, 2023		
Common stock $0.0001 par value; 200,000,000 shares authorized; 77,909,042, and 75,367,117 issued and outstanding as of September 30, 2024 and December 31, 2023, respectively		
Additional paid-in capital		
Accumulated deficit		
Accumulated other comprehensive loss		
Total stockholders' equity		
Total liabilities and stockholders' equity		
Total stockholders' (deficit) equity		
Total liabilities and stockholders' (deficit) equity		

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

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

	Three Months Ended June 30,		Six Months Ended June 30,		Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023	2024	2023	2024	2023
Net product sales								
License and collaboration revenue								
Total revenue								
Operating expenses:								
Cost of goods sold								
Cost of goods sold								
Cost of goods sold								
Research and development								
Selling, general and administrative								
In-process research and development								
Restructuring								
Total operating expenses								
Operating loss								
Other (expense) income, net:								
Other income, net:								
Interest income								
Interest income								
Interest income								
Interest expense								
Other (expense) income, net								
Other income (expense), net								
Total other (expense) income, net								
Total other (expense) income, net								
Total other (expense) income, net								
Total other income, net								
Total other income, net								
Total other income, net								
Loss from continuing operations before income tax provision								
Income tax provision on continuing operations								
Income tax benefit (provision) on continuing operations								
Loss from continuing operations, net of tax								
(Loss) income from discontinued operations, net of tax								
Net loss								
Net (loss) income								
Per share data								
Per share data								
Per share data								
Basic and diluted:								
Basic and diluted:								
Basic and diluted:								
Net loss from continuing operations								
Net loss from continuing operations								
Net loss from continuing operations								

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Net (loss) income from discontinued operations	
Net loss per common share	
Net income (loss) from discontinued operations	
Net (loss) income per common share	
Weighted average common shares outstanding	
Weighted average common shares outstanding	
Weighted average common shares outstanding	
Comprehensive loss:	
Comprehensive (loss) income:	
Comprehensive loss:	
Comprehensive (loss) income:	
Comprehensive loss:	
Net loss	
Net loss	
Net loss	
Comprehensive (loss) income:	
Net (loss) income	
Net (loss) income	
Net (loss) income	
Foreign currency translation gain (loss)	
Foreign currency translation gain (loss)	
Foreign currency translation gain (loss)	
Unrealized loss on marketable debt securities	
Comprehensive loss	
Foreign currency translation (loss) gain	
Foreign currency translation (loss) gain	
Foreign currency translation (loss) gain	
Unrealized gain (loss) on marketable debt securities	
Comprehensive (loss) income	

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

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

	Three Months Ended June 30, 2024						Three Months Ended June 30, 2023					
	Three Months Ended September 30, 2024						Three Months Ended September 30, 2023					
	Common Stock Shares	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	Common Stock	Additional Paid in Capital	Accumulated Other Comprehensive Loss	Accumula Deficit
Balance - March 31															
Balance - March 31															
Balance - March 31															
Balance - June 30															
Balance - June 30															
Balance - June 30															
Share based compensation															
Share based compensation															

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Share based compensation																
Issuance of common stock under the equity incentive plan and proceeds from exercise																
Employee stock purchase program purchase and expense																
Exercise of pre-funded common stock warrants																
Exercise of pre-funded common stock warrants																
Exercise of pre-funded common stock warrants																
Foreign currency translation adjustments																
Foreign currency translation adjustments																
Foreign currency translation adjustments																
Foreign currency translation adjustments																
Unrealized loss on marketable debt securities																
Net loss																
Balance - June 30																
Unrealized gain on marketable debt securities																
Net (loss) income																
Balance - September 30																

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

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

	Six Months Ended June 30, 2024						Six Months Ended June 30, 2023					
	Nine Months Ended September 30, 2024						Nine Months Ended September 30, 2023					
	Common Stock Shares	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	Common Stock	Additional Paid in Capital	Accumulated Other Comprehensive Loss	Accumula Deficit
Balance - December 31															
Balance - December 31															

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Balance - December 31	
Share based compensation	
Share based compensation	
Share based compensation	
Issuance of common stock under the equity incentive plan and proceeds from exercise	
Employee stock purchase program purchase and expense	
Issuance of common stock, net of issuance costs of $12.6 million	
Issuance of pre-funded common stock warrants, net of issuance costs of $1.6 million	
Exercise of pre-funded common stock warrants	
Foreign currency translation adjustments	
Foreign currency translation adjustments	
Foreign currency translation adjustments	
Unrealized loss on marketable debt securities	
Unrealized gain (loss) on marketable debt securities	
Net loss	
Balance - June 30	
Balance - September 30	

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

	For the Six Months Ended June 30,	For the Nine Months Ended September 30,

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	2024	2023	2024	2023
Cash Flows From Operating Activities:				
Net loss				
Net loss				
Net loss				
Net (loss) income from discontinued operations				
Net loss from continuing operations				
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation and amortization				
Depreciation and amortization				
Depreciation and amortization				
Share based compensation				
In-process research and development				
In-process research and development				
In-process research and development				
Other				
Changes in operating assets and liabilities:				
Accounts receivable				
Accounts receivable				
Accounts receivable				
Inventory				
Inventory				
Inventory				
Prepaid expenses and other current and non-current assets				
Accounts payable				
Accounts payable				
Accounts payable				
Accrued expenses				
Deferred revenue, current and non-current				
Other current and non-current liabilities				
Net cash used in operating activities - continuing operations				
Net cash (used in) provided by operating activities - discontinued operations				
Net cash used in operating activities				
Cash Flows From Investing Activities:	**Cash Flows From Investing Activities:**		**Cash Flows From Investing Activities:**	
Proceeds from the sale and maturity of marketable debt securities				
Purchase of marketable debt securities				
Purchase of fixed assets				
Purchase of intangible assets				
Payment of milestone				
Net cash provided by (used in) investing activities - continuing operations				
Cash Flows From Financing Activities:				
Cash Flows From Financing Activities:				
Net cash provided by investing activities - discontinued operations				
Net cash provided by investing activities				
Cash Flows From Financing Activities:			**Cash Flows From Financing Activities:**	
Payment of guaranteed minimum royalty				
Payment of guaranteed minimum royalty				

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Payment of guaranteed minimum royalty	
Proceeds from the issuance of common stock, net of issuance costs	
Proceeds from the issuance of common stock, net of issuance costs	
Proceeds from the issuance of common stock, net of issuance costs	
Proceeds from the issuance of pre-funded warrants, net of issuance costs	
Proceeds from exercise of stock options	
Proceeds from issuances under the employee stock purchase plan	
Net cash provided by financing activities - continuing operations	
Net cash provided by financing activities - continuing operations	
Net cash provided by financing activities - continuing operations	
Net cash used in financing activities - discontinued operations	
Net cash provided by financing activities	
Effect of exchange rate changes on cash	
Net (decrease) increase in cash and cash equivalents	
Cash and cash equivalents, beginning of year	
Cash and cash equivalents, end of period	

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 through licenses and acquisitions of products in areas that will serve patients with serious unmet medical need and that the Company believes offer attractive growth characteristics.

Discontinued Operations - Sale of Bile Acid Product Portfolio

In July 2023, Travere entered into an Asset Purchase Agreement (the “Purchase Agreement”) with Mirum Pharmaceuticals, Inc. ("Mirum Pharmaceuticals" or “Mirum”), pursuant to which Mirum agreed to purchase from Travere substantially all of the assets primarily related to Travere’s business of development, manufacture (including synthesis, formulation, finishing or packaging) and commercialization of Chenodal and Cholbam (also known as Kolbam, and together with Chenodal, the “Products”), collectively, the "bile acid business". On August 31, 2023, the Company and Mirum consummated the transactions contemplated by the Purchase Agreement (the “Closing”). In connection with the Closing, Mirum paid Travere an upfront cash payment of $210.0 million. Pursuant to the Purchase Agreement, after the Closing, Travere 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 the Products. The Company has reflected the bile acid business as a discontinued operation in the unaudited consolidated financial statements for all periods presented. See Note 18 for further discussion.

Unless otherwise noted, amounts and disclosures throughout the Notes to the unaudited consolidated financial statements relate to the Company's continuing operations.

Approved Products:

FILSPARI® (sparsentan)

On February 17, 2023 September 5, 2024, the U.S. Food and Drug Administration (the "FDA") FDA granted accelerated full approval of FILSPARI® (sparsentan) to reduce proteinuria slow kidney function decline in adults with primary IgAN Immunoglobulin A nephropathy (IgAN) who are at risk of rapid disease progression, generally a UPCR ≥1.5 gram/gram. progression. FILSPARI a is the only oral, once-daily, oral non-immunosuppressive medication is designed to selectively target that directly targets glomerular injury in the kidney by blocking two critical pathways (endothelin 1 and angiotensin-II) in the of IgAN disease progression of IgAN. (endothelin-1 and angiotensin II).

This indication was FILSPARI had previously been granted under accelerated approval in February 2023 based on reduction in the surrogate marker of proteinuria. The continued Full approval of FILSPARI may be contingent upon confirmation of a clinical benefit in the Company’s Phase 3 clinical trial of sparsentan for the treatment of IgAN (the "PROTECT Study"). In September 2023, the Company announced topline two-year is based on positive long-term confirmatory secondary endpoint results from the PROTECT Study and in December 2023, the Company announced the completion of a successful pre-NDA meeting with the FDA for demonstrating that FILSPARI in IgAN. In March 2024, the Company submitted a supplemental New Drug Application (sNDA) for conversion of the existing U.S. accelerated approval of significantly slowed kidney function decline over two years compared to irbesartan. FILSPARI to full approval. In May 2024, the Company announced that the FDA has accepted and granted Priority Review of its sNDA to convert FILSPARI from accelerated approval to full approval for the treatment of IgAN initially became commercially available in the U.S. The FDA assigned in February 2023 under accelerated approval, and the Company is providing a Prescription Drug User Fee Act (“PDUFA”) target action date of September 5, 2024. comprehensive patient support program throughout the patient’s treatment journey.

In September 2021, the Company entered into a license and collaboration agreement with Vifor (International) Ltd. ("CSL Vifor"). In April 2024, the Company and CSL Vifor announced that the European Commission has 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). The CMA is granted for all member states of the European Union, as well as in Iceland, Liechtenstein and Norway. The European Commission's decision follows the positive opinion from the Committee for Medicinal Products for Human Use (“CHMP”) in February 2024, based on results from the pivotal Phase 3 PROTECT Study of FILSPARI in IgAN. Under the terms of the License Agreement, the Company will be entitled to receive a regulatory milestone payment of $17.5 million upon receipt of full regulatory approval by the European Commission for IgAN, and an additional milestone payment upon achievement of market access initiatives in certain countries. FILSPARI became commercially available in Europe under the CMA in August 2024, with an initial launch in Germany and Austria. In October 2024, the Company and CSL Vifor announced that Swissmedic has granted temporary marketing authorization for FILSPARI for the treatment of adults with primary IgAN with a urine protein excretion ≥1.0 g/day (or urine protein-to-creatinine ratio ≥0.75 g/g).

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Thiola® and Thiola EC® (tiopronin tablets)

Thiola® and Thiola EC® (tiopronin tablets) are approved in the United States for the prevention of cystine (kidney) stone formation in patients with severe homozygous cystinuria.

Clinical-Stage Programs:

Sparsentan for the treatment of FSGS

Sparsentan remains a novel investigational product candidate which has been granted Orphan Drug Designation for the treatment of focal segmental glomerulosclerosis (FSGS) in the U.S. and the European Economic Area countries (the "EEA"). In December 2023, the Company announced that it had completed its planned Type C meeting with the FDA to discuss previously reported results from the Phase 3 DUPLEX Study of sparsentan in FSGS. The FDA acknowledged the high unmet need for approved therapies as well as the challenges in studying FSGS but indicated that the two-year results from the

Phase 3 DUPLEX Study alone were not sufficient to support an sNDA submission. Together with CSL Vifor, the Company also plans to engage with the EMA to determine the potential for a subsequent variation to the CMA of sparsentan for the treatment of FSGS, subject to a review decision on the pending application for CMA of sparsentan in IgAN. The Company is conducting additional analyses of FSGS data and plans to engage with regulators to evaluate potential regulatory pathways for a sparsentan FSGS indication.

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. Topline results from

In September 2024, the Company announced a voluntary pause of enrollment in the Phase 3 HARMONY Study. The voluntary enrollment pause enables the Company to work to address necessary process improvements in manufacturing scale-up to support commercial scale manufacturing as well as full enrollment in the HARMONY Study Study. Patients currently enrolled in pegtibatinase studies continue to receive study medication from small scale batches which are expected unaffected by the scale-up process. Currently enrolled patients will be able to continue on study medication as scheduled for the duration of the trials they are participating in. The voluntary enrollment pause was enacted following our determination that the desired drug substance profile was not achieved in 2026. the recent scale-up process. The Company expects to further evaluate the necessary commercial process improvements to enable the continuation of the Phase 3 program.

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

Preclinical Programs:

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

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, 2023, filed with the SEC on February 20, 2024. 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, 2023 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, including reclassifying the prior period revenues and expenses attributable to the bile acid business as net income from discontinued operations. These reclassifications did not have an impact on total assets or total liabilities in the Consolidated Balance Sheets or net loss in the Consolidated Statements of Operations.

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

Principles of Consolidation

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

Revenue Recognition

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

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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 to which the sales-based milestone or royalty has been allocated has been satisfied. Revenue from collaboration and licensing agreements may also include sales of inventory, at cost plus a margin, which is recorded in license and collaboration revenue.

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

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

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

Cost of goods sold

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

The following table summarizes cost of goods sold for the three and six nine months ended June 30, 2024 September 30, 2024 and 2023 (*in thousands*):

	Three Months Ended June 30,		Six Months Ended June 30,	
	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Cost of goods sold - product sales				
Cost of goods sold - license and collaboration				
Total cost of goods sold				

Capitalization of Inventory Costs

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

Prior to the February 2023 FDA accelerated approval of FILSPARI (sparsentan), the Company expensed the production of active pharmaceutical ingredients purchased to support the commercial launch of FILSPARI, in research and development expenses. For the three and six nine months ended June 30, 2024 September 30, 2024 and 2023, sales of FILSPARI primarily consisted of zero-cost inventories. As of June 30, 2024 September 30, 2024, the Company had approximately $3.5 $2.6 million of zero-cost inventory. The Company expects to continue to record zero cost of goods sold on the sale of previously expensed inventories through at least 2025. The Company began capitalizing inventory costs associated with FILSPARI following the February 2023 accelerated approval.

Research and Development Expenses

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

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Clinical Trial Expenses

The Company records expenses in connection with its clinical trials under contracts with contract research organizations ("CROs") that support conducting and managing clinical trials, as well as contract manufacturing organizations ("CMOs") for the manufacture of drug product supplies to support clinical development. The financial terms and activities of these agreements vary from contract to contract and may result in uneven expense levels. Generally, these agreements set forth activities that drive the recording of expenses such as start-up, initiation activities, enrollment, treatment of patients, or the completion of other clinical trial activities, and in the case of CMOs, costs associated with the production of drug product supplied and the procurement of raw materials to be consumed in the manufacturing process.

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

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

Intangible Assets with Cost Accumulation Model

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

In the second quarter of 2023, the Company reduced the estimated useful life of the Thiola Intangible to better reflect the pattern of projected future cash flows. The change in estimated useful life was accounted for as a change in accounting estimate with the remaining carrying amounts of the Thiola Intangible being amortized prospectively over the new useful life.

Consistent with all prior periods since Thiola was acquired, the Company has not accrued any liability for future royalties in excess of the annual contractual minimum at June 30, 2024 September 30, 2024 as such royalties are not yet probable and estimable.

Variable Interest Entity

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

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

Equity Securities

The Company applies the equity method of accounting for investments when it has significant influence, but no controlling interest in the investee. Judgment regarding the level of influence over each equity method investment includes key factors such as ownership interest, representation on the board of directors, participation in joint steering committees and material intercompany transactions. The Company evaluates any basis difference between the carrying value and fair value of the Company's proportionate share of the investee's net assets. Basis differences relating to in-process research and development (IPR&D) are expensed when the investee is not considered a business as defined in ASC 805, *Business Combinations,* due to substantially all of the estimated fair value of the gross assets being concentrated in a group of similar IPR&D assets with no alternative future use. For the three and six nine months ending June 30, 2024 September 30, 2024, the Company recognized $3.4 million in other expense in the Company's consolidated statements of operations for these basis adjustments. The equity method investment's carrying value was reduced to zero as the Company's proportionate share of the basis difference exceeded the carrying value.

See Note 6 for further discussion. Investments accounted for using the equity method may be reported on a lag of up to three months if the financial statements of the investee are not available in sufficient time for the Company to apply the equity method as of the current reporting date.

Discontinued Operations

Discontinued operations is presented when there is a disposal of a component or a group of components that in the Company's judgment represents a strategic shift that will have a major effect on the Company's operations and financial results. Results of operations directly related to discontinued operations are aggregated into a single line item in the Consolidated Statements of Operations for all periods presented. See Note 18 for further discussion.

Restructuring

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

In December 2023, the Company initiated a restructuring plan that resulted in a reduction of its workforce, primarily impacting non-field-based employees. One-time termination benefits include severance, continuation of health insurance coverage, and other benefits for a specified period of time. The Company estimated that it will incur a total of $12.0 million to $14.0 million in non-recurring charges in connection with the restructuring, of which the Company has recognized a total of $12.3 million $12.4 million as of June 30, 2024 September 30, 2024, including $0.7 million $0.1 million and

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$0.9 million $1.0 million for the three and six nine months ended June 30, 2024 September 30, 2024, respectively. As of June 30, 2024 September 30, 2024 and December 31, 2023, the Company had accruals related to the restructuring of $1.1 million $0.4 million and $11.4 million, respectively, which is included in accrued expenses in the Consolidated Balance Sheets. Cash payments totaling $11.3 million $12.1 million were made related to the restructuring during 2024. The Company expects that it will incur the remaining estimated restructuring costs during 2024. The Company anticipates it will and pay all remaining restructuring plan amounts costs during 2024.

The following table sets forth a summary of changes in accrued restructuring costs for the six nine months ended June 30, 2024 September 30, 2024 and 2023 (*in thousands*):

	Restructuring				
	2024	2024	2023	2024	2023
Liability balance at January 1,					
Restructuring expenses					
Payments					
Foreign currency impact					
Liability balance at June 30,					
Liability balance at September 30,					

Recently Adopted Accounting Pronouncements

In November 2023, the FASB issued ASU No. 2023-07, Improvements to Reportable Segment Disclosure. The FASB amended the guidance in ASC 280, *Segment Reporting* ("ASC 280"), to require a public entity to disclose significant segment expenses and other segment items on an annual and interim basis and to provide in interim periods all disclosures about a reportable segment's profit or loss and assets that are currently required annually. Public entities with a single reportable segment are required to provide the new disclosures and all the disclosures required under ASC 280. The guidance is applied retrospectively to all periods presented in financial statements, unless it is impracticable. This new guidance is effective for public business entities for annual periods beginning after December 15, 2023, and for interim periods beginning after December 15, 2024. The Company adopted plans to adopt this new standard as of January 1, 2024, however, for its Annual Report on Form 10-K for the adoption did not have an impact on the accompanying quarterly financial statements since the standard is not effective for interim periods until fiscal 2025. year ended December 31, 2024. While this accounting standard will increase disclosures, it will not have a material impact on the Company's financial statements.

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 December 2023, the FASB issued ASU No. 2023-09, Improvements to Income Tax Disclosures. This new standard does not change accounting for income taxes but requires new disclosures focusing on two areas, the effective rate reconciliation and taxes paid. This new standard is effective for public business entities for annual periods beginning after December 15, 2024. Early adoption is permitted. The Company expects to adopt this new standard beginning in fiscal 2025 when it becomes effective.

NOTE 3. REVENUE RECOGNITION

Product Sales, Net

Product sales consist of FILSPARI and tiopronin products (Thiola and Thiola EC). The Company sells its products to specialty pharmacies and through direct-to-patient distributors worldwide, with the United States representing over 98% of net product sales.

The Company sells FILSPARI to three two direct-to-patient specialty pharmacies. The Company sells its other 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 other tiopronin products, product sales are recognized upon delivery to the patient. The Company receives payments from sales of its other products, primarily through third party payers, based on terms that generally are within 30 days of delivery of product to the patient. Contracts do not contain significant financing components based on the typical period of time between performance of services and collection of consideration.

Deductions from Revenue

Revenues from product sales are recorded at the net sales price, which includes provisions resulting from discounts, rebates and co-pay assistance that are offered to customers, payers and other indirect customers relating to the Company's sales of its products. These provisions are based on the estimates of the amounts earned or to be claimed on the related sales. These amounts are treated as variable consideration, estimated and recognized as a reduction of the transaction price at the time of the sale, using the most likely amount method, and are classified as a reduction of accounts receivable (if the amount is payable to a customer) or as a current liability (if the amount is payable to a party other than a customer). The Company includes these estimated amounts in the transaction price to the extent it is probable that a significant reversal of cumulative revenue recognized for such transactions will not occur. Where appropriate, these reserves take into consideration the Company's historical experience, current contractual and statutory requirements and specific known market events and trends. Overall, these reserves reflect the Company's best estimates of the amount of consideration to which it is entitled based on the terms of the contract. If actual results in the future vary from the Company's provisions, the Company will adjust the estimate, which would affect net product revenue and earnings in the period such variances become known. For the six nine months ended June 30, 2024 September 30, 2024 and 2023, adjustments to net product revenue related to performance obligations satisfied in previous periods were immaterial. $0.5 million and $0.4 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

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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 six nine months ended June 30, 2024 September 30, 2024 and 2023 (*in thousands*) (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
FILSPARI				
Tiopronin products				
Total net product sales				

	Three Months Ended June 30,		Six Months Ended June 30,	
	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
FILSPARI				
Tiopronin products				
Total net product sales				

NOTE 4. COLLABORATION AND LICENSE AGREEMENTS

License Agreement with CSL Vifor

On September 15, 2021, the Company entered into a license and collaboration agreement ("CSL Vifor License Agreement") with Vifor (International) Ltd. ("CSL Vifor"), pursuant to which the Company granted an exclusive license to CSL Vifor for the commercialization of sparsentan in Europe, Australia and New Zealand ("CSL Vifor Licensed Territories"). CSL Vifor also has first right of negotiation to expand the licensed territories into Canada, China, Brazil and/ or Mexico. Under the terms of the 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 up to 40 percent of annual net sales of sparsentan in the CSL Vifor Licensed Territories.

Under the CSL Vifor License Agreement, CSL Vifor will be 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 and will retain all rights to sparsentan in the United States and rest of world outside of the CSL Vifor Licensed Territories. Development costs for any post regulatory approval development activities, subject to approval by both parties, will be borne by the Company and CSL Vifor as defined, respectively. The CSL Vifor 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 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") of active participation by both parties and exposures to significant risks and rewards dependent on the commercial success of the activities. Both parties participate on joint steering and other committees overseeing the collaboration activities. Also, both parties are exposed to significant risks and rewards based on the economic outcomes of regulatory approvals and commercialization of sparsentan.

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

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

For the three and six nine months ended June 30, 2024 September 30, 2024, the Company recognized $1.9 million $1.8 million and $3.3 million $5.1 million, respectively, in license and collaboration revenue for clinical development activities, based upon the ratio of costs incurred to total estimated costs. For the three and six nine months ended June 30, 2023 September 30, 2023, the Company recognized $2.7 million $3.2 million and $9.4 million, $9.3 million, respectively, in license and collaboration revenue for clinical development activities, based upon the ratio of costs incurred to total estimated costs. For the nine months ended September 30, 2023, the Company recognized an additional $3.3 million in license and collaboration revenue from the sale of active pharmaceutical ingredients to CSL Vifor at cost plus a margin.

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Deferred revenue related to the clinical development activities as of June 30, 2024 September 30, 2024 was $5.4 million $3.8 million, with the total amount classified as current based upon amounts expected to be realized within the next year. As of December 31, 2023, deferred revenue related to the clinical development activities was $8.9 million, of which $7.1 million was classified as current. The Company estimates that the remainder of the deferred revenue balance will be fully realized by mid-2025.

For the three and nine months ended September 30, 2024, the Company recognized $0.1 million and $0.1 million, respectively, in license and collaboration revenue for royalties earned on net sales of FILSPARI in the CSL Vifor Licensed Territories following the August 2024 launch.

Licensing Agreement with Renalys

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

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

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

The Company determined the transaction price under the Renalys License Agreement totaled $8.3 million, consisting of the fixed non-refundable upfront payment, milestone payment and estimated fair value of the Option Agreement. The variable development-related milestones were excluded from the transaction price given the substantial uncertainty related to their achievement. Sales-based milestone payments and royalties on net sales were excluded from the transaction price and will be recognized at the later of when the related sales occur or when the performance obligation to which the sales-based milestone or royalty has been allocated has been satisfied.

The Company concluded that Renalys represents a customer and applied relevant guidance from ASC 606 to evaluate the accounting under the Renalys License Agreement. In accordance with this guidance, the Company concluded that the promise to grant the license is distinct, resulting in one performance obligation as the license has stand-alone functionally at contract inception. The Buyout Right precludes transferring control of the license to Renalys under ASC 606 and the Company's option to repurchase the common stock at a price greater than the original license premium results in accounting for the Renalys License Agreement as a financing arrangement. The transaction price was recorded in other non-current liabilities, and will be recognized in revenue upon exercise or termination of the Buyout Right.

See Note 6 for further discussion of VIE's.

NOTE 5. MARKETABLE DEBT SECURITIES

The Company's marketable debt securities as of June 30, 2024 September 30, 2024 and December 31, 2023 were composed of available-for-sale commercial paper and corporate and government debt securities. The primary objective of the Company's investment portfolio is to preserve capital and liquidity while enhancing overall returns. The Company's investment policy limits interest-bearing security investments to certain types of instruments issued by institutions with primarily investment grade credit ratings and places restrictions on maturities and concentration by asset class and issuer.

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

	June 30, 2024	December 31, 2023
	September 30, 2024	December 31, 2023
Marketable debt securities:		
Commercial paper		
Commercial paper		
Commercial paper		
Corporate debt securities		
Securities of government sponsored entities		
Total available-for-sale marketable debt securities		

The following is a summary of short-term marketable debt securities classified as available-for-sale as of June 30, 2024 September 30, 2024 (*in thousands*):

Remaining Contractual Maturity (in years)	Remaining Contractual Maturity (in years)	Amortized Cost	Unrealized Gains	Unrealized Losses	Aggregate Estimated Fair Value	Remaining Contractual Maturity (in years)	Amortized Cost	Unrealized Gains	Unrealized Losses	Aggregate Estimated Fair Value

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Marketable debt securities:
Corporate debt securities
Corporate debt securities
Corporate debt securities
Securities of government-sponsored entities
Total maturity less than 1 year
Corporate debt securities
Total maturity 1 to 2 years
Total maturity 1 to 2 years
Total maturity 1 to 2 years
Total available-for-sale marketable debt securities

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

	Remaining Contractual Maturity (in years)	Amortized Cost	Unrealized Gains	Unrealized Losses	Aggregate Estimated Fair Value
Marketable debt securities:					
Commercial paper	Less than 1	$ 34,450	$ 25	$ (17)	$ 34,458
Corporate debt securities	Less than 1	133,463	29	(408)	133,084
Securities of government-sponsored entities	Less than 1	81,334	36	(274)	81,096
Total maturity less than 1 year		249,247	90	(699)	248,638
Corporate debt securities	1 to 2	233,969	1,444	(174)	235,239
Securities of government-sponsored entities	1 to 2	24,718	106	(26)	24,798
Total maturity 1 to 2 years		258,687	1,550	(200)	260,037
Total available-for-sale securities		$ 507,934	$ 1,640	$ (899)	$ 508,675

For the three and six nine months ended June 30, 2024 September 30, 2024 and 2023, realized gains and losses on marketable debt securities were immaterial. As of June 30, 2024 September 30, 2024 and December 31, 2023, the accrued interest receivable related to the Company's marketable debt securities was $2.8 million $2.5 million and $4.6 million, respectively, and was recorded in prepaid expenses and other current assets on the Consolidated Balance Sheets.

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

The following is a summary of available-for-sale marketable debt securities in an unrealized loss position with no credit losses reported as of June 30, 2024 September 30, 2024 (*in thousands*):

		Less Than 12 Months		Less Than 12 Months		12 Months or Greater		Total			Less Than 12 Months		12 Months or Greater		Total	
Description of Securities	Description of Securities	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Description of Securities	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
Corporate debt securities																
Corporate debt securities																
Corporate debt securities																
Securities of government-sponsored entities																
Total																

The following is a summary of available-for-sale marketable debt securities in an unrealized loss position with no credit losses reported as of December 31, 2023 (*in thousands*):

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Description of Securities	Less Than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
Commercial paper	$ 24,798	$ 17	$ —	$ —	$ 24,798	$ 17
Corporate debt securities	140,802	405	28,775	177	169,577	582
Securities of government-sponsored entities	61,933	217	12,540	83	74,473	300
Total	$ 227,533	$ 639	$ 41,315	$ 260	$ 268,848	$ 899

As of June 30, 2024 September 30, 2024 and December 31, 2023, the amortized cost of the available-for-sale marketable debt securities in an unrealized loss position was $252.1 million $57.5 million and $269.7 million, respectively.

As of June 30, 2024 September 30, 2024 and December 31, 2023, the Company does not intend to sell these investments and it is not more likely than not that the Company will be required to sell the investments before recovery of their amortized cost basis. The decrease in unrealized losses for the six nine months ended June 30, 2024 September 30, 2024 was primarily due to fluctuations in short-term interest rates. The Company does not believe the unrealized losses incurred during the period are due to credit-related factors. The credit ratings of the securities held remain of the highest quality. Moreover, the Company continues to receive payments of interest and principal as they become due, and our expectation is that those payments will continue to be received timely. Factors unknown to us at this time may cause actual results to differ and require adjustments to the Company's estimates and assumptions in the future.

NOTE 6. VARIABLE INTEREST ENTITIES

Stock Purchase and Collaboration Agreement with PharmaKrysto

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

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

The upfront payments were expensed to research and development and other income (expense), net upon initial consolidation. The consolidated assets and liabilities as of June 30, 2024 September 30, 2024 and December 31, 2023 were immaterial. The results of operations were not significant for the three and six nine months ended June 30, 2024 September 30, 2024 and 2023. The Company is not required to provide additional funding other than the contractually required amounts disclosed above. The creditors and beneficial holders of PharmaKrysto have no recourse to the general credit or assets of the Company.

Licensing Agreement with Renalys

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

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

As of June 30, 2024 September 30, 2024, the carrying amount of the liabilities related to the Company's variable interests was $8.3 million, recorded in other non-current liabilities in the Company's Consolidated Balance Sheets. The Company's maximum exposure to loss as of June 30, 2024 September 30, 2024 is zero. The Company is not required to provide additional funding. The creditors have no recourse to the general credit or assets of the Company.

NOTE 7. LEASES

As of June 30, 2024 September 30, 2024, the Company had two operating leases, including one operating lease with Kilroy Realty, L.P. (the "Landlord") for office space located in San Diego, California, which was entered into in April 2019 and subsequently amended in May 2020. Coinciding with the Company's ability to direct the use of the office space, which occurred in phases over 2020, and utilizing a discount rate equal to the Company's estimated incremental borrowing rate, the Company established ROU assets totaling $34.6 million and lease liabilities totaling $34.5 million. The total ROU asset and lease liability at measurement were each offset by lease incentives associated with tenant improvement allowances totaling $7.9 million.

The initial term of the office lease ends in August 2028, and the Landlord has granted the Company an option to extend the term of the lease by a period of 5 years. At lease inception, it was not reasonably certain that the Company will extend the term of the lease and therefore the renewal period has been excluded from the aforementioned ROU asset and lease liability measurements. The measurement of the lease term occurs from the February 2021 occupancy date of the office space.

The Company has one operating lease with Esprit Investments Limited for office space located in Dublin, Ireland, which was entered into in October 2022. The initial term of the office lease ends in September 2027. The lease provides the option to extend the term of the lease by a period of 5 years, although at lease inception, it was not reasonably certain that the Company would elect this option and therefore the renewal period was excluded from the initial lease measurement. Utilizing a discount rate equal to the Company's estimated incremental borrowing rate, the Company established an ROU asset and corresponding lease liability of $0.4 million.

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The following is a schedule of the future minimum rental commitments for the Company's operating leases reconciled to the lease liability and ROU asset as of June 30, 2024 September 30, 2024 (*in thousands*):

	June 30, 2024
2024 (remaining six months)	
	September 30, 2024
2024 (remaining three months)	
2025	
2026	
2027	
2028	
Total undiscounted future minimum payments	
Present value discount	
Total lease liability	
Unamortized lease incentives	
Cash payments in excess of straight-line lease expense	
Total ROU asset	

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

		June 30, 2024		December 31, 2023		
		September 30, 2024		December 31, 2023		
Weighted-average remaining lease term in years	Weighted-average remaining lease term in years	4.2	4.7	Weighted-average remaining lease term in years	3.9	4.7
Weighted-average discount rate	Weighted-average discount rate	6.48 %	6.48 %	Weighted-average discount rate	6.48 %	6.48 %

For the three and six nine months ended June 30, 2024 September 30, 2024, the Company recorded $1.2 $1.3 million and $2.4 million $3.7 million, respectively, in expense related to operating leases, including amortized tenant improvement allowances. For the three and six nine months ended June 30, 2023 September 30, 2023, the Company recorded $1.2 million and $2.5 million $3.7 million, respectively, in expense related to operating leases, including amortized tenant improvement allowances.

NOTE 8. FAIR VALUE MEASUREMENTS

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

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

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

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

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

Financial instruments with carrying values approximating fair value include cash and cash equivalents, accounts receivable, and accounts payable, due to their short-term nature. As of June 30, 2024 September 30, 2024, the fair value of the Company's 2.5% Convertible Senior Notes due 2025 was $61.0 million $66.0 million and the fair value of the Company's 2.25% Convertible Senior Notes due 2029 was $201.9 million $272.8 million. As of December 31, 2023, the fair value of the Company's 2.5% Convertible Senior Notes due 2025 was $58.3 million and the fair value of the Company's 2.25% Convertible Senior Notes due 2029 was $212.1 million. The fair values were estimated utilizing market quotations and are considered Level 2.

The following table presents the Company's assets, measured and recognized at fair value on a recurring basis, classified under the appropriate level of the fair value hierarchy as of June 30, 2024 September 30, 2024 (*in thousands*):

	As of June 30, 2024				As of September 30, 2024				
	Total carrying and estimated fair value	**Total carrying and estimated fair value**	**Quoted prices in active markets (Level 1)**	**Significant other observable inputs (Level 2)**	**Significant unobservable inputs (Level 3)**	**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									
Cash and cash equivalents									
Cash and cash equivalents									

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Marketable debt securities, available-for-sale	
Total	

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, 2023 (*in thousands*):

	As of December 31, 2023			
	Total carrying and estimated fair value	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Assets:				
Cash and cash equivalents	$ 58,176	$ 58,176	$ —	$ —
Marketable debt securities, available-for-sale	508,675	—	508,675	—
Total	$ 566,851	$ 58,176	$ 508,675	$ —

NOTE 9. INTANGIBLE ASSETS

Ligand License Agreement

In 2012, the Company entered into an agreement with Ligand Pharmaceuticals, Inc. ("Ligand") for a worldwide sublicense to develop, manufacture and commercialize sparsentan (the "Ligand License Agreement"). As consideration for the license, the Company is required to make substantial payments upon the achievement of certain milestones, totaling up to $114.1 million. Through June 30, 2024 September 30, 2024 the Company has capitalized $47.2 million for contractual milestones achieved under the Ligand License Agreement, including $5.8 million for the three and six nine months ended June 30, 2024 September 30, 2024. Pursuant to the Ligand License Agreement, the Company is obligated to pay to Ligand (and Bristol-Myers Squibb Company ("BMS")) an escalating royalty between 15% and 17% of net sales of sparsentan, with payments due quarterly. The Company began incurring costs associated with such royalties following the February 2023 approval of FILSPARI (sparsentan). For the three and six nine months ended June 30, 2024 September 30, 2024, the Company capitalized $4.1 million $5.4 million and $7.0 million $12.4 million, respectively, to intangible assets for royalties owed on net sales of FILSPARI. The cost of the milestone payments and royalty payments are being amortized to selling, general and administration on a straight-line basis through April 30, 2033.

The following table sets forth amortizable intangible assets as of June 30, 2024 September 30, 2024 and December 31, 2023 (*in thousands*):

	June 30, 2024	December 31, 2023
	September 30, 2024	December 31, 2023
Finite-lived intangible assets		
Less: accumulated amortization		
Net carrying value		

As of June 30, 2024 September 30, 2024 and December 31, 2023, the Company had goodwill of $0.8 million.

The following table summarizes amortization expense for the three and six nine months ended June 30, 2024 September 30, 2024 and 2023 (*in thousands*):

	Three Months Ended June 30,		Six Months Ended June 30,	
	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Research and development				
Selling, general and administrative				
Total amortization expense				

NOTE 10. CONVERTIBLE NOTES PAYABLE

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

	June 30, 2024	December 31, 2023	September 30, 2024	December 31, 2023
2.25% convertible senior notes due 2029				

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2.50% convertible senior notes due 2025	
Unamortized debt issuance costs - 2.25% convertible senior notes due 2029	
Unamortized debt issuance costs - 2.50% convertible senior notes due 2025	
Total convertible senior notes, net of unamortized debt discount and debt issuance costs	

Convertible Senior Notes Due 2029

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

The Company received net proceeds from the issuance of the 2029 Notes of $306.4 million, after deducting commissions and offering expenses of $9.9 million. At June 30, 2024 September 30, 2024, accrued interest on the 2029 Notes of $2.4 $0.6 million is included in accrued expenses in the accompanying Consolidated Balance Sheets. The 2029 Notes comprise the Company’s senior, unsecured obligations and are (i) equal in right of payment with the Company’s existing and future senior, unsecured indebtedness; (ii) senior in right of payment to the Company’s existing and future indebtedness that is expressly subordinated to the 2029 Notes; (iii) effectively subordinated to the Company’s existing and future secured indebtedness, to the extent of the value of the collateral securing that indebtedness; and (iv) structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables.

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

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

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

Convertible Senior Notes Due 2025

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

The net proceeds from the issuance of the 2025 Notes were approximately $267.2 million, after deducting commissions and the offering expenses of $8.8 million payable by the Company. At June 30, 2024 September 30, 2024, accrued interest of $0.5 million $0.1 million is included in accrued expenses in the accompanying Consolidated Balance Sheets. The 2025 Notes comprise the Company’s senior, unsecured obligations and are (i) equal in right of payment with the Company’s existing and future senior, unsecured indebtedness; (ii) senior in right of payment to the Company’s existing and future indebtedness that is expressly subordinated to the 2025 Notes; (iii) effectively subordinated to the Company’s existing and future secured indebtedness, to the extent of the value of the collateral securing that indebtedness; and (iv) structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables.

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

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

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

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

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

On March 11, 2022, the Company completed its repurchase of $207.1 million aggregate principal amount of 2025 Notes for cash, including accrued and unpaid interest, for a total of $213.8 million. This transaction involved a contemporaneous exchange of cash between the Company and holders of the 2025 Notes participating in the issuance of the 2029 Notes. Accordingly, we evaluated the transaction for modification or extinguishment accounting in accordance with ASC 470-50, *Debt – Modifications and Extinguishments* on a creditor-by creditor basis depending on whether the exchange was determined to have substantially different terms. The repurchase of the 2025 Notes and issuance of the 2029 Notes were deemed to have substantially different terms based on the present value of the cash flows or significant difference between the value of the conversion option immediately prior to and after the exchange. Therefore, the repurchase of the 2025 Notes was accounted for as a debt extinguishment. The Company recorded a $7.6 million loss on extinguishment of debt on its Consolidated Statements of Operations for the six nine months ended June 30, 2023 September 30, 2023, which includes the write-off of related deferred financing costs of $3.4 million. After giving effect to the repurchase, the total remaining principal amount outstanding under the 2025 Notes as of June 30, 2024 September 30, 2024 was $68.9 million.

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

The 2025 and 2029 Notes do not contain any financial or operating covenants or any restrictions on the payment of dividends, the issuance of other indebtedness or the issuance or repurchase of securities by the Company. There were no events of default for the 2025 Notes or 2029 Notes at June 30, 2024 September 30, 2024.

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

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

	Three Months Ended June 30,		Six Months Ended June 30,					
	Three Months Ended September 30,		Nine Months Ended September 30,					
	2024	2024	2023	2024	2023	2024	2023	2024	2023
Contractual interest expense									
Amortization of debt issuance costs									
Total interest expense for the 2025 and 2029 Notes									

Total interest expense recognized for the three and six nine months ended June 30, 2024 September 30, 2024 was $2.8 million and $5.6 $8.4 million, respectively. Total interest expense recognized for the three and six nine months ended June 30, 2023 September 30, 2023 was $2.8 million and $5.7 $8.5 million, respectively.

NOTE 11. ACCRUED EXPENSES

Accrued expenses at June 30, 2024 September 30, 2024 and December 31, 2023 consisted of the following (*in thousands*):

	June 30, 2024	December 31, 2023
	September 30, 2024	December 31, 2023
Compensation related costs		
Compensation related costs		
Compensation related costs		
Research and development		
Accrued royalties		
Sales discounts, rebates, and allowances		
Accrued royalties		
Selling, general and administrative		
French rebate accrual		
Transition services accrual		
Accrued restructuring costs		
Transition services accrual		
Miscellaneous accrued expenses		

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Total accrued expenses

NOTE 12. NET LOSS PER COMMON SHARE

Basic and diluted net income (loss) per common share is calculated by dividing net loss applicable to common stockholders by the weighted-average number of common shares outstanding during the period. In accordance with *ASC 260, Earnings per Share*, if a company had a discontinued operation, the company uses income from continuing operations, adjusted for preferred dividend and similar adjustments, as its control number to determine whether potential common shares are dilutive.

As discussed in Note 17, as part of its February 2023 underwritten public offering, the Company issued and sold pre-funded warrants to purchase 1.25 million shares of its common stock at a price to the public of $20.9999 per pre-funded warrant. The pre-funded warrants are were immediately exercisable immediately upon issuance, and are exercisable for one share were exercised in the third quarter of 2024, resulting in the issuance of 1.25 million shares of the Company's common stock. The Due to the nominal exercise price of each the pre-funded warrant is $0.0001 per share of common stock. Since the $0.0001 price per share represents little consideration and is non-substantive in relation to the $20.9999 price per pre-funded warrant warrants and the $21.00 price per share lack of the common stock offered any contingencies to the public, and as the warrants are immediately exercisable with no further vesting conditions or contingencies associated with them, exercise, the shares underlying the pre-funded warrants are therefore have been included in the calculation of basic net loss per common share. share since the date the warrants were issued.

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

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

	Three Months Ended June 30,			Three Months Ended September 30,			Three Months Ended June 30,			Three Months Ended September 30,		
	2024			2024			2023			2024			2023		
	Shares	Net Income (loss)	EPS	Shares	Net Income (loss)	EPS	Shares	Net Income (loss)	EPS	Shares	Net Income (loss)	EPS	Shares	Net Income (loss)	EPS
Continuing operations															
Discontinued operations															
Basic and diluted loss per share															

	Six Months Ended June 30,			Nine Months Ended September 30,			Six Months Ended June 30,			Nine Months Ended September 30,		
	2024			2024			2023			2024			2023		
	Shares	Net Income (loss)	EPS	Shares	Net Income (loss)	EPS	Shares	Net Income (loss)	EPS	Shares	Net Income (loss)	EPS	Shares	Net Income (loss)	EPS
Continuing operations															
Discontinued operations															
Basic and diluted loss per share															

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

	Three Months Ended June 30,				Six Months Ended June 30,			
	Three Months Ended September 30,				Nine Months Ended September 30,			
	2024	2024	2023	2024	2023	2024	2023	2024	2023
Convertible debt									
Options									
Restricted stock									
Total anti-dilutive shares									
Total anti-dilutive shares									
Total anti-dilutive shares									

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NOTE 13. COMMITMENTS AND CONTINGENCIES

Commitments

Certain of the Company's contractual arrangements with contract manufacturing organizations ("CMOs") require binding forecasts or commitments to purchase minimum amounts for the manufacture of drug product supply, which may be material to the Company's financial statements.

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 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 US and Europe, subject to certain reductions as set forth in the Agreement, and a contingent payment in the event a pediatric rare disease voucher for any pegtibatinase product is granted.

Substantially all of the value of the assets acquired was concentrated within pegtibatinase, and as of the acquisition date, the Company did not anticipate any economic benefit to be derived from pegtibatinase other than the primary indication. Accordingly, the transaction was treated as an asset acquisition with amounts charged to in-process research and development (IPR&D) IPR&D expense on the date of acquisition.

In accordance with ASC 450, *Contingencies*, contingent cash payments will be accrued for when it is probable that a liability has been incurred and the amount can be reasonably estimated. In March 2024, the Company recognized $65.2 million in IPR&D expense upon the achievement of a development milestone, which was paid during the second quarter of 2024 and recorded within investing activities in the Consolidated Statements of Cash Flows.

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 six nine months ended June 30, 2024 September 30, 2024:

	Shares Underlying Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (years)	Aggregate Intrinsic Value (in thousands)	Shares Underlying Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2023								
Granted								
Exercised								
Forfeited/canceled								
Outstanding at June 30, 2024								
Vested and expected to vest at June 30, 2024								
Outstanding at September 30, 2024								
Vested and expected to vest at September 30, 2024								

At June 30, 2024 September 30, 2024, unamortized stock compensation for stock options was $23.5 million $19.7 million, with a remaining weighted-average recognition period of 2.7 2.5 years.

At June 30, 2024 September 30, 2024, outstanding options to purchase 7.6 7.2 million shares of common stock were exercisable with a weighted-average exercise price per share of $21.42. $21.61. During the six nine months ended June 30, 2024 September 30, 2024, the Company had forfeitures of 1.2 million 1.9 million shares due primarily to a combination of employee turnover, the December 2023 reorganization.

reorganization and the August 2023 divestiture of the bile acid business.

Restricted Stock Units

Service Based Restricted Stock Units

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The following table summarizes the Company's service based restricted stock unit activity during the six nine months ended June 30, 2024 September 30, 2024:

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Unvested at December 31, 2023				
Granted				
Vested				
Forfeited/canceled				
Unvested at June 30, 2024				
Unvested at September 30, 2024				

At June 30, 2024 September 30, 2024, unamortized stock compensation for service based restricted stock units was $48.0 million $41.4 million, with a remaining weighted-average recognition period of 2.8 2.5 years.

Performance Based Restricted Stock Units

The following table summarizes the Company's performance based restricted stock unit activity during the six nine months ended June 30, 2024 September 30, 2024:

	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value	Number of Restricted Stock Units	Weighted Average Grant Date Fair Value
Unvested at December 31, 2023				
Granted				
Vested				
Forfeited/canceled				
Unvested at June 30, 2024				
Unvested at September 30, 2024				

At June 30, 2024 September 30, 2024, unamortized stock compensation for performance based restricted stock units was $2.2 million $1.3 million, with a remaining weighted-average recognition period of 1.1 1.2 years.

Share-Based Compensation

Total share-based compensation presented in the Consolidated Statements of Stockholders' Equity includes both continuing operations and discontinued operations. The following table sets forth share-based compensation for continuing operations for the three and six nine months ended June 30, 2024 September 30, 2024 and 2023 (*in thousands*):

	Three Months Ended June 30,		Six Months Ended June 30,		Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023	2024	2023	2024	2023
Research and development								
Selling, general and administrative								
Total share-based compensation								

NOTE 15. INVENTORY

Inventory consisted of the following at June 30, 2024 September 30, 2024 and December 31, 2023 (*in thousands*):

	June 30, 2024	December 31, 2023
	September 30, 2024	December 31, 2023
Raw materials		
Work in process		
Finished goods		
Total inventory		
Classified as:		
Inventory		
Inventory		
Inventory		
Long-term inventory		
Total inventory		

The balance classified as long-term inventory consists of raw materials and work in process for FILSPARI as of June 30, 2024 September 30, 2024 and December 31, 2023. The balance classified as long-term inventory as of September 30, 2024 also consists of raw materials and finished goods for tiopronin products. The Company maintains levels of these inventories beyond a one-year production plan to limit exposure to potential supply disruption. Such inventories are classified as long-term.

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NOTE 16. ACCOUNTS RECEIVABLE

Accounts receivable, net of reserves for prompt pay discounts and expected credit losses, was $24.5 million $25.2 million and $21.2 million at June 30, 2024 September 30, 2024 and December 31, 2023, respectively. The total reserves for both periods were immaterial.

The Company's evaluation and accounting for credit losses for the current period included an assessment of our aged trade receivables balances and their underlying credit risk characteristics. Our evaluation of past events, current conditions, and reasonable and supportable forecasts about the future resulted in an expectation of immaterial credit losses.

NOTE 17. EQUITY OFFERINGS

Underwritten Public Offering of Common Stock

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

The pre-funded warrants were classified as a component of permanent stockholders' equity within additional paid-in capital and were recorded at the issuance date using a relative fair value allocation method. The pre-funded warrants are equity classified because they (i) are freestanding financial instruments that are legally detachable and separately exercisable from the equity instruments, (ii) are immediately exercisable, (iii) do not embody an obligation for the Company to repurchase its shares, (iv) permit the holders to receive a fixed number of shares of common stock upon exercise, (v) are indexed to the Company's common stock and (vi) meet the equity classification criteria. In addition, such pre-funded warrants do not provide any guarantee of value or return. The Company valued the pre-funded warrants at issuance, concluding that their sale price approximated their fair value, and allocated the aggregate net proceeds from the sale proportionately to the common stock and prefunded warrants, including approximately $24.6 million allocated to the pre-funded warrants and recorded as a component of additional paid-in capital. The pre-funded warrants were exercised in the third quarter of 2024, resulting in the issuance of 1.25 million shares of the Company's common stock.

At-the-Market Equity Offering

In February 2020, the Company entered into an Open Market Sale Agreement ("ATM Agreement") with Jefferies LLC, as agent ("Jefferies"), pursuant to which the Company may offer and sell, from time to time through Jefferies, shares of its common stock having an aggregate offering price of up to $100.0 million. Of the $100.0 million originally authorized for sale under the ATM Agreement, approximately $28.6 million were sold under the Company's prior registration statement on Form S-3 (Registration No. 333-227182)(the "Prior Registration Statement"). An additional $51.9 million were sold under the Company's effective prior registration statement on Form S-3 (Registration Statement No. 333-259311), which included $20.1 million in the year ended December 31, 2022. The Company did not sell any shares under the ATM Agreement during the year ended December 31, 2023 or during the six nine months ended June 30, 2024 September 30, 2024. As of June 30, 2024, an aggregate of $19.5 million remained eligible for sale under The offering pursuant to the ATM Agreement. Agreement terminated upon the expiration of the Prior Registration Statement on September 3, 2024.

NOTE 18. DIVESTITURES

Discontinued Operations

Sale of Bile Acid Product Portfolio

On August 31, 2023, the Company closed the sale of its bile acid business to Mirum Pharmaceuticals pursuant to the terms of the Purchase Agreement dated July 16, 2023 between the Company and Mirum. The assets sold consisted of substantially all of the assets primarily related to the Company's business of development, manufacture (including synthesis, formulation, finishing or packaging) and commercialization of the products, Chenodal and Cholbam (also known as Kolbam). In connection with the Closing, the Company received an upfront cash payment of $210.0 million.

Pursuant to the Purchase Agreement, after the Closing, 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 the Products. The Company will recognize the contingent consideration receivable in earnings when the target annual sales for the milestones are met and the contingency is resolved.

The Company's sale of the bile acid business resulted in a gain, net of tax, of $226.0 million, which was recognized in 2023. The net gain consists of net consideration, including the upfront payment and the deduction of investment banker fees owed upon Closing, plus the derecognition of the carrying value of the net liabilities included in the transaction and the immaterial tax due on the sale.

The Company and Mirum have also entered into a transition services agreement ("TSA") pursuant to which the Company has agreed to perform certain services for a period of time following the Closing, with respect to Mirum's use and operation of the assets purchased in the Purchase Agreement. The TSA is designed to ensure and facilitate an orderly transfer of business operations, and the consideration to be received by the Company primarily consists of cost reimbursement. For the three and six nine months ended June 30, 2024 September 30, 2024, the Company recognized less than $0.1 million and $0.5 million, respectively, under the TSA, included in continuing operations within other income (expense), net. The uncollected balance is included in accounts receivable of the Consolidated Balance Sheets. As part of the TSA, the Company is collecting certain receivables related to purchased assets for a period of time and remitting them to Mirum. The transition services accrual as of June 30, 2024 September 30, 2024 was $0.3 million $1.2 million, and is included in accrued expenses in the accompanying Consolidated Balance Sheets. TSA services provided by the Company are anticipated to be substantially complete 12 months post-close. as of September 30, 2024.

The Company determined that the divestiture represents a strategic shift that will have a major effect on the Company's operations and financial results, and has therefore reflected the bile acid business as a discontinued operation for all periods presented.

Results of discontinued operations are as follows (*in thousands*):

Three Months Ended June 30,		Six Months Ended June 30,		Three Months Ended September 30,		Nine Months Ended September 30,	
2024	2023	2024	2023	2024	2023	2024	2023

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Net product sales	
Total revenue	
Operating expenses:	
Cost of goods sold	
Cost of goods sold	
Cost of goods sold	
Research and development	
Selling, general and administrative	
Change in fair value of contingent consideration	
Total operating expenses	
Operating (loss) income	
Other income (expenses), net:	
Interest expense	
Interest expense	
Interest expense	
Total other expense, net	
Total other expense, net	
Gain on disposal of discontinued operations, net of tax	
Total other expense, net	
Net (loss) income from discontinued operations	

The Company held no assets or liabilities of discontinued operations as of June 30, 2024 September 30, 2024 and December 31, 2023.

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, 2023 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, 2023, filed with the Securities and Exchange Commission (SEC) on February 20, 2024. 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.

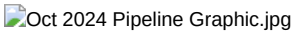
Overview

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 over the last 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 February 17, 2023 September 5, 2024, the FDA granted accelerated full approval of FILSPARI® (sparsentan) to reduce proteinuria slow kidney function decline in adults with primary IgAN who are at risk of rapid disease progression, generally a urine protein-to-creatinine ratio (UPCR) ≥1.5 gram/gram. In September 2023, we announced topline two-year confirmatory secondary endpoint results from the pivotal Phase 3 PROTECT Study and in March 2024, we submitted a supplemental New Drug Application (sNDA) for conversion of the existing U.S. progression. FILSPARI had previously been granted accelerated approval of FILSPARI to full approval, as described below. In May 2024, the FDA accepted the sNDA and granted Priority Review with a PDUFA target action date of September 5, 2024. in February 2023.

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

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

FILSPARI® (sparsentan)

On February 17, 2023 September 5, 2024, the FDA granted accelerated full approval of FILSPARI® (sparsentan) to reduce proteinuria slow kidney function decline in adults with primary IgAN Immunoglobulin A nephropathy (IgAN) who are at risk of rapid disease progression, generally a UPCR ≥1.5 gram/gram. progression. FILSPARI had previously been granted accelerated approval in February 2023 based on the surrogate marker of proteinuria. Full approval is 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 initially became commercially available in the U.S. beginning the week of February 27, 2023, in February 2023 under accelerated approval, and we are providing a comprehensive patient support program throughout the patient's treatment journey.

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This indication was granted under accelerated approval based on reduction in proteinuria. The continued approval of FILSPARI may be contingent upon confirmation of a clinical benefit is the only oral, once-daily, non-immunosuppressive medication that directly targets glomerular injury in the Phase 3 PROTECT Study. As described kidney by blocking two critical pathways of IgAN disease progression (endothelin-1 and angiotensin II).

The two-year efficacy data contained in more detail below, the FDA-approved label is a modified intention to treat (ITT) analysis, and as preferred by the FDA, 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 September 2023, we announced topline 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 confirmatory secondary endpoint measurement period. Additional results from the PROTECT Study and in December 2023, we announced demonstrated the completion of a successful pre-NDA meeting with the FDA for FILSPARI in IgAN. In March 2024, we submitted a supplemental New Drug Application (sNDA) for conversion of the existing U.S. accelerated approval benefit of FILSPARI to full approval. In May 2024, we announced that the FDA has accepted on

absolute eGFR accrued over time and granted Priority Review of the sNDA to convert FILSPARI from accelerated approval to full approval for the treatment of IgAN by Week 110 resulted in a 3.8 mL/min/1.73 m_2 difference in the U.S. The FDA assigned mean change from baseline between FILSPARI and irbesartan.

Results from the PROTECT Study showed that FILSPARI was well tolerated with a PDUFA target action date of September 5, 2024.

FILSPARI, a once-daily, oral medication is designed clearly defined safety profile that has been consistent across all clinical trials conducted to selectively target two critical pathways in the disease progression of IgAN (endothelin 1 and angiotensin-II) and is the first and only non-immunosuppressive therapy approved for the treatment of this condition.

date.

FILSPARI is a dual endothelin angiotensin receptor antagonist (DEARA). Pre-clinical data have shown that blockade of both endothelin type A and angiotensin II type 1 pathways in forms of rare chronic kidney disease, reduces proteinuria, protects podocytes and prevents glomerulosclerosis and mesangial cell proliferation. Sparsentan has been granted Orphan Drug Designation for the treatment of IgAN in the U.S. and the European Economic Area countries (the "EEA") and 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. 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 and only oral, once-daily, non-immunosuppressive therapy approved for this condition. 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 approximately 30,000 to 50,000 over 70,000 patients in the United States to be addressable under FILSPARI's accelerated full approval indication, with the potential to grow with full approval. indication.

Data to support the accelerated 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 ACE or ARB therapy, and is currently ongoing in the open label extension phase of the study.

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

FILSPARI is available only through a risk evaluation and mitigation strategy (REMS) approved by the FDA.

Per request from FDA, regarding mandatory birth control for patients of child-bearing potential regarding risk of embryo-fetal toxicity, as has been required for other approved endothelin antagonists, and a REMS for liver monitoring regarding potential risk of hepatotoxicity, as has been required for certain other approved endothelin antagonists. As part of the liver monitoring REMS, monthly monitoring of each patient is required for the first year the patient is on treatment, and quarterly thereafter. Following engagement with the FDA, the efficacy data contained in the FDA-approved label under accelerated approval is Company has submitted an sNDA for a post-hoc sensitivity analysis that evaluates the first 281 randomized patients, a subset of the full trial population. The mean reduction in proteinuria from baseline in the post-hoc sensitivity analysis is 45% for FILSPARI versus 15% for the active control, irbesartan. Both the pre-specified and post-hoc sensitivity analyses have demonstrated that FILSPARI achieves a rapid and sustained reduction in proteinuria, with statistically significant and clinically meaningful improvement compared potential modification to the active comparator irbesartan.

In September 2023, we announced topline two-year confirmatory secondary endpoint results from the PROTECT Study; eGFR total and chronic slope are the secondary confirmatory endpoints for the U.S. and the EU, respectively. FILSPARI demonstrated long-term kidney function preservation and achieved a clinically meaningful difference in eGFR total and chronic slope versus irbesartan, narrowly missing statistical significance in eGFR total slope while achieving statistical significance in eGFR chronic slope for purposes of regulatory review in the EU. All topline efficacy endpoints favored FILSPARI as compared to irbesartan. A preliminary review of the safety results through 110 weeks of treatment indicates FILSPARI was generally well-tolerated and the overall safety profile in the study has been consistent between treatment groups. In December 2023, we announced the completion of a successful pre-NDA meeting with the FDA for FILSPARI in IgAN. In March 2024, we submitted a supplemental New Drug Application (sNDA) for conversion of the existing U.S. accelerated approval of FILSPARI to full approval. In May 2024, we announced that the FDA has accepted and granted Priority Review of the sNDA to convert FILSPARI from accelerated approval to full approval for the treatment of IgAN in the U.S. The FDA assigned a PDUFA target action date of September 5, 2024. liver-monitoring REMS.

In April 2024, we and our partner CSL Vifor announced that the European Commission has 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). The CMA is granted for all member states of the European Union, as well as in Iceland, Liechtenstein and Norway. The European Commission's decision follows the positive opinion from the Committee for Medicinal Products for Human Use ("CHMP") in February 2024, based on results from the pivotal Phase 3 PROTECT Study of FILSPARI in IgAN. Under the terms of our license agreement with CSL Vifor, we will be entitled to receive a regulatory milestone payment of $17.5 million upon receipt of full regulatory approval by the European Commission for IgAN, and we anticipate receiving an additional milestone payment upon achievement of market access initiatives in certain

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countries. CSL Vifor expects to submit submitted an application for full regulatory approval in mid-2024. The first launch of FILSPARI in Europe is expected in the second half quarter of 2024. The decision on full regulatory approval, if positive, will convert the CMA to a standard Marketing Authorization ("MA"). 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 January 2024, we announced our entry into an exclusive licensing agreement with Renalys Pharma, Inc. ("Renalys"), to bring sparsentan to patients in Japan and other countries in Asia, for the treatment of IgAN. Renalys will hold regional rights to sparsentan for Japan, South Korea, Taiwan, Brunei, Cambodia, Indonesia, Laos, Malaysia, Myanmar, the Philippines, Singapore, Thailand, and Vietnam. Following successful meetings with the Pharmaceuticals and Medical Devices Agency (PMDA) in 2023, in the second quarter of 2024 Renalys initiated an open label registrational study of sparsentan in Japan to support potential approval of sparsentan in Japan. In July 2024, Renalys announced that the first patient was dosed in the study. Results from the urine protein/creatinine ratio (UP/C) endpoint in the study are expected in the second half of 2025 to support a submission for approval to PMDA. Under the terms of the licensing agreement, Renalys will be responsible for development, regulatory matters, and commercialization in the licensed territories.

Clinical-Stage Programs:

Sparsentan for the treatment of FSGS

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

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

In 2016, we generated positive data from our Phase 2 DUET study in FSGS. In 2018, we announced the initiation of the Phase 3 clinical trial designed to serve as the basis for an NDA and MAA filing for sparsentan for the treatment of FSGS (the "DUPLEX Study"). The DUPLEX Study is a global, randomized, multicenter, double-blind, parallel-arm, active-controlled clinical trial evaluating the safety and efficacy of sparsentan in 371 patients. The DUPLEX Study protocol provided for an unblinded analysis of at least 190 patients to be performed after 36 weeks of treatment to evaluate the interim efficacy endpoint - the proportion of patients achieving a FSGS partial remission of proteinuria endpoint (FPRE), which is defined as urine protein-to-creatinine ratio (UPCR) ≤1.5 g/g and a >40% reduction in UPCR from baseline, at week 36. In February 2021, we announced that the ongoing Phase 3 DUPLEX Study achieved its pre-specified interim FSGS partial remission of proteinuria endpoint following the 36-week interim period. After 36 weeks of treatment, 42.0 percent of patients receiving sparsentan achieved FPRE, compared to 26.0 percent of irbesartan-treated patients (p=0.0094). Following engagement with the FDA on the interim proteinuria analysis and a subsequent eGFR data-cut, we elected to forego the previously planned submission for accelerated approval and pursue a potential traditional approval upon completion of the DUPLEX Study.

In May 2023, we announced topline primary efficacy results from the pivotal Phase 3 DUPLEX Study of sparsentan in FSGS. The confirmatory primary endpoint of the DUPLEX Study designed to support traditional regulatory approval was the rate of change in eGFR over 108 weeks of treatment. At the end of the 108-week double-blind period, sparsentan was observed to have a 0.3 mL/min/1.73m$_2$ per year (95% CI: -1.74, 2.41) favorable difference on eGFR total slope and a 0.9 mL/min/1.73m$_2$ per year (95% CI: -1.27, 3.04) favorable difference on eGFR chronic slope compared to the active control irbesartan, which was not statistically significant. After 108 weeks of treatment, sparsentan achieved a mean reduction in proteinuria from baseline of 50%, compared to 32% for irbesartan. Although the DUPLEX Study did not achieve its two-year primary endpoint with statistical significance over the active control irbesartan, we are encouraged by the results, including the secondary endpoints on proteinuria and exploratory endpoints, including renal outcomes, which trended favorably for sparsentan. In addition, a review of the safety results through 108 weeks of treatment indicate sparsentan was generally well-tolerated and the overall safety profile in the study to date was generally consistent between treatment groups.

In December 2023, we announced that we completed our planned Type C meeting with the FDA to discuss results from the Phase 3 DUPLEX Study of sparsentan in FSGS. The FDA acknowledged the high unmet need for approved therapies as well as the challenges in studying FSGS but indicated that the two-year results from the Phase 3 DUPLEX Study alone are were not sufficient to support an sNDA submission. The FDA acknowledged the work being done by the larger nephrology community to better understand proteinuria and eGFR as endpoints in clinical trials of FSGS and indicated a willingness to continue to engage with us on a potential path forward for sparsentan in FSGS following our consideration of additional evidence. Subsequently, a collaborative international effort referred to as the PARASOL project was initiated with a goal to define the quantitative relationships between short-term changes in biomarkers (proteinuria and GFR) and long-term outcomes in order to support the use of alternative proteinuria-based endpoints as a basis for accelerated and traditional approval. The PARASOL project is led by several patient advocacy organizations focused on glomerular diseases, with participation from regulators and industry representatives. Following the recent PARASOL public workshop in which a multi-stakeholder group of rare kidney disease experts aligned around a potential proteinuria-based clinical trial endpoint for FSGS, we have scheduled a Type C meeting with the FDA to discuss a potential regulatory pathway for a sparsentan FSGS indication.

Together with CSL Vifor, we also plan to engage with the EMA to determine the potential for a subsequent variation to the Conditional Marketing Authorization (CMA) of sparsentan for the treatment of FSGS, if the MAA for full approval of sparsentan in IgA nephropathy is approved. Given the high unmet need of FSGS patients, with no medicines currently approved for the condition, and the challenges associated with studying FSGS due to its heterogeneity and other attributes, we are conducting additional analyses of FSGS data and plans to engage with regulators to evaluate potential regulatory pathways for a sparsentan FSGS indication.

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

Pegtibatinase

Pegtibatinase is a novel investigational human enzyme replacement candidate being evaluated for the treatment of classical homocystinuria (HCU). Classical HCU is a rare metabolic disorder characterized by elevated levels of plasma homocysteine that can lead to vision, skeletal, circulatory and central nervous system complications. It is estimated that there are approximately 7,000 to 10,000 people living with HCU 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 1.5mg/kg of pegtibatinase twice weekly (BIW), treatment with pegtibatinase resulted in rapid and sustained reductions in total homocysteine

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(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 2.5mg/kg of lyophilized pegtibatinase or placebo twice weekly (BIW), with four patients assigned to the treatment group. In this highest dose cohort to date, treatment with pegtibatinase resulted in rapid and sustained reductions in total homocysteine (tHcy), with a 67.1% mean relative reduction in tHcy from baseline, as well as maintenance of mean tHcy below the clinically meaningful threshold of 100 µmol, over weeks 6 to 12. In the double-blind period, pegtibatinase was generally well-tolerated, with no discontinuations due to treatment-related adverse events.

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

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

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

Preclinical Programs:

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

Other Commercial Products:

Thiola and Thiola EC (tiopronin)

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

In June 2019 we announced that the FDA approved 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. As of June 30, 2024 September 30, 2024, the FDA has approved three four generic options of Thiola EC (100mg and 300mg). In May 2024 As of September 30, 2024, four generic options for the 100mg and July 2024, generic options 300mg versions of Thiola EC (100mg have been approved by the FDA and 300mg) became three have become available. Accordingly, Thiola EC is subject to generic competition.

Sale of Bile Acid Product Portfolio

On July 16, 2023, we entered into an Asset Purchase Agreement (the “Purchase Agreement”) with Mirum Pharmaceuticals, Inc. ("Mirum Pharmaceuticals" or “Mirum”), pursuant to which Mirum agreed to purchase substantially all of the assets primarily related to our business of development, manufacture (including synthesis, formulation, finishing or packaging) and commercialization of Chenodal and Cholbam (also known as Kolbam, and together with Chenodal, the “Products”), collectively, the "bile acid business". On August 31, 2023, we consummated the transactions contemplated by the Purchase Agreement (the "Closing"). In connection with the Closing, we received an upfront cash payment of $210.0 million. Pursuant to the Purchase Agreement, after the Closing, 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.

A $226.0 million gain, net of tax, was recognized on the transaction as a component of net income from discontinued operations in the Consolidated Statements of Operations. The bile acid business has been classified as a discontinued operation for all periods presented and is excluded from the following discussion of the results of our continuing operations in the results of operations. Refer to Note 18 of our Consolidation Financial Statements for additional information.

Strategic Reorganization

In December 2023, we implemented an approximate 20% workforce reduction focused on non-field-based employees in an effort to align our resources on the ongoing FILSPARI launch and the pivotal Phase 3 HARMONY Study to support the potential approval of pegtibatinase as the first potential disease-modifying treatment for HCU. These restructuring adjustments are expected to result in an estimated annualized savings of approximately $25.0 million beginning in 2024, and an estimated non-recurring charge of approximately $12.0 million to $14.0 million in connection with the restructuring, of which we have recognized a total of $12.3 million $12.4 million as of June 30, 2024 September 30, 2024, including $0.7 million $0.1 million and $0.9 million $1.0 million for the three and six nine months ended June 30, 2024 September 30, 2024, respectively. We expect that we will incur the remaining estimated restructuring costs during the remainder of 2024.

Results of Operations

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Results of operations for the three and six nine months ended June 30, 2024 September 30, 2024 compared to the three and six nine months ended June 30, 2023 September 30, 2023

Unless noted otherwise, the discussion below, and the revenue and expense amounts discussed below, are based on and relate to our continuing operations.

Revenue

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

	Three Months Ended June 30,			Six Months Ended June 30,			Three Months Ended September 30,			Nine Months Ended September 30,		
	2024	2023	Change	2024	2023	Change	2024	2023	Change	2024	2023	Change
FILSPARI												
Tiopronin products												
Total net product revenues												
License and collaboration revenue												
Total revenue												

Net product sales

The increase in total net product revenues for the three and six nine months ended June 30, 2024 September 30, 2024 compared to the three and six nine months ended June 30, 2023 September 30, 2023 was primarily due to growth in sales of FILSPARI, including a full six nine months of sales in 2024, following the February 2023 launch.

License and collaboration revenue

The decrease in license and collaboration revenue for the three months ended June 30, 2024 September 30, 2024 compared to the three months ended June 30, 2023 September 30, 2023 was due to a $0.7 million $1.4 million decrease in collaboration revenue associated with the CSL Vifor License Agreement, which is derived from the performance of clinical development activities and based upon the ratio of costs incurred to total estimated costs. costs, offset by $0.1 million in license and collaboration revenue for royalties earned in 2024 on net sales of FILSPARI in the CSL Vifor Licensed Territories following the August 2024 launch. The decrease in license and collaboration revenue for the six nine months ended June 30, 2024 September 30, 2024 compared to the six nine months ended June 30, 2023 September 30, 2023 was primarily due to a $2.7 million $4.2 million decrease in collaboration revenue associated with the CSL Vifor License Agreement as derived from the performance of clinical development activities and based upon the ratio of costs incurred to total estimated costs, and the $3.3 million sale of active pharmaceutical ingredient to CSL Vifor in March 2023, at cost plus a margin. We estimate that the remainder of the deferred revenue balance associated with these clinical development activities will be fully realized by mid-2025.

Operating Expenses

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

	Three Months Ended June 30,			Six Months Ended June 30,			Three Months Ended September 30,			Nine Months Ended September 30,		
	2024	2023	Change	2024	2023	Change	2024	2023	Change	2024	2023	Change
Cost of goods sold - product sales												
Cost of goods sold - license and collaboration												
Total cost of goods sold												
Research and development												
Selling, general and administrative												
In-process research and development												
Restructuring												
Total operating expenses												

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.

Prior to the February 2023 FDA accelerated approval of FILSPARI (sparsentan), we expensed the production of active pharmaceutical ingredients purchased to support the commercial launch of FILSPARI, in research and development expenses. For the three and six nine months ended June 30, 2024 September 30, 2024 and 2023, sales of FILSPARI primarily consisted of zero-cost inventories, and therefore cost of goods sold did not increase proportionally to the increase in product sales. As of June 30, 2024 September 30, 2024, we had $3.5 $2.6 million of zero-cost inventory remaining. We expect to continue to record zero cost of goods sold on the sale of previously expensed inventories through at least 2025. We began capitalizing inventory costs associated with FILSPARI following the February 2023 accelerated approval.

For the six nine months ended June 30, 2024 September 30, 2024 as compared to the six nine months ended June 30, 2023 September 30, 2023, our cost of goods sold - license and collaboration decreased by $2.9 million, primarily due to the sale of active pharmaceutical ingredients to CSL Vifor in the first quarter of 2023.

Research and development expenses

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

We currently have one Phase 1/2 clinical trial, two Phase 2 clinical trials and three four Phase 3 clinical trials in process that are in various stages of activity, with ongoing non-clinical support trials. As such, clinical trial expenses will vary depending on the 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 June 30,			Six Months Ended June 30,			Three Months Ended September 30,			Nine Months Ended September 30,		
	2024	2023	Change	2024	2023	Change	2024	2023	Change	2024	2023	Change
External service provider costs:												
Sparsentan												
Sparsentan												
Sparsentan												
Pegtibatinase												
General and other product candidates												
Total external service provider costs												
Internal personnel costs												
Total research and development												

For the three and six nine months ended June 30, 2024 September 30, 2024 as compared to the three and six nine months ended June 30, 2023 September 30, 2023, our research and development expenses decreased by $12.2 million $8.9 million and $20.9 million $29.8 million, respectively. Internal personnel costs to support all programs decreased by $3.7 million $4.0 million and $6.6 million $10.9 million, respectively, primarily as a result of our restructuring initiatives. External service provider costs decreased by $8.5 million $4.9 million and $14.3 million $19.0 million, respectively, which was largely driven by a decrease in costs associated with the development of sparsentan as our Phase 3 programs advance towards completion. completion, offset by an increase in costs associated with the development of pegtibatinase following the December 2023 initiation of the Phase 3 HARMONY Study. In September 2024, we announced a voluntary pause of enrollment in the Phase 3 HARMONY Study to enable us to work to address necessary process improvements in manufacturing scale-up to support commercial scale manufacturing as well as full enrollment in the HARMONY Study. As a result of this pause, certain previously planned investments related to clinical enrollment in HARMONY and large-scale production are expected to be delayed beyond 2025. Together with the expected decline in costs associated with the development of sparsentan as the Phase 3 programs advance towards completion, we expect that research and development expenses will be reduced in 2025 compared to 2024.

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 six nine months ended June 30, 2024 September 30, 2024 as compared to the three and six nine months ended June 30, 2023 September 30, 2023, our selling, general and administrative expenses decreased by $3.4 $2.2 million and $5.2 million $7.3 million, respectively, primarily as a result of the restructuring and other cost saving initiatives.

IPR&D expense

In March 2024, we recognized $65.2 million in IPR&D in-process research and development (IPR&D) expense upon the achievement of a development milestone associated with our treatment candidate pegtibatinase, which we acquired as part of the November 2020 acquisition of Orphan Technologies Limited.

Restructuring expenses

In December 2023, we implemented an approximate 20% workforce reduction focused on non-field-based employees in an effort to align our resources on the ongoing FILSPARI launch and the pivotal Phase 3 HARMONY Study to support the potential approval of pegtibatinase as the first potential disease-modifying treatment for HCU. These restructuring adjustments were expected to result in an estimated non-recurring charge of approximately $12.0 million to $14.0 million, the majority of which was recognized in the fourth quarter of 2023. Of the $12.3 million $12.4 million recognized to date, $0.7 million $0.1 million and $0.9 million $1.0 million was recognized during the three and six nine months ended June 30, 2024 September 30, 2024, respectively. We expect that we will incur the remaining estimated restructuring costs during 2024.

Other Income/Expenses

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

	Three Months Ended June 30,			Six Months Ended June 30,			Three Months Ended September 30,			Nine Months Ended September 30,		
	2024	2023	Change	2024	2023	Change	2024	2023	Change	2024	2023	Change
Interest income												
Interest expense												
Other (expense) income, net												
Total other (expense) income, net												

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Other income (expense), net

Total other income, net

The change in our total other income, (expense) net for the three and six nine months ended June 30, 2024 September 30, 2024 as compared to the three and six nine months ended June 30, 2023 September 30, 2023, is partly attributable to changes in interest income, driven by a decrease in the overall balance of interest-bearing security investments held along with fluctuations in short-term interest rates on our interest-bearing security those investments. For the three and six nine months ended June 30, 2024 September 30, 2024, we recognized $3.4 million in other expense in connection with our equity investment in Renalys, for basis adjustments related to the difference in basis between the carrying value and fair value of our proportionate share of the investee's net assets. IPR&D as measured at inception.

Discontinued Operations

Results of discontinued operations are as follows (*in thousands*):

	Three Months Ended June 30,			Six Months Ended June 30,								
	Three Months Ended September 30,			Nine Months Ended September 30,								
	2024	2024	2023	Change	2024	2023	Change	2024	2023	Change	2024	2023	Change
(Loss) income from discontinued operations, net of tax													

The change in net (loss) income from discontinued operations for the three and six nine months ended June 30, 2024 September 30, 2024 compared to the three and six nine months ended June 30, 2023 September 30, 2023 is due to the August 2023 divestiture of our bile acid business. business, which resulted in a gain, net of tax, of $226.0 million.

See Note 18 to our unaudited Consolidated Financial Statements for further discussion.

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 recent commercial launch of FILSPARI in the United States for IgAN. In December 2023, we implemented an approximate 20% workforce reduction focused on non-field-based employees in an effort to align our resources on the ongoing FILSPARI launch and the pivotal Phase 3 HARMONY Study to support the potential approval of pegtibatinase as the first potential disease-modifying treatment for HCU. These restructuring adjustments are expected to result in an estimated annualized savings of approximately $25.0 million in 2024.

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

We had the following balances at June 30, 2024 September 30, 2024 and December 31, 2023 (*in thousands*):

	June 30, 2024	December 31, 2023
	September 30, 2024	December 31, 2023
Cash and cash equivalents		
Marketable debt securities, at fair value		
Convertible debt		
Accumulated deficit		
Stockholders' equity		
Stockholders' (deficit) equity		
Net working capital*		
Net working capital ratio**		

* Current assets less current liabilities.

**Current assets divided by current liabilities.

As of June 30, 2024 September 30, 2024, we had cash and cash equivalents of $32.3 million $36.4 million and available-for-sale marketable debt securities of $293.1 million $241.0 million. Substantial sources of funds since the beginning of 2023, as summarized further below, include an upfront cash payment of $210.0 million in connection with the sale of our bile acid product portfolio, and net proceeds of $215.8 million from an underwritten public offering of our common stock and pre-funded warrants to purchase our common stock.

Over the next 12 months, our expected financial obligations include, but are not limited to, funding our operations, operating lease payments, interest payments on our outstanding debt, 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 launch of FILSPARI. FILSPARI and the repayment of principal on the outstanding 2025 Notes, which mature on September 15, 2025. Sources of cash over this period include net revenues from sales of our products, the sale or maturity of investments in our portfolio of marketable debt securities, and certain earned and potential milestone payments. We anticipate achieving milestones with FILSPARI that will result in us receiving payments of approximately $17.5 million during the next 12 months.

Beyond the next 12 months and over the foreseeable future, our known commitments and potential financial obligations will likely include ongoing operations funding, operating lease payments, interest payments on our outstanding debt, royalties on sales of our existing commercialized products, research and development expenses pertaining to clinical and preclinical development activities across our pipeline, milestone and royalty payments associated with FILSPARI, pegtibatinase, and other developmental programs based upon the achievement of certain agreement-specific criteria, along with sales-based royalties and the repayment of principal on the outstanding 2025 Notes and 2029 Notes, upon their respective maturities. which mature on September 1, 2029. Potential sources of

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cash over this time horizon may include net revenues from sales of our existing products and, if commercialized, our pipeline products, licensing revenue, the sale or maturity of marketable debt securities in our investment portfolio, the refinancing of all or a portion of our debt, including the 2025 Notes and 2029 Notes, on or before maturity, or the issuance of additional debt or equity. In addition, depending on prevailing market conditions, our liquidity requirements, contractual restrictions, and other factors, we may also from time to time seek to retire or purchase our outstanding debt including the 2025 Notes or 2029 Notes, through cash purchases and/or exchanges for equity securities, in open market purchases, privately negotiated transactions or otherwise. 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

On July 16, 2023, we entered into the Purchase Agreement with Mirum, pursuant to which Mirum agreed to purchase substantially all of the assets primarily related to our business of development, manufacture and commercialization of the Products, which comprised our bile acid business. Upon the Closing of the transaction on August 31, 2023, we received an upfront cash payment of $210.0 million. Pursuant to the Purchase Agreement, we are eligible to receive up to an additional $235.0 million upon the achievement of certain milestones based on specified amounts of annual net sales (tiered from $125.0 million to $500.0 million) of the Products.

Collaboration and License Proceeds

License and Collaboration Agreement with CSL Vifor

In September 2021, we entered into a license agreement with CSL Vifor, pursuant to which we granted an exclusive license to CSL Vifor for the commercialization of sparsentan 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. 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.

We have earned $0.1 million in royalties on net sales of FILSPARI in the CSL Vifor Licensed Territories since the August 2024 launch.

Licensing Agreement with Renalys

In January 2024, our license agreement with Renalys Pharma, Inc. came into effect. Under the terms of the license agreement, we granted an exclusive license to Renalys for the commercialization of sparsentan in Japan and other countries in Asia. Pursuant to the terms of the agreement, we are eligible to receive up to $120.0 million in 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.

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

Equity Offerings

2023 Underwritten Public Offering of Common Stock

In February 2023, we sold an aggregate of approximately 9.7 million shares of our common stock and pre-funded warrants to purchase 1.25 million shares of our common stock in an underwritten public offering, at a price to the public of $21.00 per share of common stock and $20.9999 per prefunded warrant. The pre-funded warrants are exercisable immediately, subject to certain beneficial ownership limitations which can be modified by the respective holders with at least 61 days' notice, and are exercisable for one share of our common stock. The exercise price of each pre-funded warrant is $0.0001 per share of common stock. The net proceeds to us from the offering, after deducting the underwriting discounts and offering expenses, were approximately $215.8 million. The pre-funded warrants were exercised in September 2024, resulting in the issuance of 1.25 million shares of the Company's common stock.

At-the-Market Equity Offering

In February 2020, On October 31, 2024, we entered into an Amended and Restated Open Market Sale Agreement ("ATM Agreement") with Jefferies LLC, as agent ("Jefferies"), pursuant to which we may offer and sell, from time to time through Jefferies, shares of our common stock, having pursuant to a prospectus or a prospectus supplement under an aggregate offering price of up to $100.0 million. Of the $100.0 million originally authorized for sale under the ATM Agreement, approximately $28.6 million were sold under our prior registration statement on Form S-3 (Registration No. 333-227182). An additional $51.9 million were sold under our effective registration statement on Form S-3 (Registration Statement No. that we may file and that relates to an "at-the-market offering" of our common stock pursuant to the ATM Agreement.

333-259311), which included $20.1 million in the year ended December 31, 2022. We did not sell any shares under the ATM Agreement during the year ended December 2023 or the six months ended June 30, 2024. As of June 30, 2024, an aggregate of $19.5 million remained eligible for sale under the ATM Agreement.

Operating Leases

Future Minimum Rental Commitments

As of June 30, 2024 September 30, 2024, we have future minimum rental commitments totaling $28.7 million $27.1 million arising from our operating leases. These commitments represent the aggregate base rent through August 2028.

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

Purchase Commitments

Manufactured Product

Certain of 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 sparsentan (the "Ligand License Agreement"). As consideration for the license, we are required to make substantial payments upon the achievement of certain milestones, totaling up to $114.1 million. Through June 30, 2024 September 30, 2024, we have paid $47.2 million for contractual milestones achieved under the Ligand License Agreement, which includes a $23.0 million milestone payment to Ligand (and Bristol-Myers Squibb Company ("BMS")) in March 2023 that was triggered upon the accelerated approval of FILSPARI in February 2023, and a $5.8 million regulatory milestone in the second quarter of 2024. Following commercialization of sparsentan or any products containing related compounds, we will be obligated to pay to Ligand an escalating annual royalty between 15% and 17% of net sales

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of all such products, with payments due quarterly. We began incurring costs associated with such royalties following the February 2023 approval of FILSPARI (sparsentan). For the three and six nine months ended June 30, 2024 September 30, 2024, we capitalized $4.1 million $5.4 million and $7.0 million $12.4 million, respectively, to intangible assets for royalties owed on net sales 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 sparsentan 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.

Thiola License Agreement

In 2014, we entered into a license agreement with Mission Pharmacal ("Mission"), pursuant to which we obtained an exclusive, royalty-bearing license to market, sell and commercialize Thiola (tiopronin) in the United States and Canada, and a non-exclusive license to use know-how relating to Thiola to the extent necessary to market Thiola ("Thiola License Agreement"). Under the terms of the Thiola 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 2 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 (TVT-058). 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 US and Europe, subject to certain reductions as set forth in the Agreement, and a contingent payment in the event a pediatric rare disease voucher for any pegtibatinase product is granted. We made a $65.0 million payment to Orphan in the second quarter of 2024 following the achievement of a development milestone.

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

Stock Purchase and Collaboration Agreement with PharmaKrysto

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

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 $6.3 million (€5.6 million) that will be paid during November 2024. We have appealed the pricing decision and will pursue an appeal of the amount owed with the Competent Administrative Court. As of September 30, 2024 and December 31, 2023, $6.3 million and $5.4 million, respectively, were recorded in Accrued Expenses in the Consolidated Balance Sheets.

Borrowings

Convertible Senior Notes Due 2029

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

Convertible Senior Notes Due 2025

On September 10, 2018, we completed a registered underwritten public offering of $276.0 million aggregate principal amount of 2.50% Convertible Senior Notes due 2025 ("2025 Notes"), and entered into a base indenture and supplemental indenture agreement (collectively, the "2025 Indenture") with respect to the 2025 Notes. The 2025 Notes will mature on September 15, 2025, unless earlier repurchased, redeemed, or converted. The 2025 Notes are senior unsecured obligations of the Company and bear interest at an annual rate of 2.50%, payable semi-annually in arrears on March 15 and September 15 of each year, beginning on March 15, 2019. On March 11, 2022, coinciding with the issuance of the 2029 Notes, we completed our repurchase of $207.1 million aggregate principal amount of 2025 Notes for cash. After giving effect to the repurchase, the total remaining principal amount outstanding under the 2025 Notes as of June 30, 2024 September 30, 2024 was $68.9 million. The 2025 Notes do not contain any financial or operating covenants or any restrictions on the payment of dividends, the issuance of other indebtedness or the issuance or repurchase of securities by us.

Funding Requirements

We believe that our available cash and short-term investments as of the date of this filing will be sufficient to fund our anticipated level of operations beyond the next 12 months. 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;

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- the timing and outcome of, and costs involved in, seeking and obtaining marketing approvals for our products, and in maintaining quality systems standards for our products;
- the timing of, and costs involved in, commercial activities, including product marketing, sales and distribution;
- our ability to successfully commercialize FILSPARI for IgAN, to obtain full regulatory approval for, and successfully commercialize FILSPARI for the treatment of IgAN, and to obtain regulatory approval for, and successfully commercialize, sparsentan for FSGS and our other or future product candidates;
- increases or decreases in revenue from our marketed products, including decreases in revenue resulting from generic entrants or health epidemics or pandemics;
- debt service obligations on the 2025 Notes and 2029 Notes;
- the number and development requirements of other product candidates that we pursue;
- our ability to manufacture sufficient quantities of our products to meet expected demand;
- the costs of preparing, filing, prosecuting, maintaining and enforcing any patent claims and other intellectual property rights, litigation costs and the results of litigation;
- our ability to enter into collaboration, licensing or distribution arrangements and the terms and timing of these arrangements;
- the potential need to expand our business, resulting in additional payroll and other overhead expenses;
- the potential in-licensing of other products or technologies;

- the emergence of competing technologies or other adverse market or technological developments; and
- the impacts of inflation and resulting cost increases.

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

Cash Flows from Continuing Operations

Cash Flows from Operating Activities

Cash used in operating activities from continuing operations for the six nine months ended June 30, 2024 September 30, 2024 was $158.8 million $201.4 million compared to cash used of $186.6 million $261.0 million for the six nine months ended June 30, 2023 September 30, 2023. The decrease in cash used was due to a decrease in operational spending as a result of the restructuring plan initiated in December 2023.

Cash Flows from Investing Activities

Cash provided by investing activities from continuing operations for the six nine months ended June 30, 2024 September 30, 2024 was $133.5 million $179.6 million compared to cash used of $60.6 million $133.4 million for the six nine months ended June 30, 2023 September 30, 2023. The change was due to a decrease in net purchases of marketable debt securities, offset by a $65.0 million payment to Orphan in the second quarter of 2024 following the achievement of a development milestone.

Cash Flows from Financing Activities

Cash provided by financing activities from continuing operations for the six nine months ended June 30, 2024 September 30, 2024 was $0.3 million compared to cash provided of $220.1 million $219.8 million for the six nine months ended June 30, 2023 September 30, 2023. The change was due to the March 2023 issuance of common stock and pre-funded warrants through an underwritten public offering that provided $215.8 million.

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, 2023 for information about critical accounting estimates as well as a description of our other significant accounting policies.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Our primary exposure to market risk is related to changes in interest rates. As of June 30, 2024 September 30, 2024, we had cash equivalents and marketable debt securities of approximately $325.4 million $277.4 million, consisting of money market funds, U.S. government agency debt, corporate debt and commercial paper. This exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments are in short-term debt securities. Our marketable debt securities are subject to interest rate risk and will fall in value if market interest rates continue to increase. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, a change in interest rates of 100 basis points would have approximately a $1.9 million $1.2 million impact on the fair value of our investments.

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The marketable debt securities held in our investment portfolio may subject us to credit risk, though our investment policy limits interest-bearing security investments to certain types of instruments issued by institutions with primarily investment grade credit ratings and places restrictions on maturities and concentration by asset class and issuer. Given these policy restrictions and our emphasis on preserving capital and liquidity while enhancing overall returns, we have not experienced material credit-related losses with our securities holdings.

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

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

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in our reports required by the Securities Exchange Act of 1934, as amended ("Exchange Act"), is recorded, processed, summarized and reported within the timelines specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, management recognized that any controls and procedures, no matter how well designed and operated, can only provide reasonable assurance of achieving the desired control objectives, and in reaching a reasonable level of assurance, management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

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

Changes in Internal Control over Financial Reporting

An evaluation was also performed under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, of any change to our internal control over financial reporting that occurred during the quarter covered by this report and that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting. Our evaluation did not identify any change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Securities Exchange Act of 1934) that occurred during the quarter ended June 30, 2024 September 30, 2024 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, 2023 as filed with the Securities and Exchange Commission (SEC) on February 20, 2024.

Risks Related to the Commercialization of Our Products

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

Our ability to generate significant product revenues and to achieve commercial success in the near-term will depend almost entirely on our ability to successfully commercialize our products in the United States, including FILSPARI (sparsentan) to reduce proteinuria slow kidney function decline in adults with primary IgAN who are at risk of rapid disease progression, which was approved granted full approval by the FDA in September 2024.FILSPARI had previously been granted accelerated approval in February 2023 under based on the FDA's accelerated approval regulations.

surrogate marker of proteinuria.As a product for a rare disease that had no previously-approved non-immunosuppressive treatment, the successful launch and commercialization of FILSPARI is subject to many risks. There are numerous examples of unsuccessful product launches and failures to meet high expectations of market potential, including by pharmaceutical companies with more experience and resources than we have. While we have established our commercial team and U.S. sales force, we will need to continue to train and further develop the team in order to successfully coordinate the ongoing launch and commercialization of FILSPARI in the United States. There are many factors that could cause the launch and commercialization of FILSPARI to be unsuccessful, including a number of factors that are outside our control. Because no non-immunosuppressive product has had previously been approved by the FDA for the treatment of IgAN, it is difficult to estimate FILSPARI's market potential or the time it will take to increase patient and physician awareness of FILSPARI and change current treatment paradigms.

In September 2023, we announced topline two-year confirmatory secondary endpoint results from the PROTECT Study. While FILSPARI demonstrated long-term kidney function preservation and achieved a clinically meaningful difference in estimated glomerular filtration rate (eGFR) total and chronic slope versus irbesartan as well as statistical significance in eGFR chronic slope for purposes of

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regulatory review in the EU, the PROTECT Study narrowly missed statistical significance in eGFR total slope, which was the pre-specified confirmatory endpoint in the U.S. In December 2023, we announced the completion of a successful pre-NDA meeting with the FDA for FILSPARI in IgAN. In March 2024, we submitted a supplemental New Drug Application (sNDA) for conversion of the existing U.S. accelerated approval of FILSPARI to full approval. In May 2024, we announced that the FDA has accepted and granted Priority Review of the sNDA to convert FILSPARI from accelerated approval to full approval for the treatment of IgAN in the U.S. The FDA assigned a PDUFA target action date of September 5, 2024. However, there is no guarantee that the FDA will provide a decision on the application by the target action date, that the FDA will not raise additional requirements prior to acting on the application for approval, that the FDA's accelerated approval of FILSPARI will continue, or that FILSPARI will receive full approval for the treatment of IgAN. Further, if the FDA grants full approval of FILSPARI for the treatment of IgAN, there is no guarantee that the FDA will approve an expanded label. The commercial success of FILSPARI depends on the extent to which patients and physicians accept and adopt FILSPARI for IgAN patients. For example, if the addressable patient population suffering from primary IgAN is smaller than we estimate, if it proves difficult to educate physicians as to the availability and potential benefits of FILSPARI, or if physicians are unwilling to prescribe or patients are unwilling to take FILSPARI, the commercial potential of FILSPARI will be limited. We also do not know how physicians, patients and payers will respond to the pricing of FILSPARI, the confirmatory endpoint data from the Phase 3 PROTECT Study, the results of our ongoing interactions with regulators, updated, full approval label, clinical practice guidelines and any future publications. changes thereto, and any future publications in an evolving treatment landscape. Physicians may not prescribe FILSPARI and patients may be unwilling to use FILSPARI if coverage is not provided or reimbursement is inadequate to cover a significant portion of the cost. Thus, significant uncertainty remains regarding the commercial potential of FILSPARI. If the launch or commercialization of FILSPARI is unsuccessful or perceived as disappointing, the price of our common stock could decline significantly and long-term success of the product and our company could be harmed.

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

In order to successfully commercialize our products in the United States, we have built a specialized sales force. In order to successfully commercialize any approved products, we must continue to build our sales, marketing, distribution, managerial and other non-technical capabilities. Factors that may hinder our ability to successfully market and commercially distribute our products include:

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

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

We have granted exclusive licenses to third parties for the commercialization of sparsentan in certain territories outside of the United States, including Europe, Australia, New Zealand, Japan, South Korea, Taiwan and the ASEAN member states. 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 and the ASEAN member states. 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. We do not currently know what effect, if any, this acquisition will ultimately have on our relationship with CSL Vifor. While our agreement with CSL Vifor remains in place following the acquisition, there is no guarantee that our collaboration with CSL Vifor will not be affected, adversely or otherwise, by the change in ownership. Moreover, in connection with the acquisition of CSL Vifor and related restructuring, substantially less resources could be devoted to the commercialization of sparsentan in the territories licensed to CSL Vifor, or such efforts could be discontinued entirely. If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell sparsentan in territories outside of the United States, if approved, our ability to generate product revenue outside of the United States may be limited.

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

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

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

We face substantial generic and other competition, and our operating results will suffer if we fail to compete effectively.*

Under the Hatch-Waxman Amendments of the Federal Food, Drug, and Cosmetic Act, a pharmaceutical manufacturer may file an ANDA seeking approval of a generic copy of an approved innovator product or an NDA under Section 505(b)(2) that relies on the FDA's prior findings of safety and effectiveness in approving the innovator product. A Section 505(b)(2) NDA may be for a new or improved version of the original innovator product. Our product Thiola, and products from which we may receive milestone payments including Cholbam and Chenodal, are subject to immediate competition from compounded and generic entrants, as the ANDA and/or NDA for these drug products have no remaining or current patent or non-patent exclusivity. In April 2021, a generic option for the 100mg version of the original formulation of Thiola (tiopronin tablets) was approved by the FDA and an additional generic option of the original formulation of Thiola (tiopronin tablets) was approved in June 2022 and during the year ended December 31, 2022, we experienced a decrease in total net product revenues compared to the year ended December 31, 2021, which was due in part to competition from generic tiopronin tablets (100mg version of the original formulation). Additional generic versions of Thiola may be approved in the future. In February 2023, August 2023, January 2024 and July 2024,

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generic versions of Thiola EC (100mg and 300mg) were approved by the FDA. 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 and incentives designed to spur generic competition of branded drugs. In particular, the FDA and the U.S. Federal Trade Commission ("FTC") have been focused on brand companies' denial of drug supply to potential generic competitors for testing. In December 2019, the CREATES Act was enacted, which provides a legislatively defined private right of action under which generic companies can bring suit against companies who refuse access to product for the bioequivalence testing needed to support approval of a generic product.

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

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

If additional generic versions of Thiola or Thiola EC, any generic versions of FILSPARI following the expiration of patent or regulatory exclusivity for the product, or generic versions of any other current or future products are approved, sales of that product likely would be negatively impacted, which could have a material adverse impact on our revenue and profitability. If generic versions of Cholbam or Chenodal are approved, our potential to receive milestone payments from the sale of our bile acid product portfolio may be negatively impacted. In addition, the defense of litigation and response to investigation requests could result in substantial costs, reputational impact, and the diversion of management attention and resources.

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

In October 2022, our licensor, Mission Pharmacal Company, was granted a patent covering the treatment of cystinuria by administering Thiola EC with food (US Patent No. 11,458,104, "the '104 patent") and listed this patent in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the "Orange Book"). Following Mission's listing of the '104 patent in the Orange Book, and as of December 31, 2023, Mission has received three paragraph IV notice letters from three generic manufacturers notifying Mission that each has filed an ANDA seeking approval of a proposed generic version of Thiola EC (tiopronin) 100mg and 300mg oral tablets before expiration of the '104 patent and asserting that the '104 patent is not infringed and/or is invalid, with each such ANDA having been filed prior to the granting and listing of the '104 patent. Under our agreement with Mission, we have the right to enforce the '104 patent. We and Mission entered into agreements with each of the two Paragraph IV filers that had received approval by the FDA in order to settle patent invalidity and infringement disputes related to the '104 patent and providing for a license entry date of April 1, 2026 for their generic versions of Thiola EC (100mg and 300mg), or earlier under certain circumstances. In May 2024 a As of September 30, 2024, four generic manufacturer announced options for the launch of a generic version 100mg and 300mg versions of Thiola EC (100mg have been approved by the FDA and 300mg), and in July 2024 a second manufacturer announced the launch of a second generic version of three have become available. Accordingly, Thiola EC (100mg and 300mg). Both generic versions are available, and Thiola EC is thus subject to generic competition.

There is no guarantee that the '104 patent will withstand any challenge at the Patent and Trademark Office or in litigation, if initiated. Patent litigation is expensive and time consuming, requires significant resources, may absorb significant time of our management and has an unpredictable outcome. If we determine not to pursue patent litigation or the patent is not upheld in litigation or administrative review or if a generic competitor is found not to infringe this patent, the resulting generic competition will likely negatively affect our business, financial condition and results of operations. Thiola EC is subject to generic competition.

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

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

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

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.

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

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

Outside the United States, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies. In some countries, particularly EU Member States and EFTA countries, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time (6 to 12 months or longer) after the receipt of marketing approval for a product. The EU provides options for EU Member States to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. An EU Member State may approve a specific price for the medicinal product, it may refuse to reimburse a product at the price set by the manufacturer or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. Many EU Member States also periodically review their reimbursement procedures for medicinal products, which could have an adverse impact on reimbursement status.

Moreover, to obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost effectiveness of our product candidate to other available therapies. This Health Technology Assessment ("HTA") of medicinal products is becoming an increasingly common part of the pricing and reimbursement procedures in some EU Member States, including those representing the larger markets. The HTA process is the procedure to assess therapeutic, economic and societal impact of a given medicinal product in the national healthcare systems of the individual country. The outcome of an HTA will often influence the pricing and reimbursement status granted to these medicinal products by the competent authorities of individual EU Member States. The extent to which pricing and reimbursement decisions are influenced by the HTA of the specific medicinal product currently varies between EU Member States. In December 2021, Regulation No 2021/2282 on HTA amending Directive 2011/24/EU, was adopted in the EU. This Regulation, which entered into force in January 2022 and will apply as of January 2025, 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 foresees a three-year transitional period and will permit EU Member States to use common HTA tools, methodologies, and procedures across the EU, working together in four main areas, including joint clinical assessment of the innovative health technologies with the most potential impact for patients, joint scientific consultations whereby developers can seek advice from HTA authorities, identification of emerging health technologies to identify promising technologies early, and continuing voluntary cooperation in other areas. Individual EU Member States will continue to be responsible for assessing non-clinical (e.g., economic, social, ethical) aspects of health technologies, and making decisions on pricing and reimbursement. If we or our partners are unable to maintain favorable pricing and reimbursement status in EU Member States for product 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 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. Based on In October 2024, we received an invoice from the ongoing review process, government authority for approximately €5.6 million (approximately $6.3 million). We expect to pay this amount while we expect that we will need to repay pursue an appeal of the amounts being asserted during decision and the appeal process, which we currently estimate to be approximately $6 million. amount owed. 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, generally and has been granted a urinary protein-to-creatinine ratio ("UPCR") ≥1.5 gram/gram. separate seven years of Orphan Drug Exclusivity in the U.S. (running from the date of full approval) to slow kidney function decline in adults with primary IgAN who are at risk for disease progression, excluding the use provided for in the aforementioned Orphan Drug Exclusivity granted in connection with the accelerated approval. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, that product is entitled to a period of marketing exclusivity, which precludes the applicable regulatory authority from approving another marketing application for the same drug for the same indication for that time period. The applicable period is seven years in the United States and ten years in the EU or, in the case of orphan drugs for which a pediatric investigation plan has been completed, 12 years. Even though we have been awarded orphan drug designation in the United States and the EU for sparsentan for the treatment of IgAN and FSGS and for pegtibatinase for the treatment of HCU, we may not be able to maintain it in the EU and the orphan drug designation may not result in orphan drug exclusivity in the United States for FSGS or the EU if approved. For example, if a competitive product that contains the same active moiety and treats the same disease as our product is shown to be clinically superior to our product, any orphan drug exclusivity we have obtained will not block the approval of such competitive product and we may effectively lose orphan drug exclusivity. Similarly, if a competitive product that contains the same active moiety and treats the same disease as our product candidate is approved for orphan drug exclusivity before our product candidate, we may not be able to obtain approval for our product candidate until the expiration of the competitive product's orphan drug exclusivity unless our product candidate is shown to be clinically superior to the competitive product.

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

Government agencies promulgate regulations and guidelines directly applicable to us and to our products. However, professional societies, industry groups, practice management groups, insurance carriers, physicians, private foundations and other organizations involved in various diseases or conditions from time to time may also publish guidelines or recommendations to healthcare providers, administrators and payers, and patient communities. Recommendations by government agencies or those other groups/organizations may relate to such matters as clinical guidelines, usage and reimbursement of our products by government and private payers. Recommendations or guidelines that are followed by patients, healthcare providers and payers could impact the use of our products in positive or negative ways. In addition, recommendations or guidelines may not be followed by patients, healthcare providers or payors, 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;

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- regulators may require us to conduct studies of the long-term effects associated with the use of our product candidates;

- regulators, institutional review boards or ethics committees may not authorize us to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- the FDA or any non-United States regulatory authority may impose conditions on us regarding the scope or design of our clinical trials or may require us to resubmit our clinical trial protocols to institutional review boards or ethics committees for re-inspection due to changes in the regulatory environment;

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

We will only obtain regulatory approval to commercialize a product candidate if we can demonstrate to the satisfaction of the FDA, and in the case of foreign commercialization, to the applicable foreign regulatory authorities, in well-designed and conducted clinical trials, that our product candidates are safe and effective and otherwise meet the appropriate standards required for approval for a particular indication.

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

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

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

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

For example, in our pivotal Phase 3 DUPLEX Study of sparsentan in FSGS, although we achieved the pre-specified interim FSGS partial remission of proteinuria endpoint after 36 weeks of treatment, the study did not achieve the primary efficacy eGFR slope endpoint over 108 weeks of treatment. While we plan to continue to engage with the FDA to explore a potential path forward for a supplemental New Drug Application (sNDA) in the U.S. and work with our collaborator CSL Vifor to engage with the European Medicines Agency ("EMA") to also explore a potential regulatory path forward in FSGS in the EU based on the DUPLEX data, there is no guarantee that we will be successful in doing so. In addition, a collaborative international effort referred to as the PARASOL project was initiated in late 2023 with a goal to define the quantitative relationships between short-term changes in biomarkers (proteinuria and GFR) and long-term outcomes in order to support the use of alternative proteinuria-based endpoints as a basis for accelerated and traditional approval. Even though representatives of regulatory agencies have been participating in the discussions, there is no guarantee that the outcome of those discussions will be reflected in any future formal determination by such regulatory agencies. There is no guarantee that the PARASOL group will achieve its intended goal, or that, even if it does, that we will be able to establish a pathway to a potential submission of sparsentan for FSGS based on the results from the DUPLEX Study, that the FDA and/or EMA will support an application for sparsentan in FSGS, or that sparsentan will be approved for FSGS.

Also, in our pivotal Phase 3 PROTECT Study of sparsentan in IgAN, although we achieved the pre-specified primary efficacy proteinuria endpoint after 36 weeks of treatment, and after 110 weeks of treatment, FILSPARI demonstrated long-term kidney function preservation and achieved a clinically meaningful difference in estimated glomerular filtration rate (eGFR) total and chronic slope versus irbesartan, the study narrowly missed statistical significance in eGFR total slope while achieving statistical significance in eGFR chronic slope for purposes of regulatory review in the EU. In December 2023, we announced the completion of a successful pre-NDA meeting with the FDA for FILSPARI in IgAN. In March 2024, we submitted a supplemental New Drug Application (sNDA) for conversion of the existing U.S. accelerated approval of FILSPARI to full approval. In May 2024, we announced that the FDA has accepted and granted Priority Review of the sNDA to convert FILSPARI from accelerated approval to full approval for the treatment of IgAN in the U.S. The FDA assigned a PDUFA target action date of September 5, 2024. However, there is no guarantee that the FDA will provide a decision on the application by the target action date, that the FDA will not raise additional requirements prior to acting on the application for approval, that the FDA's accelerated approval of FILSPARI will continue, or that FILSPARI will receive full approval for the treatment of IgAN. Furthermore, if the FDA grants full approval for FILSPARI for the treatment of IgAN, there is no guarantee that the FDA will approve an expanded label.

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.

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

Success in nonclinical testing and early clinical trials does not ensure that later clinical trials will be successful. For example, while we saw trends in favor of sparsentan in the two-year confirmatory endpoint analysis in the DUPLEX Study in FSGS, the positive eGFR results from the open-label portion of the DUET study of sparsentan in FSGS were not replicated in the Phase 3 clinical trial with statistical significance. Similarly, while the Phase 3 PROTECT Study of FILSPARI in IgAN demonstrated long-term kidney function preservation in IgAN and met the endpoint for eGFR chronic slope for the purposes of regulatory review in the EU, and all topline efficacy endpoints favored FILSPARI as compared to the active control (irbesartan), the study narrowly missed statistical significance with respect to the eGFR total slope endpoint. Similarly, the positive nonclinical data we have seen from pegtibatinase being tested in a mouse model of homocystinuria and the positive topline results we reported in December 2021 and May 2023 from the ongoing Phase 1/2 clinical trial of pegtibatinase may not be replicated in future studies. We cannot assure that any current or future clinical trials of sparsentan or pegtibatinase will ultimately be successful. Before obtaining regulatory approval to conduct clinical trials of our product candidates, we must conduct extensive nonclinical tests to demonstrate the safety of our product candidates in animals. Nonclinical testing is expensive, difficult to design and implement, and can take many years to complete. In addition, during the clinical development process, additional nonclinical toxicology studies are routinely conducted concurrently with the clinical development of a product candidate. If any of our product candidates show unexpected findings in concurrent toxicology studies, we could experience potentially significant delays in, or be required to abandon, development of that product candidate. A failure of one or more of our nonclinical studies can occur at any stage of testing.

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

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

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

In February 2023, the FDA granted accelerated approval to FILSPARI (sparsentan) to reduce proteinuria in adults with primary IgAN at risk of rapid disease progression, generally a UPCR ≥1.5 gram/gram. In September 2023, we announced topline two-year confirmatory secondary endpoint results from the PROTECT Study. While FILSPARI demonstrated long-term kidney function preservation and achieved a clinically meaningful difference in estimated glomerular filtration rate (eGFR) total and chronic slope versus irbesartan as well as statistical significance in eGFR chronic slope for purposes of regulatory review in the EU, the PROTECT Study narrowly missed statistical significance in eGFR total slope, which was the pre-specified confirmatory endpoint in the U.S. In December 2023, we announced the completion of a successful pre-NDA meeting with the FDA for FILSPARI in IgAN. In March 2024, we submitted a supplemental New Drug Application (sNDA) for conversion of the existing U.S. accelerated approval of FILSPARI to full approval. In May 2024, we announced that the FDA has accepted and granted Priority Review of the sNDA to convert FILSPARI from accelerated approval to full approval for the treatment of IgAN in the U.S. The FDA assigned a PDUFA target action date of September 5, 2024. However, there is no guarantee that the FDA will provide a decision on the application by the target action date, that the FDA will not raise additional requirements prior to acting on the application for approval, that the FDA's accelerated approval of FILSPARI will continue, or that FILSPARI will receive full approval for IgAN.

In April 2024, we and CSL Vifor announced that the European Commission has 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). The CMA is granted for all member states of the European Union, as well as in Iceland, Liechtenstein and Norway. The European Commission's decision follows the positive opinion from the Committee for Medicinal Products for Human Use ("CHMP") in February 2024, based on results from the pivotal Phase 3 PROTECT Study of FILSPARI in IgAN. There is no guarantee that European regulators will grant full approval of sparsentan for IgAN, that our timelines will not be delayed notwithstanding the availability of an expedited regulatory review pathway, or that we will receive related milestone payments.

In May 2023, we announced that the DUPLEX Study did not achieve its two-year primary endpoint with statistical significance over the active control irbesartan. Although we are encouraged by the results for the secondary endpoints on proteinuria and exploratory endpoints, including renal outcomes, which trended favorably for sparsentan, and we are continuing to analyze the data to further evaluate the potential for sparsentan as a treatment for FSGS and are engaging with the regulators to explore a potential path to a submission for sparsentan in FSGS, there is no guarantee that we or our collaborator CSL Vifor will be able to establish a pathway to a potential submission of sparsentan for FSGS based on the results from the DUPLEX Study, that the FDA and/or EMA will support an application for sparsentan in FSGS, or that sparsentan will be approved for FSGS.

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. Topline results from In September 2024, we announced a voluntary pause of enrollment in the HARMONY Study are expected Study. The voluntary enrollment pause was enacted following our determination that the desired drug substance profile was not achieved in 2026. the recent scale-up process. We expect to further evaluate the necessary commercial process improvements to enable the continuation of the Phase 3 program. After we conclude our evaluation, we will need to engage with regulators, and there is no guarantee that they will agree with our assessment. Although the FDA has granted Fast Track and Breakthrough Therapy designations to pegtibatinase for the treatment of HCU, there is no guarantee that our pivotal Phase 3 Harmony HARMONY Study will be successful or that pegtibatinase will be approved for HCU in the future, on an expedited the anticipated timeline or at all.

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

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

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

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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.*

The FDA's accelerated In September 2024, the FDA granted full approval of FILSPARI is limited to slow kidney function decline in adults with primary IgAN who are at risk of rapid disease progression, generally a UPCR ≥1.5 gram/gram. The continued approval of FILSPARI may be contingent upon confirmation of a clinical benefit in the Phase 3 PROTECT Study. In September 2023, we announced data from the Phase 3 PROTECT Study as further described herein, including in the risk factor titled "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." progression. Any future regulatory approvals that sparsentan or any of our other product candidates receives may be subject to significant limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate.

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

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

For example, we have certain post-marketing requirements and commitments associated with FILSPARI. Further, we face risks relating to those post-marketing obligations, as well as the commercial acceptance of FILSPARI. If the regulatory approval for FILSPARI and/or Thiola are withdrawn for any reason, it would have a material adverse impact on our sales and profitability. Furthermore, if the regulatory approval for Chenodal and/or Cholbam are withdrawn for any reason, it would reduce the chance that we will receive any or all of the milestone payments from the sale of our bile acid product portfolio in August 2023.

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

We depend on third-party clinical investigators and contract research organizations ("CROs") to conduct our clinical trials under agreements with us. The CROs play a significant role in the conduct of our clinical trials. Failure of the CROs to meet their obligations could adversely affect clinical development of our product candidates. The third-party clinical investigators are not our employees and we cannot control the timing or amount of resources they devote to our studies. If their performance is substandard, it could delay or prevent approval of our FDA applications. Moreover, these third-party investigators and CROs may also have relationships with other commercial entities, some of which may compete with us. If third-party investigators and CROs allocate their resources to assist our competitors at our expense, it could harm our competitive position. The introduction of new third parties into our ongoing clinical trials increases the risks associated with our dependence on third parties, including the risk that substandard performance by, or competing interests of, such third parties could have a negative impact on our clinical trials.

Risks Related to our Products and Product Candidates

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

The success of our products is dependent upon achieving and maintaining market acceptance. Commercializing products is time consuming, expensive and unpredictable. There can be no assurance that we will be able to, either by ourselves or in collaboration with our partners or through our licensees,

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

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

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

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

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

The FDA-approved label of FILSPARI is currently limited to adult patients with IgAN at risk of rapid disease progression, generally a UPCR ≥1.5 gram/gram. Based on our interactions with the FDA, we believe that the FDA has imposed the rapid disease progression limitation on the FILSPARI label because of the accelerated approval pathway under which the product has been approved, and that there may be an opportunity to further expand the label to cover a broader population of IgAN patients based on the confirmatory data from the PROTECT Study, pending favorable regulatory review. However, there can be no guarantee that this will be the case. For additional information, see the risk factor titled "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."

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

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

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

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

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

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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"), as well as a pending U.S. patent application directed to Thiola EC, we do not know whether the pending U.S. patent application or any future patent application will result in a granted patent covering Thiola EC. More generally, we may not be able to obtain additional issued patents relating to our technology or products. Even if issued, patents issued to us or our licensors may be challenged, narrowed, invalidated, held to be unenforceable or circumvented, which could limit our ability to stop competitors from marketing similar products or reduce the term of patent protection we may have for our products. In addition, in certain circumstances with respect to method of use patents, an ANDA applicant may certify that its proposed ANDA label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent. On January 30, 2024, the FDA approved Torrent Pharmaceuticals Limited's (Torrent) ANDA for Thiola EC (100mg and 300mg), and accordingly, Thiola EC is now subject to immediate generic competition. Changes in either patent laws or in interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property or narrow the scope of our patent protection.

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

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

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

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

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

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

We have negotiated a license agreement with Ligand Pharmaceuticals for the rights to sparsentan which we are initially developing for the treatment of IgAN and FSGS. This license subjects us to various commercialization, reporting and other obligations. If we were to default on our obligations, we and our licensees (including CSL Vifor and Renalys Pharma) could lose our rights to sparsentan. We have obtained a U.S. patent and European patent each covering the use of sparsentan for treating glomerulosclerosis, including FSGS, as well as a second U.S. patent and a second European patent each covering both the use of sparsentan for treating IgAN and the use of sparsentan for treating glomerulosclerosis, including FSGS. In November 2020, a third party filed an opposition to our second European patent (European Patent No. EP3222277, "the '277 EP Patent"), in the European Patent Office ("EPO"). While we are vigorously defending the '277 EP Patent against the opposition, there is no guarantee that we will be successful in doing so.

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

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

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

We expect to rely on orphan drug exclusivity for sparsentan and potential future product candidates that we may develop. Orphan drug status currently confers seven years of marketing exclusivity in the United States under the FDC Act, and up to ten years of marketing exclusivity in the EU for a particular product in a specified indication or, in the case of orphan drugs for which a pediatric investigation plan has been completed, 12 years. The FDA and European Commission have granted orphan designation for sparsentan for the treatment of IgAN and FSGS, and pegtibatinase for the treatment of homocystinuria. While we have been granted these orphan designations, we will not be able to rely on these designations to exclude other companies from manufacturing or selling these molecules for the same indication beyond these time frames. Furthermore, in the EU, orphan drug status is re-evaluated in connection with the marketing authorization review process and a product candidate must re-qualify as of such time in order to maintain orphan drug status and benefit from the potential regulatory exclusivity periods related to marketing authorizations granted to orphan products. The period of market exclusivity in the EU may, however, be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria on the basis of which it received orphan medicinal product destination, including where it can be demonstrated on the basis of available evidence that the original orphan medicinal product is sufficiently profitable not to justify maintenance of market exclusivity or where the prevalence of the condition has increased above the threshold. Additionally, a marketing authorization may be granted to a similar medicinal product with the same orphan indication during the 10 year period if: (i) the applicant consents to a second original orphan medicinal product application, (ii) the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities; or (iii) the second applicant can establish that its product, although similar, is safer, more effective or otherwise clinically superior to the original orphan medicinal product. A company may voluntarily remove a product from the register of orphan products.

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

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

In March 2010, President Obama signed the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the "PPACA"), a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The PPACA revised the definition of “average manufacturer price” for reporting purposes, which could increase the amount of Medicaid drug rebates to states. The PPACA also increased the mandated Medicaid rebate from 15.1% to 23.1% of the average manufacturer price, expanded the rebate to Medicaid managed care utilization and increased the types of entities eligible for the federal 340B drug discount program. Further, the law imposed a significant annual fee on companies that manufacture or import certain branded prescription drug products. There have been executive, judicial, Congressional, and political challenges to certain aspects of the PPACA. For example, on June 17, 2021 the U.S. Supreme Court dismissed a challenge on procedural grounds that argued the PPACA is unconstitutional in its entirety because the “individual mandate” was repealed by Congress. Further, on August 16, 2022, President Biden signed the Inflation Reduction Act of 2022 ("IRA") into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in PPACA marketplaces through plan year 2025. The IRA also eliminates the "donut hole" under the Medicare Part D program beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost and through a newly established manufacturer discount program. It is possible that the PPACA will be subject to judicial or Congressional challenges in the future. It is unclear how any additional healthcare reform measures of the Biden administration will impact the PPACA and our business.

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. For example, in August 2011, President Obama signed into law the Budget Control Act of 2011, which, among other things, includes aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect beginning on April 1, 2013 and, due to subsequent legislative amendments, will stay in effect until 2032 unless additional Congressional action is taken. Additionally, in January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers, including hospitals and imaging centers.

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

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

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

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

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

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Further, there has been increasing legislative and enforcement interest in the United States with respect to drug pricing practices, including several recent U.S. congressional inquiries and federal and state legislation designed to, among other things, increase drug pricing transparency, expedite generic competition, review relationships between pricing and manufacturer patient assistance programs, and reform government program drug reimbursement methodologies. At the federal level, in July 2021, the Biden administration released an executive order that included multiple provisions aimed at prescription drugs. In response to Biden's executive order, on September 9, 2021, HHS released a Comprehensive Plan for Addressing High Drug Prices that outlines principles for drug pricing reform. The plan sets out a variety of potential legislative policies that Congress could pursue as well as potential administrative actions HHS can take to advance these principles. In addition, the IRA, among other things (i) directs HHS to negotiate the price of certain high-expenditure, single-source drugs and biologics covered under Medicare and (ii) imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation. These provisions take effect progressively starting in fiscal year 2023. On August 29, 2023 August 15, 2024, HHS announced the list agreed-upon price of the first ten drugs that will be subject to price negotiations, although the Medicare drug price negotiation program is currently subject to legal challenges. HHS will select up to fifteen additional drugs covered under Part D for negotiation in 2025. HHS has and will continue to issue and update guidance as these programs are implemented. It is currently unclear how the IRA will be implemented but it is likely to have a significant impact on the pharmaceutical industry. In addition, in response to the Biden administration's October 2022 executive order, on February 14, 2023, HHS released a report outlining three new models for testing by the Center for Medicare and Medicaid Innovation which will be evaluated on their ability to lower the cost of drugs, promote accessibility, and improve quality of care. It is unclear whether the models will be utilized in any health reform measures in the future. Further, on December 7, 2023, the Biden administration announced an initiative to control the price of prescription drugs through the use of march-in rights under the Bayh-Dole Act. On December 8, 2023, the National Institute of Standards and Technology published for comment a Draft Interagency Guidance Framework for Considering the Exercise of March-In Rights which for the first time includes the price of a product as one factor an agency can use when deciding to exercise march-in rights. While march-in rights have not previously been exercised, it is uncertain if that will continue under the new framework. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. For example, on January 5, 2024, the FDA

approved Florida's Section 804 Importation Program (SIP) proposal to import certain drugs from Canada for specific state healthcare programs. It is unclear how this program will be implemented, including which drugs will be chosen, and whether it will be subject to legal challenges in the United States or Canada. Other states have also submitted SIP proposals that are pending review by the FDA. Any such approved importation plans, when implemented, may result in lower drug prices for products covered by those programs.

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

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

The use of any of our potential products in clinical trials, and the sale of any approved products, may expose us to liability claims. These claims might be made directly by consumers, health care providers, pharmaceutical companies or others selling our products. We have obtained limited product liability insurance coverage for our clinical trials in the amount of $10 million per occurrence and $30 million in the aggregate. However, our insurance may not reimburse us or may not be sufficient to reimburse us for any expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive, and we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. We intend to expand our insurance coverage as we obtain marketing approval for additional product candidates in development, but we may be unable to obtain commercially reasonable product liability insurance. On occasion, juries have awarded large judgments in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or series of claims brought against us would decrease our cash reserves and could cause our stock price to fall.

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

Several of our competitors have substantially greater financial, research and development, distribution, manufacturing and marketing experience and resources than we do and represent substantial long-term competition for us. Other companies may succeed in developing and marketing products that are more effective and/or less costly than any products that may be developed and marketed by us, or that are commercially accepted before or perceived as preferred relative to any of our products. 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. Although In particular, the competitive landscape for IgAN is rapidly evolving and is expected to continue to evolve as multiple new modalities advance in development and potentially gain approval. Furthermore, although we believe that our orphan drug status and proprietary position with respect to sparsentan may give us a competitive advantage, new developments are expected to continue and there can be no assurance that discoveries by others will not render our products and product candidates noncompetitive. Furthermore, competitors could enter the market with generic versions of our products. For example, a generic option for the 100mg version of the original formulation of Thiola (tiopronin tablets) was approved by the FDA in May 2021 and a second 100mg version of the original formulation of Thiola (tiopronin tablets) was approved by the FDA in June 2022. Also, as of June 30, 2024 September 30, 2024, three four generic options for the 100mg and 300mg versions of Thiola EC have been approved by the FDA and two three have 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 requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up initial production and in maintaining required quality control. These problems include difficulties with production costs and yields and quality control, including stability of the product candidate.

In September 2024, we announced a voluntary pause of enrollment in the Phase 3 HARMONY Study evaluating pegtibatinase for the treatment of classical homocystinuria (HCU). The voluntary enrollment pause enables us to work to address necessary process improvements in manufacturing scale-up to support commercial scale manufacturing as well as full enrollment in the HARMONY Study. The voluntary enrollment pause was enacted following our determination that the desired drug substance profile was not achieved in the recent scale-up process. We expect to further evaluate

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the necessary commercial process improvements to enable the continuation of the Phase 3 program and anticipate the earliest date to restart enrollment in the Phase 3 HARMONY Study will be in 2026. While we believe we will be able to successfully implement the necessary process improvements, there is no guarantee that we will be able to successfully implement the necessary process improvements on the anticipated timeline, or at all.

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

- reliance on the third party for regulatory compliance and quality assurance;
- limitations on supply availability resulting from capacity and scheduling constraints of the third parties;
- less control over cost increases resulting from inflationary pressures affecting raw materials and other supply chain components;
- impact on our reputation in the marketplace if manufacturers of our products fail to meet the demands of our customers;
- the possible breach of the manufacturing agreement by the third party because of factors beyond our control; and
- the possible termination or nonrenewal of the agreement by the third party, based on its own business priorities, at a time that is costly or inconvenient for us.

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

Our contract manufacturers are required to adhere to FDA regulations setting forth cGMP and comparable foreign regulatory authority requirements. These regulations cover all aspects of the manufacturing, testing, quality control and recordkeeping relating to our product candidates and any products that we may commercialize. Our manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our manufacturers are subject to unannounced inspections by the FDA, state regulators and similar regulators outside the United States to monitor and ensure compliance with cGMP. We are ultimately responsible for ensuring that our API and finished products are manufactured in accordance with cGMP regulations and similar regulatory requirements outside the United States, and it is therefore critical that we maintain effective management practices and oversight with respect to our third-party manufacturers, including routine auditing. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including shutdown of the third-party vendor or invalidation of drug product lots or processes, fines, injunctions, civil penalties, failure of regulatory authorities to grant marketing approval of our product candidates, delays, suspension, variation or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect regulatory approval and supplies of our product candidates.

Our product and any products that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that are both capable of manufacturing for us and willing to do so. A health epidemic or pandemic and associated vaccine or treatment development and manufacturing efforts may increase demand for the services supplied by many third-party manufacturers, including some of those that we utilize for our products and product candidates, which may result in decreased availability of manufacturing slots at many such facilities. If the third parties that we engage to manufacture products for our developmental or commercial products should halt or cease to continue to do so for any reason, we likely would experience interruptions in cash flows and/or delays in advancing our clinical trials while we identify and qualify replacement suppliers, and we may be unable to obtain replacement supplies on terms that are favorable to us. Later relocation to another manufacturer will also require notification, review and other regulatory approvals from the FDA and other regulators and will subject our production to further cost and instability in the availability of our product candidates. In addition, if we are not able to obtain adequate supplies of our products and product candidates, or the drug substances used to manufacture them, it will be more difficult for us to sell our products and to develop our product candidates. This could greatly reduce our competitiveness and negatively affect our results of operations.

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

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

We rely on the manufacturers of our products and product candidates to purchase from third-party suppliers the materials necessary to produce the compounds for our nonclinical and clinical studies and rely on these other manufacturers for commercial distribution if we obtain marketing approval for any of our product candidates. Suppliers may not sell these materials to our manufacturers at the time we need them or on commercially reasonable terms and all such prices are susceptible to fluctuations in price and availability due to transportation costs, government regulations, price controls, and changes in economic climate or other foreseen circumstances. We do not have any control over the process or timing of the acquisition of these materials by our manufacturers. In addition, inflation and global supply chain disruptions, as well as past disruptions related to COVID-19 and potential future disruptions related to a future health epidemic or pandemic, wars, armed conflicts, and global geopolitical tension, including between the U.S. and China, have had and may continue to have a negative impact on our manufacturers' ability to acquire the materials necessary for our business. Changes in legislation could potentially impact our ability to secure the materials we need for our products and product candidates. For example, the U.S. Senate's homeland security committee House of Representatives recently voted to approve passed a bill that could restrict business with Chinese biotech companies. If this bill becomes law, or if other new laws or regulations prohibiting us from dealing with suppliers in China, we may have to find alternative suppliers and our ability to secure the materials we need on our planned timelines could be adversely impacted. Moreover, we currently do not have any agreements for the commercial production of these materials. If our manufacturers are unable to obtain these materials for our nonclinical and clinical studies, product testing and potential regulatory approval of our product candidates would be delayed, significantly impacting our ability to develop our product candidates. If our manufacturers or we are unable to purchase these materials after regulatory approval has been obtained for our product candidates, the commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would materially affect our ability to generate revenues from the sale of our product candidates. For example, in 2021 a membrane used in pegtibatinase drug substance manufacturing became more difficult to acquire due to the same or similar membranes being used in certain of the COVID-19 vaccine manufacturing processes. While our contingency plans enabled us to initiate Additionally, in September 2024, we announced a voluntary pause of enrollment in the Phase 3 study of pegtibatinase on HARMONY Study to enable us to work to address necessary process improvements in manufacturing scale-up to support commercial scale manufacturing as well as full enrollment in the HARMONY Study. The voluntary enrollment pause was enacted following our determination that the desired timeline, from drug substance profile was not achieved in the recent scale-up process. From time to time we continue to, and may in the future, face supply challenges or shortages of other materials necessary to manufacture pegtibatinase or our other products and product candidates. If our risk mitigation plans are not successful in overcoming these challenges, our pegtibatinase program or other products and product candidates, could be delayed.

Risks Related to Our Business

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.

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We have experienced significant growth over the past five years in the number of our employees and the scope of our operations. We have expanded our sales and marketing, compliance and legal functions in addition to expansion of all functions to support a commercial organization. We have also expanded our operations in connection with the commercial launch of FILSPARI in the United States, including by adding additional members to our sales force. To appropriately manage for our future, we must continue to implement and improve our managerial, operational and financial systems, continue to recruit, train and retain qualified personnel as needed, and successfully integrate any changes into our existing business. To succeed, we must recruit, retain, manage and motivate qualified clinical, scientific, technical, commercial and management personnel, and we face significant competition for experienced personnel.

Due to our limited resources, we may not be able to effectively manage the expansion of our operations or recruit, train and retain qualified personnel, including in connection with the ongoing commercial launch of FILSPARI in the United States. The management of changes to our operations may lead to significant costs and may divert our management and business development resources. Any inability on the part of our management to manage growth or other changes in our organization could delay the execution of our business plans or disrupt our operations.

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

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

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

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

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

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

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

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

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

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

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

The timelines and conduct of our ongoing clinical trials previously have been affected by COVID-19 and we may experience similar delays or interruptions due to other health epidemics or pandemics in the future. For example, in 2020 we experienced a reduction in the rates of patient enrollment in our ongoing clinical trials as a result of the COVID-19 pandemic. New health epidemics or pandemics may emerge that result in similar or more severe disruptions to our business, which could adversely impact our business and operating results.

Our strategic reorganization and the associated workforce reduction may not result in the level of savings that we currently anticipate, could result in costs and expenses that are greater than expected, and could disrupt our business.

In December 2023, we announced a strategic reorganization including an approximate 20% workforce reduction focused on non-field-based employees in an effort to align our resources on the ongoing FILSPARI launch and the pivotal Phase 3 HARMONY Study to support the potential approval of pegtibatinase as the first potential disease-modifying treatment for HCU. There is a chance that we will not realize the level of savings and improvements in our cost structure that we currently anticipate, and there may be unforeseen difficulties, delays or unexpected costs. If we are unable to realize the expected operational efficiencies and cost savings, our operating results and financial condition would be adversely affected. Furthermore, our strategic reorganization may be disruptive to our operations. For example, our workforce reductions could yield unanticipated consequences, such as increased difficulties in implementing our business strategy, including retention of our remaining employees. Employee litigation related to the headcount reduction could be costly and prevent management from fully concentrating on the business.

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

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We expect that our operating results will vary significantly from quarter-to-quarter and year-to-year as a result of investments in research and development, specifically our clinical and nonclinical development activities. We anticipate that certain of our expenses will continue to increase, depending on factors including but not limited to: the continuation and cost of our clinical trials and the research and development of additional product candidates; the costs involved in seeking and obtaining marketing approvals for our products, and in maintaining quality systems standards for our products; the timing of, and costs involved in, commercial activities, including product marketing, sales and distribution, costs related to our operational, financial, and management information systems and personnel, including personnel to support product development efforts and our obligations as a public company.

To attain and sustain profitability, we must succeed in developing and commercializing therapies with significant market potential. This will require us to be successful in a range of challenging activities, including the discovery of product candidates, successful completion of nonclinical testing and clinical trials of our product candidates, obtaining regulatory approval for these product candidates and manufacturing, marketing and selling those products for which we may obtain regulatory approval. We may not be successful enough in these activities to generate revenues that are substantial enough to recoup the expenses we have expended in conducting these activities to achieve profitability. Pursuant to the Ligand License Agreement, we are obligated to pay to Ligand an escalating annual royalty between 15% and 17% of net sales of FILSPARI and any other products containing sparsentan or related compounds, which will impact our potential future profit from the commercialization of FILSPARI in the United States and sparsentan for the treatment of IgAN in the EU if approved, as well as sparsentan for the treatment of FSGS, if approved. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become or remain profitable could depress the market price of our common stock and could impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations. A decline in the market price of our common stock may also cause a loss of a part or all of your investment.

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

If any of our products or any similar products distributed by other companies prove to be, or are asserted to be, harmful to consumers and/or subject to FDA 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, which covers all our directors and officers in some circumstances, our insurance coverage does not cover all of our indemnification obligations and may not be adequate to cover any indemnification or other claims against us. In addition, the underwriters of our present coverage may seek to avoid coverage in certain circumstances based upon the terms of the respective policies. If we incur liabilities that exceed our coverage under our directors and officers insurance policy or incur liabilities not covered by our insurance, we would have to self-fund any indemnification amounts owed to our directors and officers and employees in which case our results of operations and financial condition could be materially adversely affected. Further, if D&O insurance becomes prohibitively expensive to maintain in the future, we may be unable to renew such insurance on economic terms or unable to renew such insurance at all. The 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 increasing inflation in the United States and abroad. We currently have no additional commitments or arrangements for any additional financing to fund the research and development and commercial launch of our product candidates. General market conditions, including high interest rates and stock price volatility, actual or anticipated bank failures, and ongoing issues arising global geopolitical tensions, including the wars and other armed conflicts, as well as market conditions affecting companies in the life sciences industry in general, may make it difficult for us to seek financing from the capital markets on attractive terms, or at all.

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

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

- the timing, progress, cost and results of our clinical trials, preclinical studies and other discovery and research and development activities;
- the timing of, and costs involved in, seeking and obtaining marketing approvals for our products, and in maintaining quality systems standards for our products;
- the timing of, and costs involved in, commercial activities, including product marketing, sales and distribution;
- our ability to successfully commercialize FILSPARI in adult patients with IgAN, and to obtain regulatory approval for and successfully commercialize our other or future product candidates;
- increases or decreases in revenue from our marketed products, including decreases resulting from generic entrants or health epidemics or pandemics;
- debt service obligations on the 2025 Notes and 2029 Notes;
- the number and development requirements of other product candidates that we pursue;
- our ability to manufacture sufficient quantities of our products to meet expected demand;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property related claims;
- our ability to enter into collaboration, licensing or distribution arrangements and the terms and timing of these arrangements;
- the potential need to expand our business, resulting in additional payroll and other overhead expenses;

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- 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, 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 Products. We are also party to license agreements with CSL Vifor and Renalys Pharma, Inc. pursuant to which we are entitled to receive certain payments contingent on the future achievement of specified milestones, and royalty payments based on potential future sales in specified licensed territories. There is a risk that any or all of the milestone events under these various agreements might not be achieved, that our licensees may not achieve sales that would entitle us to royalty payments, and that any or all of the consideration tied to the achievement of the milestone events and/or royalties might not be received.

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

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

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

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

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challenges or risks could impair our ability to realize any benefit from our acquisitions or arrangements after we have expended resources on them.

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

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

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

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

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

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

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

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

We are also subject to regulation by supranational, national, regional, state and local agencies and regulatory authorities, including but not limited to the FDA, the Centers for Medicare & Medicaid Services ("CMS"), Department of Justice, the Federal Trade Commission, the HHS Office of Inspector General and other regulatory bodies. The FDC Act, Social Security Act, Public Health Service Act and other federal and state statutes and regulations, 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

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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.

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

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

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

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from commercializing the product candidate in the jurisdiction of the regulatory authority. We have only limited experience in meeting the regulatory requirements incumbent on the sale of drugs in the United States and elsewhere, and expect to rely on third parties to assist us in these processes. If these third parties fail to adequately adhere to the regulations governing drug distribution and promotion, we may be unable to sell our products, which could have a material effect on our ability to generate revenue.

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

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

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

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

The process of obtaining regulatory approvals is expensive, often takes many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in regulatory approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application may cause delays in the approval or rejection of an application. The FDA and non-United States regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional nonclinical, clinical or other studies. In addition, varying interpretations of the data obtained from nonclinical and clinical testing could delay, limit or prevent regulatory approval of a product candidate. Any regulatory approval we ultimately obtain may be limited or subject to restrictions or post approval commitments that render the approved product not commercially viable. Any FDA or other regulatory approval of our product candidates, once obtained, may be suspended, varied or withdrawn, including for failure to comply with regulatory requirements or if clinical or manufacturing problems follow initial marketing.

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

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

In the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal data privacy laws, consumer health data laws, consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act) and other similar laws (e.g., wiretapping laws). For example, HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), and their respective implementing regulations, imposes specific requirements relating to the privacy, security and transmission of individually identifiable health information. Among other things, HITECH, through its implementing regulations, makes certain of HIPAA's privacy and security standards directly applicable to business associates, defined as a person or organization, other than a member of a covered entity's workforce, that creates, receives, maintains or transmits protected health information for or on behalf of a covered entity for a function or activity regulated by HIPAA as well as their covered subcontractors.

Additionally, in the past few years, numerous U.S. states—including but not limited to California, Colorado, Connecticut, Texas, Utah and Virginia—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 may include the right to access, correct, or delete certain personal data, and to opt-out of certain data processing activities, such as targeted advertising, profiling, and automated decision-making. The exercise of these rights may impact our business and ability to provide our products and services. Certain states also impose stricter requirements for processing certain personal data, including sensitive information, such as conducting data privacy impact assessments. These state laws allow for statutory fines for noncompliance. For example, the California Consumer Privacy Act of 2018, as amended by the California Privacy Rights Act of 2020 ("CPRA"), (collectively "CCPA"), 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 afford California residents certain privacy rights related to their personal data, such as those noted herein. The CCPA allows for fines for noncompliance (up to $7,500 per intentional violation) 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 other personal data we maintain about residents in these states. Similar laws are being considered in several other states, as well as at the local level, and we expect more jurisdictions to pass similar laws in the future.

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

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

Outside the United States, an increasing number of laws, regulations, and industry standards apply to data privacy and security. For example, the European Union's General Data Protection Regulation ("EU GDPR"), the United Kingdom's GDPR ("UK GDPR") (EU GDPR and UK GDPR, collectively "GDPR"), Brazil's General Data Protection Law (Lei Geral de Proteção de Dados Pessoais, or "LGPD") (Law No. 13,709/2018), and China's Personal Information Protection Law ("PIPL") impose strict requirements for processing personal data. For example, the GDPR imposes significant and complex burdens on processing personal data, particularly for processing "special category personal data" (such as personal data related to health and genetic information), which could be relevant to our operations in the context of our conduct of clinical trials and is of interest to relevant regulators. Under the GDPR, government regulators may impose temporary or definitive bans on data processing, as well as fines of up to 20 million euros under the EU GDPR, 17.5 million pounds sterling under the UK GDPR or, in each case, or 4% of annual global revenue, whichever is greater. Further, under the GDPR, individuals may initiate litigation related to processing of their personal data, as well as consumer protection organizations authorized at law to represent data subjects' interests.

In addition, privacy advocates and industry groups around the world have proposed, and may propose, standards with which we are legally or contractually bound to comply, or 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. If these policies, materials or statements are found to be deficient, lacking in transparency, deceptive, unfair, or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators or other adverse consequences.

In the ordinary course of business, we may transfer personal data from Europe and other jurisdictions to the United States or other countries. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal data to other countries. In particular, the European Economic Area ("EEA") and the United Kingdom ("UK") have significantly restricted the transfer of personal data to the United States and other countries whose privacy laws it generally believes are inadequate. Other jurisdictions may adopt similarly stringent interpretations of their data localization and cross-border transfer laws, which could make it more difficult to transfer information across jurisdictions or prevent us from conducting business in certain countries. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and UK to the United States in compliance with 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 activities groups. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers out of Europe for allegedly violating the GDPR's cross-border data transfer limitations. Regulators in the United States are also increasingly scrutinizing certain personal data transfers and may impose data localization requirements, for example, the Biden Administration's executive order Preventing Access to Americans' Bulk Sensitive Personal Data and United States Government-Related Data by Countries of Concern.

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 may be subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions. Preparing for and complying with these obligations requires significant resources and may necessitate changes to our information technologies, systems, and practices and to those of any third parties 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 may process proprietary, confidential, and sensitive data, including personal data (such as health-related data and data related to our clinical trials), intellectual property, and trade secrets (collectively, sensitive information).

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 may be subject to a variety of other evolving threats, including, but not limited to, social-engineering attacks (including through deep fakes, which may be 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

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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 become more common and has increased risks to our information technology systems and data, as more of our employees utilize network connections, computers, and devices outside our premises or network, including working at home, while in transit, and in public locations. Future or past business transactions (such as acquisitions or integrations) could also expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. Furthermore, we may discover security issues that were not found during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

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

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 may not be able to detect, mitigate, and remediate all such vulnerabilities including on a timely basis. It may also be difficult and/or costly to detect, investigate, mitigate, contain, and remediate a security incident. Further, we may experience delays in developing and deploying remedial measures and patches designed to address identified vulnerabilities. Vulnerabilities could be exploited and 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.

Any of the previously identified or similar threats could 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. A security incident or other interruption could disrupt our ability (and that of third parties with whom we work) to provide our products. We may expend significant resources or modify our business activities (including our clinical trial activities) to try to protect against security incidents. Certain data privacy and security obligations require us to implement and maintain specific security measures, industry-standard or reasonable security measures to protect our information technology systems and sensitive information.

Applicable data security and public company disclosure obligations may 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, such as providing credit monitoring and identity theft protection services. 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. If we (or a third party with whom we work) experience a security incident or are perceived to have experienced a security incident, we may experience adverse consequences. These consequences may include: government enforcement actions (for example, investigations, fines, penalties, audits, and inspections); additional reporting requirements and/or oversight; restrictions on processing sensitive information (including personal data); litigation (including class claims); indemnification obligations; negative publicity; reputational harm; monetary fund diversions; 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.

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. Moreover, experiencing a material cybersecurity incident and any mandatory disclosures could lead to negative publicity, loss of customer, investor or partner confidence in the effectiveness of our cybersecurity measures, diversion of management's attention, governmental investigations, lawsuits, and the expenditure of significant capital and other resources.

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

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.

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

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

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

Effective January 1, 2022, the Tax Cuts and Jobs Act of 2017 eliminated the option to deduct research and development expenses for tax purposes in the year incurred and requires taxpayers to capitalize and subsequently amortize such expenses over five years for research activities conducted in the United States and over 15 years of research activities conducted outside the United States. Unless the United States Department of the Treasury issues regulations that narrow the application of this provision to a smaller subset of our research and development expenses or the provision is deferred, modified, or repealed by Congress, in future years we may experience a material decrease in our cash flows from operations and an offsetting similarly sized increase in our net deferred tax

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assets over these amortization periods. The actual impact of this provision will depend on multiple factors, including the amount of research and development expenses we will incur and whether we conduct our research and development activities inside or outside the United States and our overall net operating loss position.

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

Under current law, our federal net operating losses ("NOLs") generated in tax years beginning after December 31, 2017, may be carried forward indefinitely, but the deductibility of such federal NOL carryforwards in a taxable year is limited to 80% of taxable income in such year. As of December 31, 2023, we had federal NOL carryforwards of $154.3 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 completed a study to analyze whether any ownership changes occurred through December 31, 2023, and determined an ownership change occurred in May of 2023. In addition, we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control.

As a result, our federal NOL carryforwards may be subject to a percentage limitation if used to offset income in tax years following an ownership change. In addition, it is possible that we have in the past undergone, and in the future may undergo, additional ownership changes that could limit our ability to use all of our pre-change NOL carryforwards and other pre-change tax attributes (such as research tax credits) to offset our post-change income or taxes. Similar provisions of state tax law may also apply to limit our use of accumulated state tax attributes. In addition, at the state level, there may be periods during which the use of NOL carryforwards is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. 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 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 and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical FDA, SEC and other government employees and stop critical activities. If a prolonged government shutdown occurs, or if the FDA or EDA experience resource constraints, it could significantly impact the ability of the applicable regulatory agency to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations. 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.*

There is an increasing focus from certain investors, employees, partners, and other stakeholders concerning ESG matters. While we have internal efforts directed at ESG matters and preparations for increased future disclosures, we may be perceived by certain stakeholders as not acting responsibly in connection with these matters, which could negatively impact us. Moreover, the SEC recently adopted rules designed to enhance and standardize climate-related disclosures, which have been stayed pending judicial review. If these rules or other climate-related disclosures rules become effective, 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 United Kingdom's ("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 Medicines and Healthcare products Regulatory Agency, or MHRA, is now the UK's standalone regulator for medicinal products and medical devices. Great Britain (England, Scotland and Wales) is now a third country to the EU. Northern Ireland will, with regard to EU regulations, continue to follow the EU regulatory rules for now.

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, and which aimed to streamline clinical trials approvals, enable innovation, enhance clinical trials transparency, enable greater risk proportionality, and promote patient and public involvement in 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. These resulting legislative amendments will determine how closely the UK regulations will align with the CTR. In October 2023, the MHRA announced a new Notification Scheme for clinical trials which enables a more streamlined and risk-proportionate approach to initial clinical trial applications for Phase 4 and low-risk Phase 3 clinical trial applications.

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 currently remains within the scope of EU authorizations in relation to centrally authorized medicinal products. Accordingly, until the Windsor Framework is implemented in Northern Ireland on January 1, 2025, products falling within the scope of the EU centralized procedure can only be authorized through UK national authorization procedures in Great Britain.

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 Great Britain, 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 Great Britain.

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

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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 chemical intermediates we use that are manufactured in China), political unrest or unstable economic conditions in China. Any recall of the manufacturing lots or similar action regarding our API used in clinical trials could delay the trials or detract from the integrity of the trial data and its potential use in future regulatory filings. In addition, manufacturing interruptions or failure to comply with regulatory requirements by any of these manufacturers could significantly delay clinical development of potential products and reduce third-party or clinical researcher interest and support of proposed trials. These interruptions or failures could also impede commercialization of our product candidates and impair our competitive position.

We have previously identified a material weakness in our internal control over financial reporting. If additional 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.

We previously concluded that there was a matter that constituted a material weakness in our internal control over financial reporting that has since been remediated. A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting such that there is a reasonable possibility that a material misstatement of our annual or interim consolidated financial statements will not be prevented or detected on a timely basis. As disclosed under Item 9A of our Annual Report on Form 10-K for the fiscal year ended December 31, 2022, there was a material weakness in our internal control over financial reporting as of December 31, 2022 because we did not design effective controls and procedures to evaluate the accounting for a certain pre-launch inventory contract affecting the timing of recognition of research and development expense. As a result of the material weakness, we added controls for the timely accounting evaluation of research and development contracts that were intended to ensure appropriate expense recognition of certain pre-launch inventory. As of December 31, 2023, the material weakness has been remediated.

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

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

Adverse developments that affect financial institutions, such as events involving liquidity that are rumored or actual, have in the past and may in the future lead to bank failures and market-wide liquidity problems. For example, on March 10, 2023, Silicon Valley Bank (SVB) was closed by the California Department of Financial Protection and Innovation, which appointed the Federal Deposit Insurance Corporation (FDIC) as receiver. Similarly, on March 12, 2023, Signature Bank and Silvergate Capital Corp. were each swept into receivership. In addition, on May 1, 2023, the FDIC seized First Republic Bank and sold its assets to JPMorgan Chase & Co. 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 higher interest rates or costs and tighter financial and operating covenants, or systemic limitations on access to credit and liquidity sources, thereby making it more difficult for us to acquire financing on acceptable terms or at all. Any decline in available funding or access to our cash and liquidity resources could, among other risks, adversely impact our ability to meet our operating expenses, financial obligations or fulfill our other obligations, result in breaches of our financial and/or contractual obligations or result in violations of federal or state wage and hour laws. Any of these impacts, or any other impacts resulting from the factors described above or other related or similar factors not described above, could have material adverse impacts on our liquidity and our current and/or projected business operations and financial condition and results of operations.

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

We maintain the majority of our cash and cash equivalents in accounts at banking institutions in the United States that we believe are of high quality. Cash held in these accounts often exceed the FDIC insurance limits. If such banking institutions were to fail, we could lose all or a portion of amounts held in excess of such insurance limitations. 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.

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Risks Related to our Indebtedness and Investments

Our indebtedness could adversely affect our financial condition.

As of June 30, 2024 September 30, 2024, we had approximately $385 million of total debt outstanding, classified as long term. As a result of our indebtedness, a portion of our cash flow will be required to pay interest and principal on the 2025 Notes and 2029 Notes if the notes are not converted to shares of common stock prior to maturity. We may not generate sufficient cash flow from operations or have future borrowings available to enable us to repay our indebtedness or to fund other liquidity needs.

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

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

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

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

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

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

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

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

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

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

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

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

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

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

General Risk Factors

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

The global credit and financial markets have experienced extreme volatility and disruptions, including as a result of inflation and high interest rates, bank failures, 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, increase in inflation, declines in consumer confidence, declines in economic growth, increases in unemployment rates and uncertainty about economic stability. There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. Our general business strategy may be adversely affected by any such economic downturn, volatile business environment, higher inflation, or unpredictable and unstable market conditions. If the current equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our operations, growth strategy, financial performance and stock price and could require us to delay or abandon clinical development plans.

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

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

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

None of Trading Arrangements

During the Company's fiscal quarter ended September 30, 2024, our directors and/or officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted, modified or terminated a Rule the "Rule 10b5-1 trading arrangement arrangements" or a non-Rule "non-Rule 10b5-1 trading arrangement during the quarter ended June 30, 2024.

There arrangements," as those terms are no other disclosures required by this item. defined in Regulation S-K, Item 408, set forth below:

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 D. Baynes, member of our Board of Directors	August 16, 2024	Rule 10b5-1 Trading Arrangement	Up to 26,000 shares (3)	October 31, 2025
Sandra Calvin, SVP and Chief Accounting Officer	September 9, 2024	Rule 10b5-1 Trading Arrangement	Up to 86,187 shares (4)	September 9, 2025

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 of underlying stock options expiring in 2026 and 2027.

4 Includes shares underlying stock options expiring in January 2025.

Trading Arrangements Modified or Terminated:

None.

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Item 6. Exhibits

(a) Exhibits

1.1	Amended and Restated Open Market Sale Agreement, dated October 31, 2024, by and between the Company and Jefferies LLC.
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† 5.1	The Company's 2018 Equity Incentive Plan. Opinion of Cooley LLP.
10.1*	Amendment No. 5 to Sublicense Agreement, dated as amended (incorporated by reference to of March 20, 2018, between the Company and Ligand Pharmaceuticals Incorporated.
23.1	Consent of Cooley LLP (included in Exhibit 99.1 to the Company's Current Report on Form 8-K, filed with the SEC on May 13, 2024). 5.1).
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 Certain confidential information contained in this Exhibit, marked in brackets, has been omitted, because it is both not material and of the type of information that the registrant treats as private or compensatory plan. confidential.

SIGNATURES

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

Date: August 1, 2024 October 31, 2024

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

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Exhibit 1.1

AMENDED & RESTATED OPEN MARKET SALE AGREEMENT℠

October 31, 2024

JEFFERIES LLC
520 Madison Avenue
New York, New York 10022

Ladies and Gentlemen:

Travere Therapeutics, Inc., a Delaware corporation (the "**Company**"), proposes, subject to the terms and conditions stated herein, to issue and sell from time to time through Jefferies LLC, as sales agent and/or principal (the "**Agent**"), shares of the Company's common stock, par value $0.0001 per share (the "**Common Shares**") on the terms set forth in this amended and restated agreement (this "**Agreement**").

Whereas, the Agent and the Company are parties to that certain Open Market Sales Agreement℠ dated as of February 24, 2020 (the "**Original Agreement**"); and

Whereas, the Agent and the Company desire to amend and restate the Original Agreement on the terms and conditions set forth herein.

Now, Therefore, in consideration of the mutual promises herein contained, the Agent and the Company agree as follows:

Section 1. DEFINITIONS

(a) Certain Definitions. For purposes of this Agreement, capitalized terms used herein and not otherwise defined shall have the following respective meanings:

"**Affiliate**" of a Person means another Person that directly or indirectly, through one or more intermediaries, controls, is controlled by, or is under common control with, such first- mentioned Person. The term "control" (including the terms "controlling," "controlled by" and "under common control with") means the possession, direct or indirect, of the power to direct or cause the direction of the management and policies of a Person, whether through the ownership of voting securities, by contract or otherwise.

"**Agency Period**" means the period commencing on the date of this Agreement and expiring on the earliest to occur of (x) the date on which the Agent shall have placed the Maximum Program Amount pursuant to this Agreement and (y) the date this Agreement is terminated pursuant to Section 7.

"**Commission**" means the U.S. Securities and Exchange Commission.

℠ "Open Market Sale Agreement" is a service mark of Jefferies LLC

"**Exchange Act**" means the Securities Exchange Act of 1934, as amended, and the rules and regulations of the Commission thereunder.

"**Floor Price**" means the minimum price set by the Company in the Issuance Notice below which the Agent shall not sell Shares during the applicable period set forth in the Issuance Notice, which may be adjusted by the Company at any time during the period set forth in the Issuance Notice by delivering written notice of such change to the Agent and which in no event shall be less than $1.00 without the prior written consent of the Agent, which may be withheld in the Agent's sole discretion.

"**Issuance Amount**" means the aggregate Sales Price of the Shares to be sold by the Agent pursuant to any Issuance Notice.

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"**Issuance Notice**" means a written notice delivered to the Agent by the Company in accordance with this Agreement in the form attached hereto as Exhibit A that is executed by its Chief Executive Officer, President or Chief Financial Officer.

"**Issuance Notice Date**" means any Trading Day during the Agency Period that an Issuance Notice is delivered pursuant to Section 3(b)(i).

"**Issuance Price**" means the Sales Price less the Selling Commission.

"**Maximum Program Amount**" means Common Shares with an aggregate Sales Price of the lesser of (a) the number or dollar amount of Common Shares registered under the effective Registration Statement (as defined below) pursuant to which the offering is being made, (b) the number of authorized but unissued Common Shares (less Common Shares issuable upon exercise, conversion or exchange of any outstanding securities of the Company or otherwise reserved from the Company's authorized capital stock), (c) the number or dollar amount of Common Shares permitted to be sold under Form S-3 (including General Instruction I.B.6 thereof, if applicable), or (d) the number or dollar amount of Common Shares for which the Company has filed a Prospectus (as defined below).

"**Person**" means an individual or a corporation, partnership, limited liability company, trust, incorporated or unincorporated association, joint venture, joint stock company, governmental authority or other entity of any kind.

"**Principal Market**" means the Nasdaq Global Market or such other national securities exchange on which the Common Shares, including any Shares, are then listed.

"**Sales Price**" means the actual sale execution price of each Share placed by the Agent pursuant to this Agreement.

"**Securities Act**" means the Securities Act of 1933, as amended, and the rules and regulations of the Commission thereunder.

"**Selling Commission**" means three percent (3%) of the gross proceeds of Shares sold pursuant to this Agreement, or as otherwise agreed between the Company and the Agent with respect to any Shares sold pursuant to this Agreement.

"**Settlement Date**" means the first business day following each Trading Day during the period set forth in the Issuance Notice on which Shares are sold pursuant to this Agreement, when the Company shall deliver to the Agent the amount of Shares sold on such Trading Day and the Agent shall deliver to the Company the Issuance Price received on such sales.

"**Shares**" shall mean the Company's Common Shares issued or issuable pursuant to this Agreement.

"**Trading Day**" means any day on which the Principal Market is open for trading.

Section 2. REPRESENTATIONS AND WARRANTIES OF THE COMPANY

The Company represents and warrants to, and agrees with, the Agent that as of (1) the date of this Agreement, (2) each Issuance Notice Date, (3) each Settlement Date, (4) each Triggering Event Date (as defined below) and (5) as of each Time of Sale (each of the times referenced above is referred to herein as a "**Representation Date**"), except as may be disclosed in the Prospectus (including any documents incorporated by reference therein and any supplements thereto) on or before a Representation Date:

(a) Registration Statement. The Company has prepared and filed with the Commission a shelf registration statement on Form S-3 (File No. 333-281194) that contains a base prospectus. Such registration statement registers the issuance and sale by the Company of the Shares under the Securities Act. The Company may file one or more additional registration statements from time to time that will contain a base prospectus and related prospectus or prospectus supplement, if applicable, with respect to the Shares. Except where the context otherwise requires, such registration statement(s), including any information deemed to be a part thereof pursuant to Rule 430B under the Securities Act, including all financial statements, exhibits and schedules thereto and all documents incorporated or deemed to be incorporated therein by reference pursuant to Item 12 of Form S-3 under the Securities Act as from time to time amended or supplemented, is herein referred to as the "**Registration Statement**," and the prospectus constituting a part of such registration statement(s), together with any prospectus supplement filed with the Commission pursuant to Rule 424(b) under the Securities Act relating to a particular issuance of the Shares, including all documents incorporated or deemed to be incorporated therein by reference pursuant to Item 12 of Form S-3 under the Securities Act, in each case, as from time to time amended or supplemented, is referred to herein as the "**Prospectus**," except that if any revised prospectus is provided to the Agent by the Company for use in connection with the offering of the Shares that is not required to be filed by the Company pursuant to Rule 424(b) under the Securities Act, the term "**Prospectus**" shall refer to such revised prospectus from and after the time it is first provided to the Agent for such use. The Registration Statement at the time it originally became effective is herein called the "**Original Registration Statement**." As used in this Agreement, the terms "amendment" or "supplement" when applied to the Registration Statement or the Prospectus shall be deemed to include the

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filing by the Company with the Commission of any document under the Exchange Act after the date hereof that is or is deemed to be incorporated therein by reference.

All references in this Agreement to financial statements and schedules and other information which is "contained," "included" or "stated" in the Registration Statement or the Prospectus (and all other references of like import) shall be deemed to mean and include all such financial statements and schedules and other information which is or is deemed to be incorporated by reference in or otherwise deemed under the Securities Act to be a part of or included in the Registration Statement or the Prospectus, as the case may be, as of any specified date; and all references in this Agreement to amendments or supplements to the Registration Statement or the Prospectus shall be deemed to mean and include, without limitation, the filing of any document under the Exchange Act which is or is deemed to be incorporated by reference in or otherwise deemed under the Securities Act to be a part of or included in the Registration Statement or the Prospectus, as the case may be, as of any specified date.

At the time the Registration Statement was or will be originally declared effective and at the time the Company's most recent annual report on Form 10-K was filed with the Commission, if later, the Company met the then-applicable requirements for use of Form S-3 under the Securities Act. During the Agency Period, each time the Company files an annual report on Form 10-K the Company will meet the then-applicable requirements for use of Form S-3 under the Securities Act.

(b) Compliance with Registration Requirements. The Original Registration Statement and any Rule 462(b) Registration Statement have been declared effective by the Commission under the Securities Act. The Company has complied to the Commission's satisfaction with all requests of the Commission for additional or supplemental information. No stop order suspending the effectiveness of the Registration Statement or any Rule 462(b) Registration Statement is in effect and no proceedings for such purpose have been instituted or are pending or, to the best knowledge of the Company, are contemplated or threatened by the Commission.

The Prospectus, when filed, complied in all material respects with the Securities Act and, if filed with the Commission through its Electronic Data Gathering, Analysis and Retrieval system ("**EDGAR**") (except as may be permitted by Regulation S-T under the Securities Act), was identical to the copy thereof delivered to the Agent for use in connection with the issuance and sale of the Shares. Each of the Registration Statement, any Rule 462(b) Registration Statement and any post-effective amendment thereto, at the time it became effective and at each Representation Date, complied and will comply in all material respects with the Securities Act and did not and will not contain any untrue statement of a material fact or omit to state a material fact required to be stated therein or necessary to make the statements therein not misleading. As of the date of this Agreement, the Prospectus and any Free Writing Prospectus (as defined below) considered together (collectively, the "**Time of Sale Information**") did not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements therein, in the light of the circumstances under which they were made, not misleading. The Prospectus, as amended or supplemented, as of its date and at each Representation Date, did

not and will not contain any untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading. The representations and warranties set forth in the three immediately preceding sentences do not apply to statements in or omissions from the Registration Statement, any Rule 462(b) Registration Statement, or any post-effective amendment thereto, or the Prospectus, or any amendments or supplements thereto, made in reliance upon and in conformity with information relating to the Agent furnished to the Company in writing by the Agent expressly for use therein, it being understood and agreed that the only such information furnished by the Agent to the Company consists of the information described in Section 6 below. There are no contracts or other documents required to be described in the Prospectus or to be filed as exhibits to the Registration Statement which have not been described or filed as required. The Registration Statement and the offer and sale of the Shares as contemplated hereby meet the requirements of Rule 415 under the Securities Act and comply in all material respects with said rule.

(c) Ineligible Issuer Status. The Company is not an "ineligible issuer" in connection with the offering of the Shares pursuant to Rules 164, 405 and 433 under the Securities Act. Any Free Writing Prospectus that the Company is required to file pursuant to Rule 433(d) under the Securities Act has been, or will be, filed with the Commission in accordance with the requirements of the Securities Act. Each Free Writing Prospectus that the Company has filed, or is required to file, pursuant to Rule 433(d) under the Securities Act or that was prepared by or on behalf of or used or referred to by the Company complies or will comply in all material respects with the requirements of Rule 433 under the Securities Act including timely filing with the Commission or retention where required and legending, and each such Free Writing Prospectus, as of its issue date and at all subsequent times through the completion of the issuance and sale of the Shares did not, does not and will not include any information that conflicted, conflicts with or will conflict with the information contained in the Registration Statement or the Prospectus, including any document incorporated by reference therein. Except for the Free Writing Prospectuses, if any, and electronic road shows, if any, furnished to you before first use, the Company has not prepared, used or referred to, and will not, without your prior consent, prepare, use or refer to, any Free Writing Prospectus.

(d) This Agreement. This Agreement has been duly authorized, executed and delivered by the Company.

(e) Authorization of the Shares. The Shares have been duly authorized for issuance and sale pursuant to this Agreement and, when issued and delivered by the Company against payment therefor pursuant to this Agreement, will be validly issued, fully paid and nonassessable, and the

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issuance and sale of the Shares is not subject to any preemptive rights, rights of first refusal or other similar rights to subscribe for or purchase the Shares.

(f) No Applicable Registration or Other Similar Rights. There are no persons with registration or other similar rights to have any equity or debt securities registered for sale under the Registration Statement or included in the offering contemplated by this Agreement.

(g) No Material Adverse Change. Except as otherwise disclosed in the Registration Statement and the Prospectus, subsequent to the respective dates as of which information is given in the Registration Statement and the Prospectus: (i) there has been no material adverse change, or any development that would reasonably be expected to result in a material adverse change, in the condition, financial or otherwise, or in the earnings, business, properties, operations, assets, liabilities or prospects, whether or not arising from transactions in the ordinary course of business, of the Company and its subsidiaries, considered as one entity (any such change being referred to herein as a "**Material Adverse Change**"); (ii) the Company and its subsidiaries, considered as one entity, have not incurred any material liability or obligation, indirect, direct or contingent, including without limitation any losses or interference with their business from fire, explosion, flood, earthquakes, accident or other calamity, whether or not covered by insurance, or from any strike, labor dispute or court or governmental action, order or decree, that are material, individually or in the aggregate, to the Company and its subsidiaries, considered as one entity, and have not entered into any transactions not in the ordinary course of business; and (iii) there has not been any material decrease in the capital stock or any material increase in any short-term or long-term indebtedness of the Company or its subsidiaries and there has been no dividend or distribution of any kind declared, paid or made by the Company or, except for dividends paid to the Company or other subsidiaries, by any of the Company's subsidiaries on any class of capital stock, or any repurchase or redemption by the Company or any of its subsidiaries of any class of capital stock.

(h) Independent Accountants. Ernst & Young LLP, which has expressed its opinion with respect to certain of the financial statements (which term as used in this Agreement includes the related notes thereto) filed with the Commission as a part of the Registration Statement and the Prospectus, is (i) an independent registered public accounting firm as required by the Securities Act, the Exchange Act, and the rules of the Public Company Accounting Oversight Board ("**PCAOB**"), (ii) in compliance with the applicable requirements relating to the qualification of accountants under Rule 2-01 of Regulation S-X under the Securities Act and (iii) a registered public accounting firm as defined by the PCAOB whose registration has not been suspended or revoked and who has not requested such registration to be withdrawn. BDO USA, LLP which has expressed its opinion with respect to certain of the financial statements (which term as used in this Agreement includes the related notes thereto) filed with the Commission as a part of the Registration Statement and the Prospectus, is (i) an independent registered public accounting firm as required by the Securities Act, the Exchange Act, and the rules of the PCAOB, (ii) in compliance with the applicable requirements relating to the qualification of accountants under Rule 2-01 of Regulation S-X under the Securities Act and (iii) a registered public accounting firm as defined by the PCAOB whose registration has not been suspended or revoked and who has not requested such registration to be withdrawn.

(i) Financial Statements. The financial statements filed with the Commission as a part of the Registration Statement and the Prospectus present fairly, in all material respects, the consolidated financial position of the Company and its subsidiaries as of the dates indicated and the results of their operations, changes in stockholders' equity and cash flows for the periods specified. Such financial statements have been prepared in conformity with generally accepted accounting principles as applied in the United States applied on a consistent basis throughout the

periods involved, except as may be expressly stated in the related notes thereto and except in the case of unaudited financial statements, which are subject to normal and recurring year-end adjustments and do not contain certain footnotes as permitted by the applicable rules of the Commission. The interactive data in eXtensible Business Reporting Language included or incorporated by reference in the Registration Statement fairly presents the information called for in all material respects and has been prepared in accordance with the Commission's rules and guidelines applicable thereto. No other financial statements or supporting schedules are required to be included in the Registration Statement or the Prospectus. The financial data set forth in each of the Registration Statement and the Prospectus fairly present, in all material respects, the information set forth therein on a basis consistent with that of the audited or unaudited financial statements, as applicable, contained in the Registration Statement and the Prospectus. To the Company's knowledge, no person who has been suspended or barred from being associated with a registered public accounting firm, or who has failed to comply with any sanction pursuant to Rule 5300 promulgated by the PCAOB, has participated in the preparation of, or audited, the financial statements, supporting schedules or other financial data filed with the Commission as a part of the Registration Statement and the Prospectus.

(j) Company's Accounting System. The Company and each of its subsidiaries make and keep accurate books and records and maintain a system of internal accounting controls sufficient to provide reasonable assurance that: (i) transactions are executed in accordance with management's general or specific authorization; (ii) transactions are recorded as necessary to permit preparation of financial statements in conformity with generally accepted accounting principles as applied in the United States and to maintain accountability for assets; (iii) access to assets is permitted only in accordance with management's general or specific authorization; (iv) the recorded accountability for assets is compared with existing assets at reasonable intervals and appropriate action is taken with respect to any differences; and (v) the interactive data in eXtensible Business Reporting Language included or incorporated by reference in the Registration Statement and the Prospectus fairly presents the information called for in all material respects and is prepared in accordance with the Commission's rules and guidelines applicable thereto.

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(k) Disclosure Controls and Procedures; Deficiencies in or Changes to Internal Control Over Financial Reporting. Except as otherwise disclosed in the Registration Statement and the Prospectus, the Company has established and maintains disclosure controls and procedures (as defined in Rules 13a-15 and 15d-15 under the Exchange Act), which (i) are designed to ensure that material information relating to the Company, including its consolidated subsidiaries, is made known to the Company's principal executive officer and its principal financial officer by others within those entities, particularly during the periods in which the periodic reports required under the Exchange Act are being prepared; (ii) have been evaluated by management of the Company for effectiveness as of the end of the Company's most recent fiscal quarter; and (iii) are effective in all material respects to perform the functions for which they were established. Since the end of the Company's most recent audited fiscal year, there have been no significant deficiencies or material weaknesses in the Company's internal control over financial reporting (whether or not remediated) and no change in the Company's internal control over financial reporting that has materially affected, or is reasonably likely to materially affect,

the Company's internal control over financial reporting. The Company is not aware of any change in its internal control over financial reporting that has occurred during its most recent fiscal quarter that has materially affected, or is reasonably likely to materially affect, the Company's internal control over financial reporting.

(l) Incorporation and Good Standing of the Company. The Company has been duly incorporated and is validly existing as a corporation in good standing under the laws of the jurisdiction of its incorporation and has the corporate power and authority to own, lease and operate its properties and to conduct its business as described in the Registration Statement and the Prospectus and to enter into and perform its obligations under this Agreement. The Company is duly qualified as a foreign corporation to transact business and is in good standing in the State of California and each other jurisdiction in which such qualification is required, whether by reason of the ownership or leasing of property or the conduct of business, except where the failure to be so qualified or in good standing, as the case may be, would not, individually or in the aggregate, have a Material Adverse Effect (as defined below).

(m) Subsidiaries. Each of the Company's "subsidiaries" (for purposes of this Agreement, as defined in Rule 405 under the Securities Act) has been duly incorporated or organized, as the case may be, and is validly existing and in good standing (where such concept is recognized) under the laws of the jurisdiction of its incorporation or organization and has the power and authority (corporate or other) to own, lease and operate its properties and to conduct its business as described in the Registration Statement and the Prospectus. Each of the Company's subsidiaries is duly qualified to transact business and is in good standing (where such concept is recognized) in each jurisdiction in which such qualification is required, whether by reason of the ownership or leasing of property or the conduct of business, except where the failure to be so qualified or in good standing, as the case may be, would not, individually or in the aggregate, have a Material Adverse Effect. All of the issued and outstanding capital stock or other equity or ownership interests of each of the Company's subsidiaries have been duly authorized and validly issued, are fully paid and nonassessable and are owned by the Company, directly or through subsidiaries, free and clear of any security interest, mortgage, pledge, lien, encumbrance or adverse claim. The Company does not own or control, directly or indirectly, any corporation, association or other entity other than the subsidiaries listed in Exhibit 21.1 to the Company's most recent Annual Report on Form 10-K.

(n) Capitalization and Other Capital Stock Matters. The authorized, issued and outstanding capital stock of the Company is as set forth in the Registration Statement and the Prospectus under the caption "Description of Capital Stock" (other than for subsequent issuances, if any, pursuant to employee benefit plans, or upon the exercise of outstanding options or warrants, in each case described in the Registration Statement and the Prospectus). The Common Shares (including the Shares, when issued pursuant to the terms of this Agreement) conform in all material respects to the description thereof contained in the Prospectus. All of the issued and outstanding Common Shares have been duly authorized and validly issued, are fully paid and nonassessable and have been issued in compliance with all applicable federal and state securities laws. None of the outstanding Common Shares were issued in violation of any preemptive rights, rights of first refusal or other similar rights to subscribe for or purchase

securities of the Company. There are no authorized or outstanding options, warrants, preemptive rights, rights of first refusal or other rights to purchase, or equity or debt securities convertible into or exchangeable or exercisable for, any capital stock of the Company or any of its subsidiaries other than those described in the Registration Statement and the Prospectus. The descriptions of the Company's stock option, stock bonus and other stock plans or arrangements, and the options or other rights granted thereunder, set forth in the Registration Statement and the Prospectus accurately and fairly presents, in all material respects, the information required to be shown with respect to such plans, arrangements, options and rights.

(o) Stock Exchange Listing. The Common Shares are registered pursuant to Section 12(b) of the Exchange Act and are listed on the Principal Market, and the Company has taken no action designed to, or likely to have the effect of, terminating the registration of the Common Shares under the Exchange Act or delisting the Common Shares from the Principal Market, nor has the Company received any notification that the Commission or the Principal Market is contemplating terminating such registration or listing. To the Company's knowledge, it is in compliance with all applicable listing requirements of the Principal Market.

(p) Non-Contravention of Existing Instruments; No Further Authorizations or Approvals Required. Neither the Company nor any of its subsidiaries is in violation of its charter or by-laws, partnership agreement or operating agreement or similar organizational documents, as applicable,

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or is in default (or, with the giving of notice or lapse of time, would be in default) ("**Default**") under any indenture, loan, credit agreement, note, lease, license agreement, contract, franchise or other instrument (including, without limitation, any pledge agreement, security agreement, mortgage or other instrument or agreement evidencing, guaranteeing, securing or relating to indebtedness) to which the Company or any of its subsidiaries is a party or by which it or any of them may be bound, or to which any of their respective properties or assets are subject (each, an "**Existing Instrument**"), except for such Defaults as could not be expected, individually or in the aggregate, to have a material adverse effect on the condition (financial or other), earnings, business, properties, operations, assets, liabilities or prospects of the Company and its subsidiaries, considered as one entity (a "**Material Adverse Effect**"). The Company's execution, delivery and performance of this Agreement, consummation of the transactions contemplated hereby and by the Registration Statement and the Prospectus and the issuance and sale of the Shares (including the use of proceeds from the sale of the Shares as described in the Registration Statement and the Prospectus under the caption "Use of Proceeds") (i) have been duly authorized by all necessary corporate action and will not result in any violation of the provisions of the charter or by-laws, partnership agreement or operating agreement or similar organizational documents, as applicable, of the Company or any subsidiary, (ii) will not conflict with or constitute a breach of, or Default or a Debt Repayment Triggering Event (as defined below) under, or result in the creation or imposition of any lien, charge or encumbrance upon any property or assets of the Company or any of its subsidiaries pursuant to, or require the consent of any other party to, any Existing Instrument and (iii) will not result in any violation of any law, administrative regulation or administrative or court decree applicable to the Company or any of its subsidiaries. No consent, approval, authorization or other order of, or registration or filing with, any court or other governmental or regulatory authority or agency, is required for the Company's execution, delivery and performance of this Agreement, issuance of the Shares and

consummation of the transactions contemplated by this Agreement and by the Registration Statement and the Prospectus, except such as have been obtained or made by the Company and are in full force and effect under the Securities Act and such as may be required under applicable state securities or blue sky laws or FINRA (as defined below). As used herein, a "**Debt Repayment Triggering Event**" means any event or condition which gives, or with the giving of notice or lapse of time would give, the holder of any note, debenture or other evidence of indebtedness (or any person acting on such holder's behalf) the right to require the repurchase, redemption or repayment of all or a portion of such indebtedness by the Company or any of its subsidiaries.

(q) Compliance with Laws. The Company and its subsidiaries have been and are in compliance with all applicable laws, rules and regulations, except where failure to be so in compliance could not be expected, individually or in the aggregate, to have a Material Adverse Effect.

(r) No Material Actions or Proceedings. Except as otherwise disclosed in the Prospectus, there is no action, claim, suit, demand, hearing, arbitration, enforcement, investigation, subpoena, notice of violation or deficiency, proceeding, inquiry or investigation (collectively, "**Proceedings**") brought by or before any governmental entity now pending or, to the knowledge of the Company, threatened, against or affecting the Company or any of its subsidiaries, which, could be expected, individually or in the aggregate, to have a Material Adverse Effect or materially and adversely affect the consummation of the transactions contemplated by this Agreement or the performance by the Company of its obligations under this Agreement; and the aggregate of all pending legal or governmental proceedings to which the Company or any such subsidiary is a party or of which any of their respective properties or assets is the subject, including ordinary routine litigation incidental to the business, if determined adversely to the Company, could not be expected to have a Material Adverse Effect. No material labor dispute with the employees of the Company or any of its subsidiaries, or with the employees of any principal supplier, manufacturer, customer or contractor of the Company, exists or, to the knowledge of the Company, is threatened or imminent.

(s) Intellectual Property Rights. Except as otherwise disclosed in the Registration Statement or the Prospectus, the Company and its subsidiaries own, or have obtained valid and enforceable licenses for, the inventions, patent applications, patents, trademarks, trade names, service names, copyrights, trade secrets and other intellectual property described in the Registration Statement and the Prospectus as being owned or licensed by them or which are necessary for the conduct of their respective businesses as currently conducted or as currently proposed to be conducted (collectively, "**Intellectual Property**"), except to the extent the failure to own, possess or license such Intellectual Property would not be expected, individually or in the aggregate, to have a Material Adverse Effect, and the conduct of their respective businesses does not and will not infringe, misappropriate or otherwise conflict in any material respect with any such rights of others. The Intellectual Property of the Company has not been adjudged by a court of competent jurisdiction to be invalid or unenforceable, in whole or in part, and the Company is unaware of any facts which would form a reasonable basis for any such adjudication. To the Company's knowledge: (i) there are no third parties who have rights to any

Intellectual Property, except for customary reversionary rights of third-party licensors or rights of third-party licensees as applicable with respect to Intellectual Property that is disclosed in the Registration Statement and the Prospectus as licensed to the Company or one or more of its subsidiaries; and (ii) there is no infringement by third parties of any Intellectual Property. There is no pending or, to the Company's knowledge, threatened action, suit, proceeding or claim by others: (A) challenging the Company's rights in or to any Intellectual Property, and the Company is unaware of any facts which would form a reasonable basis for any such action, suit, proceeding or claim; (B) challenging the validity, enforceability or scope of any Intellectual Property, and the Company is unaware of any facts which would form a reasonable basis for any such action, suit, proceeding or claim; or (C) asserting that the Company or any of its subsidiaries infringes or otherwise violates, or would, upon the commercialization of any product or service described in the Registration Statement or the Prospectus as under development, infringe or violate, any patent, trademark, trade name,

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service name, copyright, trade secret or other proprietary rights of others, and the Company is unaware of any facts which would form a reasonable basis for any such action, suit, proceeding or claim. The Company and its subsidiaries have complied in all material respects with the terms of each agreement pursuant to which Intellectual Property has been licensed to the Company or any subsidiary, and all such agreements are in full force and effect. To the Company's knowledge, there are no material defects in any of the patents or patent applications included in the Intellectual Property. The Company and its subsidiaries have taken all reasonable steps to protect, maintain and safeguard their Intellectual Property, including the execution of appropriate nondisclosure, confidentiality agreements and invention assignment agreements and invention assignments with their employees, to the Company's knowledge, and no employee of the Company is in or has been in violation of any term of any employment contract, patent disclosure agreement, invention assignment agreement, non-competition agreement, non-solicitation agreement, nondisclosure agreement, or any restrictive covenant to or with a former employer where the basis of such violation relates to such employee's employment with the Company. The duty of candor and good faith as required by the United States Patent and Trademark Office during the prosecution of the United States patents and patent applications included in the Intellectual Property have been complied with; and in all foreign offices having similar requirements, all such requirements have been complied with. None of the Company owned Intellectual Property or technology (including information technology and outsourced arrangements) employed by the Company or its subsidiaries has been obtained or is being used by the Company or its subsidiary in violation of any contractual obligation binding on the Company or its subsidiaries or any of their respective officers, directors or employees or otherwise in violation of the rights of any persons. The product candidates described in the Registration Statement and the Prospectus as under development by the Company or any subsidiary fall within the scope of claims of one or more patents owned by, or exclusively licensed to, the Company or any subsidiary.

(t) All Necessary Permits, etc. Except as otherwise disclosed in the Prospectus, the Company and its subsidiaries possess, and are operating in compliance with, such valid and current certificates, authorizations or permits required by state, federal or foreign regulatory agencies or bodies to conduct their respective businesses as currently conducted and as described in the Registration Statement or the Prospectus ("**Permits**"). Neither the Company nor any of its subsidiaries is in violation of, or in default under, any of the Permits or has received any notice

of proceedings relating to the revocation or modification of, or non-compliance with, any such certificate, authorization or permit.

(u) Title to Properties. Except as otherwise disclosed in the Prospectus, the Company and its subsidiaries have good and marketable title to all of the real and personal property and other assets reflected as owned in the financial statements referred to in Section 2(i) above (or elsewhere in the Registration Statement or the Prospectus), in each case free and clear of any security interests, mortgages, liens, encumbrances, equities, adverse claims and other defects. The real property, improvements, equipment and personal property held under lease by the Company or any of its subsidiaries are held under valid and enforceable leases, with such exceptions as are not material and do not materially interfere with the use made or proposed to be made of such real property, improvements, equipment or personal property by the Company or such subsidiary.

(v) Tax Law Compliance. The Company and its subsidiaries have filed all necessary federal, state and foreign income and franchise tax returns or have properly requested extensions thereof and have paid all taxes required to be paid by any of them and, if due and payable, any related or similar assessment, fine or penalty levied against any of them except as may be being contested in good faith and by appropriate proceedings. The Company has made adequate charges, accruals and reserves in the applicable financial statements referred to in Section 2(i) above in respect of all federal, state and foreign income and franchise taxes for all periods as to which the tax liability of the Company or any of its subsidiaries has not been finally determined.

(w) Insurance. Except as otherwise disclosed in the Prospectus, each of the Company and its subsidiaries are insured by recognized, financially sound and reputable institutions with policies in such amounts and with such deductibles and covering such risks as are generally deemed adequate and customary for their businesses including, but not limited to, policies covering real and personal property owned or leased by the Company and its subsidiaries against theft, damage, destruction, acts of vandalism and earthquakes and policies covering the Company and its subsidiaries for product liability claims and clinical trial liability claims. The Company has no reason to believe that it or any of its subsidiaries will not be able (i) to renew its existing insurance coverage as and when such policies expire or (ii) to obtain comparable coverage from similar institutions as may be necessary or appropriate to conduct its business as now conducted and at a cost that could not be expected to have a Material Adverse Effect. Neither the Company nor any of its subsidiaries has been denied any insurance coverage which it has sought or for which it has applied.

(x) Compliance with Environmental Laws. Except as described in the Prospectus and except as could not be expected, individually or in the aggregate, to have a Material Adverse Effect: (i) neither the Company nor any of its subsidiaries is in violation of any federal, state, local or foreign statute, law, rule, regulation, ordinance, code, policy or rule of common law or any judicial or administrative interpretation thereof, including any judicial or administrative order, consent, decree or judgment, relating to pollution or protection of human health, the environment (including, without limitation, ambient air, surface water, groundwater, land surface or subsurface strata) or wildlife, including, without limitation, laws and regulations relating to

the release or threatened release of chemicals, pollutants, contaminants, wastes, toxic substances, hazardous substances, petroleum or petroleum products (collectively, "**Hazardous Materials**") or to the manufacture, processing, distribution, use, treatment, storage, disposal, transport or handling of Hazardous Materials (collectively, "**Environmental Laws**"); (ii) the Company and its subsidiaries have all permits, authorizations and approvals

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required under any applicable Environmental Laws and are each in compliance with their requirements; (iii) there are no pending or, to the knowledge of the Company, threatened administrative, regulatory or judicial actions, suits, demands, demand letters, claims, liens, notices of noncompliance or violation, investigation or proceedings relating to any Environmental Law against the Company or any of its subsidiaries; and (iv) there are no events or circumstances that might reasonably be expected to form the basis of an order for clean-up or remediation, or an action, suit or proceeding by any private party or governmental body or agency, against or affecting the Company or any of its subsidiaries relating to Hazardous Materials or any Environmental Laws.

(y) Periodic Review of Costs of Environmental Compliance. In the ordinary course of its business, the Company conducts a periodic review of the effect of Environmental Laws on the business, operations and properties of the Company and its subsidiaries, in the course of which it identifies and evaluates associated costs and liabilities (including, without limitation, any capital or operating expenditures required for clean-up, closure of properties or compliance with Environmental Laws or any permit, license or approval, any related constraints on operating activities and any potential liabilities to third parties). No facts or circumstances have come to the Company's attention that could result in costs or liabilities that could be expected, individually or in the aggregate, to have a Material Adverse Effect.

(z) ERISA Compliance. Except as otherwise disclosed in the Prospectus, the Company and its subsidiaries and any "employee benefit plan" (as defined under the Employee Retirement Income Security Act of 1974, as amended, and the regulations and published interpretations thereunder (collectively, "**ERISA**")) established or maintained by the Company, its subsidiaries or their "ERISA Affiliates" (as defined below) are in compliance in all material respects with ERISA. "**ERISA Affiliate**" means, with respect to the Company or any of its subsidiaries, any member of any group of organizations described in Sections 414(b), (c), (m) or (o) of the Internal Revenue Code of 1986, as amended, and the regulations and published interpretations thereunder (the "Code") of which the Company or such subsidiary is a member. No "reportable event" (as defined under ERISA) has occurred or is reasonably expected to occur with respect to any "employee benefit plan" established or maintained by the Company, its subsidiaries or any of their ERISA Affiliates. No "employee benefit plan" established or maintained by the Company, its subsidiaries or any of their ERISA Affiliates, if such "employee benefit plan" were terminated, would have any "amount of unfunded benefit liabilities" (as defined under ERISA). Neither the Company, its subsidiaries nor any of their ERISA Affiliates has incurred or reasonably expects to incur any liability under (i) Title IV of ERISA with respect to termination of, or withdrawal from, any "employee benefit plan" or (ii) Sections 412, 4971, 4975 or 4980B of the Code. Each "employee benefit plan" established or maintained by the Company, its subsidiaries or any of their ERISA Affiliates that is intended to be qualified under Section 401(a) of the Code is so qualified and nothing has occurred, whether by action or failure to act, which would reasonably be expected to cause the loss of such qualification.

(aa) Company Not an "Investment Company." The Company is not, and will not be, either after receipt of payment for the Shares or after the application of the proceeds therefrom as described under "Use of Proceeds" in the Registration Statement or the Prospectus, required to register as an "investment company" under the Investment Company Act of 1940, as amended (the "**Investment Company Act**").

(bb) No Price Stabilization or Manipulation; Compliance with Regulation M. Neither the Company nor any of its subsidiaries has taken, directly or indirectly, any action designed to or that would reasonably be expected to cause or result in stabilization or manipulation of the price of the Common Shares or of any "reference security" (as defined in Rule 100 of Regulation M under the Exchange Act ("**Regulation M**")) with respect to the Common Shares, whether to facilitate the sale or resale of the Shares or otherwise, and has taken no action which would directly or indirectly violate Regulation M.

(cc) Related-Party Transactions. There are no business relationships or related-party transactions involving the Company or any of its subsidiaries or any other person required to be described in the Registration Statement or the Prospectus that have not been described as required.

(dd) FINRA Matters. All of the information provided to the Agent or to counsel for the Agent by the Company, its counsel, its officers and directors and the holders of any securities (debt or equity) or options to acquire any securities of the Company in connection with the offering of the Shares is true, complete, correct and compliant with Financial Industry Regulatory Authority, Inc.'s ("**FINRA**") rules and any letters, filings or other supplemental information provided to FINRA pursuant to FINRA Rules or NASD Conduct Rules is true, complete and correct. The Company meets the requirements for use of Form S-3 under the Securities Act specified in FINRA Rule 5110(b)(7)(C)(i).

(ee) Statistical and Market Related Data. All statistical, demographic and market-related data included in the Registration Statement or the Prospectus are based on or derived from sources that the Company believes, after reasonable inquiry, to be reliable and accurate. To the extent required, the Company has obtained the written consent to the use of such data from such sources.

(ff) Termination of Contracts or Agreements. Except as described or incorporated by reference in the Registration Statement or the Prospectus, neither the Company nor any of its subsidiaries has sent or received any communication regarding termination of, or intent not to renew, any of the contracts or agreements referred to or described in any preliminary prospectus, the Prospectus or any free writing prospectus, or referred to or described in, or filed as an exhibit to, the Registration Statement, or any document incorporated by reference therein, and no such termination or non-

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renewal has been threatened by the Company or any of its subsidiaries or, to the Company's knowledge, any other party to any such contract or agreement, which threat of termination or non-renewal has not been rescinded as of the date hereof.

(gg) No Unlawful Contributions or Other Payments. Except as otherwise disclosed in the Prospectus, neither the Company nor any of its subsidiaries nor, to the best of the Company's

knowledge, any employee or agent of the Company or any subsidiary, has made any contribution or other payment to any official of, or candidate for, any federal, state or foreign office in violation of any law or of the character required to be disclosed in the Registration Statement or the Prospectus.

(hh) Anti-Corruption and Anti-Bribery Laws. Neither the Company nor any of its subsidiaries nor any director, officer, or employee of the Company or any of its subsidiaries, nor to the knowledge of the Company, any agent, affiliate or other person acting on behalf of the Company or any of its subsidiaries has, in the course of its actions for, or on behalf of, the Company or any of its subsidiaries (i) used any corporate funds for any unlawful contribution, gift, entertainment or other unlawful expenses relating to political activity; (ii) made or taken any act in furtherance of an offer, promise, or authorization of any direct or indirect unlawful payment or benefit to any foreign or domestic government official or employee, including of any government-owned or controlled entity or public international organization, or any political party, party official, or candidate for political office; (iii) violated or is in violation of any provision of the U.S. Foreign Corrupt Practices Act of 1977, as amended (the "**FCPA**"), the UK Bribery Act 2010, or any other applicable anti-bribery or anti-corruption law; or (iv) made, offered, authorized, requested, or taken an act in furtherance of any unlawful bribe, rebate, payoff, influence payment, kickback or other unlawful payment or benefit. The Company and its subsidiaries and, to the knowledge of the Company, the Company's affiliates acting on behalf of the Company have conducted their respective businesses in compliance with the FCPA and have instituted and maintain policies and procedures designed to ensure, and which are reasonably expected to continue to ensure, continued compliance therewith.

(ii) Money Laundering Laws. The operations of the Company and its subsidiaries are, and have been conducted at all times, in compliance with applicable financial recordkeeping and reporting requirements of the Currency and Foreign Transactions Reporting Act of 1970, as amended, the money laundering statutes of all applicable jurisdictions, the rules and regulations thereunder and any related or similar applicable rules, regulations or guidelines, issued, administered or enforced by any governmental agency (collectively, the "**Money Laundering Laws**") and no action, suit or proceeding by or before any court or governmental agency, authority or body or any arbitrator involving the Company or any of its subsidiaries with respect to the Money Laundering Laws is pending or, to the best knowledge of the Company, threatened.

(jj) Sanctions. Neither the Company nor any of its subsidiaries, directors, officers, or employees, nor, to the knowledge of the Company, after due inquiry, any agent, affiliate or other person acting on behalf of the Company or any of its subsidiaries is currently the subject or the target of any U.S. sanctions administered by the Office of Foreign Assets Control of the U.S. Department of the Treasury or the U.S. Department of State, the United Nations Security Council, the European Union, His Majesty's Treasury of the United Kingdom, or other relevant sanctions authority (collectively, "**Sanctions**"); nor is the Company or any of its subsidiaries located, organized or resident in a country or territory that is the subject or the target of Sanctions, including, without limitation, the Crimea, Kherson and Zaporizhzhia Regions of Ukraine, the so-called Donetsk People's Republic, the so-called Luhansk People's Republic, Cuba, Iran, North Korea, and Syria (collectively, "**Sanctioned Countries**"); and the Company

will not directly or indirectly use the proceeds of this offering, or lend, contribute or otherwise make available such proceeds to any subsidiary, or any joint venture partner or other person or entity, for the purpose of financing the activities of or business with any person, or in any country or territory, that at the time of such financing, is the subject or the target of Sanctions or in any other manner that will result in a violation by any person (including any person participating in the transaction whether as underwriter, advisor, investor or otherwise) of applicable Sanctions. Since April 24, 2019, the Company and its subsidiaries have not knowingly engaged in and are not now knowingly engaged in any dealings or transactions with any person that at the time of the dealing or transaction is or was the subject or the target of Sanctions or with any Sanctioned Country.

(kk) Brokers. Except pursuant to this Agreement, there is no broker, finder or other party that is entitled to receive from the Company any brokerage or finder's fee or other fee or commission as a result of any transactions contemplated by this Agreement.

(ll) Forward-Looking Statements. Each financial or operational projection or other "forward-looking statement" (as defined by Section 27A of the Securities Act or Section 21E of the Exchange Act) contained in the Registration Statement or the Prospectus (i) was so included by the Company in good faith and with reasonable basis after due consideration by the Company of the underlying assumptions, estimates and other applicable facts and circumstances and (ii) is accompanied by meaningful cautionary statements identifying those factors that could cause actual results to differ materially from those in such forward-looking statement. No such statement was made with the knowledge of an executive officer or director of the Company that is was false or misleading.

(mm) No Outstanding Loans or Other Extensions of Credit. The Company does not have any outstanding extension of credit, in the form of a personal loan, to or for any director or executive officer (or equivalent thereof) of the Company except for such extensions of credit as are expressly permitted by Section 13(k) of the Exchange Act.

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(nn) Regulatory Compliance. Except as described in the Registration Statement and Prospectus, each of the Company and its subsidiaries: (A) is and at all times has been in compliance with all statutes, rules or regulations of the U.S. Food and Drug Administration (the "**FDA**") and any other comparable U.S. or non-U.S. federal, state, local or other governmental or regulatory authority, governmental or regulatory agency or body, court, arbitrator or self-regulatory organization (each, a "**Governmental Authority**") applicable to the ownership, testing, development, manufacture, packaging, processing, use, marketing, distribution, storage, import, export or disposal of any product under development, manufactured or distributed by the Company or each such subsidiary (collectively, the "**Applicable Laws**"), except where such noncompliance would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect; (B) has not had any product or manufacturing site (whether Company- or subsidiary-owned or that of a contract manufacturer for Company or subsidiary products) subject to a Governmental Authority (including the FDA) shutdown or import or export prohibition, nor received any FDA Form 483 or other Governmental Authority notice of inspectional observations, warning letter, untitled letter or other correspondence or notice from

the FDA or any Governmental Authority alleging or asserting material noncompliance with any Applicable Laws or any licenses, certificates, approvals, clearances, exemptions, authorizations, permits and supplements or amendments thereto required by any such Applicable Laws (collectively, the "**Authorizations**"); (C) possesses all Authorizations, except where failure to possess an Authorization would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect, and such Authorizations are valid and in full force and effect and the Company or such subsidiary is in compliance with the terms of such Authorizations and has fulfilled and performed all of its obligations with respect to such Authorizations, except where such noncompliance or failure to fulfill or perform such obligations would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect; (D) has not received notice that the FDA or any Governmental Authority has taken, is taking or intends to take action to limit, suspend, modify or revoke any material Authorizations, has no knowledge that the FDA or any Governmental Authority is considering such action and has no knowledge that any event has occurred which allows, or after notice or lapse of time would allow, revocation or termination thereof or results in any other material impairment of the rights of the holder of any Authorizations; and (E) has filed, obtained, maintained or submitted all reports, documents, forms, notices, applications, records, claims, data, submissions and supplements or amendments as required by any Applicable Laws or Authorizations except where failure to do so would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect and that all such reports, documents, forms, notices, applications, records, claims, data, submissions and supplements or amendments were materially complete, true and correct on the date filed (or were corrected or supplemented by a subsequent submission).

(oo) Clinical Data. The studies, tests and preclinical and clinical trials conducted by, sponsored by or, to the Company's knowledge, conducted on behalf of the Company and its subsidiaries that are described in the Registration Statement and the Prospectus, or the results of which are referred to in the Registration Statement and the Prospectus, as applicable, were and, if still ongoing, are being conducted in all material respects in accordance with Applicable Laws, including, without limitation, the Federal Food, Drug and Cosmetic Act and the rules and regulations promulgated thereunder at 21 C.F.R. Parts 50, 54, 56, 58 and 312; the descriptions of such studies, tests and trials, including any related results and regulatory status, contained in the Registration Statement and the Prospectus are accurate and complete in all material respects and fairly present the data derived from such studies, tests and trials; except to the extent disclosed in the Registration Statement and the Prospectus, the Company is not aware of any studies, tests or trials, the results of which reasonably call into question the study, test, or trial results described or referred to in the Registration Statement and the Prospectus when viewed in the context in which such results are described and the clinical state of development; and, except to the extent disclosed in the Registration Statement and the Prospectus, the Company has not received any notices or correspondence from the FDA or any other Governmental Authority requiring the termination or suspension of any studies, tests or preclinical or clinical trials conducted by, sponsored by or conducted on behalf of the Company that are described in the Registration Statement and the Prospectus or the results of which are referred to in the Registration Statement and the Prospectus, other than ordinary course communications with respect to modifications in connection with the design and implementation of such trials, and neither the FDA nor any other Governmental Authority has commenced, or, to the Company's knowledge, threatened to

initiate, any action to place a clinical hold order on, or otherwise terminate, delay or suspend such studies, tests or preclinical or clinical trials.

(pp) Safety Notices. Except as described in the Registration Statement and the Prospectus: (A) there have been no recalls, field notifications, field corrections, market withdrawals or replacements, warnings, "dear doctor" letters, investigator notices, safety alerts or other notices of action relating to an alleged lack of safety, efficacy or regulatory compliance of the Company products (collectively, "**Safety Notices**") that remain unresolved or open; and (B) to the Company's knowledge, there are no material complaints with respect to the Company products that are currently unresolved. To the Company's knowledge, there are no facts that would be reasonably likely to result in: (A) a material Safety Notice with respect to the Company products; (B) a material change in labeling of any of the Company products; or (C) a termination or suspension of marketing or testing of any of the Company products.

(qq) Compliance with Health Care Laws. Each of the Company and its subsidiaries is, and has been, in compliance in all respects with all applicable Health Care Laws (as defined below), except where such noncompliance would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect, and there are no facts or circumstances that could reasonably be expected to be result in false claims liability, civil penalties, or mandatory or permissive exclusion from Medicare, Medicaid, or any other government health care program. For purposes of this

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Agreement, "**Health Care Laws**" means: (i) the Federal Food, Drug, and Cosmetic Act (21 U.S.C. §§ 301 et seq.) and the regulations promulgated thereunder; (ii) all applicable federal, state, local and all applicable foreign health care related fraud and abuse laws, including, without limitation, the U.S. Anti-Kickback Statute (42 U.S.C. Section 1320a-7b(b)), the U.S. Physician Payments Sunshine Act (42 U.S.C. § 1320a-7h), the U.S. Civil False Claims Act (31 U.S.C. Section 3729 et seq.), the criminal False Claims Law (42 U.S.C. § 1320a-7b(a)), all criminal laws relating to health care fraud and abuse, including but not limited to 18 U.S.C. Sections 286 and 287, and the health care fraud criminal provisions under the U.S. Health Insurance Portability and Accountability Act of 1996 ("**HIPAA**") (42 U.S.C. Section 1320d et seq.), the exclusions laws (42 U.S.C. § 1320a-7), the civil monetary penalties law (42 U.S.C. § 1320a-7a), HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act (42 U.S.C. Section 17921 et seq.), and the regulations promulgated pursuant to such statutes; (iii) Medicare (Title XVIII of the Social Security Act); (iv) Medicaid (Title XIX of the Social Security Act); and (v) any and all other applicable health care laws and regulations, including the collection and reporting requirements, and the processing of any applicable rebate, chargeback or adjustment, under applicable rules and regulations relating to the Medicaid Drug Rebate Program (42 U.S.C. § 1396r-8) and any state supplemental rebate program, Medicare average sales price reporting (42 U.S.C. § 1395w-3a), the VA Federal Supply Schedule (38 U.S.C. § 8126) or under any state pharmaceutical assistance program or U.S. Department of Veterans Affairs agreement, and any successor government programs. Neither the Company nor any of its subsidiaries has received notice of any Proceeding from any Governmental Authority or third party alleging that any product operation or activity is in material violation of any Health Care Laws, and, to the Company's knowledge, no such Proceeding is threatened. Neither the Company nor any of its subsidiaries is a party to or has any ongoing reporting obligations pursuant to any corporate

integrity agreements, deferred prosecution agreements, monitoring agreements, consent decrees, settlement orders, plans of correction or similar agreements with or imposed by the FDA or any committee thereof or from any other U.S. or foreign government or drug or medical device regulatory agency, or health care facility Institutional Review Board or other Governmental Authority. Additionally, neither the Company, its subsidiaries nor the employees, officers or directors of the Company or its subsidiaries have been excluded, suspended or debarred from, or are otherwise ineligible for participation in, any U.S. federal health care program or human clinical research; nor, to the knowledge of the Company, are they subject to any Proceeding by a Governmental Authority that could reasonably be expected to result in debarment, suspension, exclusion, or other ineligibility.

(rr) Incorporated Documents. The documents incorporated or deemed to be incorporated by reference in the Registration Statement and the Prospectus, at the time they were filed with the Commission, complied in all material respects with the requirements of the Exchange Act, as applicable, and, when read together with the other information in the Prospectus, do not contain an untrue statement of a material fact or omit to state a material fact required to be stated therein or necessary to make the statements therein, in light of the circumstances under which they were made, not misleading.

(ss) Exchange Act Compliance. The documents incorporated or deemed to be incorporated by reference in the Prospectus, at the time they were or hereafter are filed with the Commission, and any Free Writing Prospectus or amendment or supplement thereto complied and will comply in all material respects with the requirements of the Exchange Act, and, when read together with the other information in the Prospectus, at the time the Registration Statement and any amendments thereto become effective and at each Time of Sale, as the case may be, will not contain an untrue statement of a material fact or omit to state a material fact required to be stated therein or necessary to make the fact required to be stated therein or necessary to make the statements therein, in the light of the circumstances under which they were made, not misleading.

(tt) Sarbanes-Oxley. The Company is in compliance, in all material respects, with all applicable provisions of the Sarbanes-Oxley Act of 2002 and the rules and regulations promulgated thereunder.

(uu) Duties, Transfer Taxes, Etc. No stamp or other issuance or transfer taxes or duties and no capital gains, income, withholding or other taxes are payable by the Agent in the United States or any political subdivision or taxing authority thereof or therein in connection with the execution, delivery or performance of this Agreement by the Company or the sale and delivery by the Company of the Shares.

(vv) Cybersecurity. To the best of the Company's knowledge, the Company and its subsidiaries' information technology assets and equipment, computers, systems, networks, hardware, software, websites, applications, and databases (collectively, "**IT Systems**") are adequate for, and operate and perform in all material respects as required in connection with the operation of the business of the Company and its subsidiaries as currently conducted, free and clear of all material bugs, errors, defects, Trojan horses, time bombs, malware and other corruptants. The Company and its subsidiaries have implemented and maintained commercially

reasonable physical, technical and administrative controls, policies, procedures, and safeguards to maintain and protect their material confidential information and the integrity, continuous operation, redundancy and security of all IT Systems and data, including "Personal Data," used in connection with their businesses. "**Personal Data**" means (i) a natural person's name, street address, telephone number, e-mail address, photograph, social security number or tax identification number, driver's license number, passport number, credit card number, bank information, or customer or account number; (ii) any information which would qualify as "personally identifying information" under the Federal Trade Commission Act, as amended; (iii) "personal data" as defined by GDPR (as defined below); (iv) any information which would qualify as "protected health information" under the Health

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Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act (collectively, "**HIPAA**"); and (v) any other piece of information that allows the identification of such natural person, or his or her family, or permits the collection or analysis of any data related to an identified person's health or sexual orientation. There have been no breaches, violations, outages or unauthorized uses of or accesses to same, except for those that have been remedied without material cost or liability or the duty to notify any other person, nor any incidents under internal review or investigations relating to the same, except as would not be expected, individually or in the aggregate, to have a Material Adverse Effect. The Company and its subsidiaries are presently in material compliance with all applicable laws or statutes and all judgments, orders, rules and regulations of any court or arbitrator or governmental or regulatory authority, internal policies and contractual obligations relating to the privacy and security of IT Systems and Personal Data and to the protection of such IT Systems and Personal Data from unauthorized use, access, misappropriation or modification, except as would not be expected, individually or in the aggregate, to have a Material Adverse Effect.

(ww) Compliance with Data Privacy Laws. The Company and its subsidiaries are, and at all prior times were, in compliance with all applicable state and federal data privacy and security laws and regulations, including without limitation HIPAA, and the Company and its subsidiaries have taken commercially reasonable actions to prepare to comply with, and since May 25, 2018, have been and currently are in compliance with, the European Union General Data Protection Regulation ("**GDPR**") (EU 2016/679) (collectively, the "**Privacy Laws**"), except as would not be expected, individually or in the aggregate, to have a Material Adverse Effect. To ensure compliance with the Privacy Laws, the Company and its subsidiaries have in place, comply with, and take appropriate steps reasonably designed to ensure compliance in all material respects with their policies and procedures relating to data privacy and security and the collection, storage, use, disclosure, handling, and analysis of Personal Data (the "**Policies**"). Except as would not be expected, individually or in the aggregate, to have a Material Adverse Effect, the Company and its subsidiaries have at all times made all disclosures to users or customers required by applicable laws and regulatory rules or requirements, and none of such disclosures made or contained in any Policy have, to the knowledge of the Company, been inaccurate or in violation of any applicable laws and regulatory rules or requirements in any respect. Except as would not be expected, individually or in the aggregate, to have a Material Adverse Effect, the Company further certifies that neither it nor any subsidiary: (i) has received notice of any actual or potential liability under or relating to, or actual or potential violation of, any of the Privacy Laws, and has no knowledge of any event or condition that would reasonably

be expected to result in any such notice; (ii) is currently conducting or paying for, in whole or in part, any investigation, remediation, or other corrective action pursuant to any Privacy Law; or (iii) is a party to any order, decree, or agreement that imposes any obligation or liability under any Privacy Law.

(xx) Other Underwriting Agreements. The Company is not a party to any agreement with an agent or underwriter for any other "at the market" or continuous equity transaction.

Any certificate signed by any officer or representative of the Company or any of its subsidiaries and delivered to the Agent or counsel for the Agent in connection with an issuance of Shares shall be deemed a representation and warranty by the Company to the Agent as to the matters covered thereby on the date of such certificate.

The Company acknowledges that the Agent and, for purposes of the opinions to be delivered pursuant to Section 4(p) hereof, counsel to the Company and counsel to the Agent, will rely upon the accuracy and truthfulness of the foregoing representations and hereby consents to such reliance.

Section 3. ISSUANCE AND SALE OF COMMON SHARES

(a) Sale of Securities. On the basis of the representations, warranties and agreements herein contained, but subject to the terms and conditions herein set forth, the Company and the Agent agree that the Company may from time to time seek to sell Shares through the Agent, acting as sales agent, or directly to the Agent, acting as principal, as follows, with an aggregate Sales Price of up to the Maximum Program Amount, based on and in accordance with Issuance Notices as the Company may deliver, during the Agency Period.

(b) Mechanics of Issuances.

(i) Issuance Notice. Upon the terms and subject to the conditions set forth herein, on any Trading Day during the Agency Period on which the conditions set forth in Section 5(a) and Section 5(b) shall have been satisfied, the Company may exercise its right to request an issuance of Shares by delivering to the Agent an Issuance Notice; *provided, however*, that (A) in no event may the Company deliver an Issuance Notice to the extent that (I) the sum of (x) the aggregate Sales Price of the requested Issuance Amount, plus (y) the aggregate Sales Price of all Shares issued under all previous Issuance Notices effected pursuant to this Agreement, would exceed the Maximum Program Amount; and (B) prior to delivery of any Issuance Notice, the period set forth for any previous Issuance Notice shall have expired or been terminated. An Issuance Notice shall be considered delivered on the Trading Day that it is received by e-mail to the persons set forth in Schedule A hereto and confirmed by the Company by telephone (including a voicemail message to the persons so identified), with the understanding that, with adequate prior written notice, the Agent may modify the list of such persons from time to time.

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(ii) Agent Efforts. Upon the terms and subject to the conditions set forth in this Agreement, upon the receipt of an Issuance Notice, the Agent will use its commercially reasonable efforts consistent with its normal sales and trading practices to place the Shares with

respect to which the Agent has agreed to act as sales agent, subject to, and in accordance with the information specified in, the Issuance Notice, unless the sale of the Shares described therein has been suspended, cancelled or otherwise terminated in accordance with the terms of this Agreement. For the avoidance of doubt, the parties to this Agreement may modify an Issuance Notice at any time provided they both agree in writing to any such modification.

(iii) Method of Offer and Sale. The Shares may be offered and sold (A) in negotiated transactions with the consent of the Company or (B) by any other method permitted by law deemed to be an "at the market offering" as defined in Rule 415(a)(4) under the Securities Act, including block transactions, sales made directly on the Principal Market or sales made into any other existing trading market of the Common Shares. Nothing in this Agreement shall be deemed to require either party to agree to the method of offer and sale specified in the preceding sentence, and (except as specified in clause (A) above) the method of placement of any Shares by the Agent shall be at the Agent's discretion.

(iv) Confirmation to the Company. If acting as sales agent hereunder, the Agent will provide written confirmation to the Company on the Trading Day on which it has placed Shares hereunder setting forth the number of shares sold on such Trading Day, the corresponding Sales Price and the Issuance Price payable to the Company in respect thereof.

(v) Settlement. Each issuance of Shares will be settled on the applicable Settlement Date for such issuance of Shares and, subject to the provisions of Section 5, on or before each Settlement Date, the Company will, or will cause its transfer agent to, electronically transfer the Shares being sold by crediting the Agent or its designee's account at The Depository Trust Company through its Deposit/Withdrawal At Custodian (DWAC) System, or by such other means of delivery as may be mutually agreed upon by the parties hereto and, upon receipt of such Shares, which in all cases shall be freely tradable, transferable, registered shares in good deliverable form, the Agent will deliver, by wire transfer of immediately available funds, the related Issuance Price in same day funds delivered to an account designated by the Company prior to the Settlement Date. The Company may sell Shares to the Agent as principal at a price agreed upon at each relevant time Shares are sold pursuant to this Agreement (each, a "**Time of Sale**").

(vi) Suspension or Termination of Sales. Consistent with standard market settlement practices, the Company or the Agent may, upon notice to the other party hereto in writing or by telephone (confirmed immediately by verifiable email), suspend any sale of Shares, and the period set forth in an Issuance Notice shall immediately terminate; *provided, however*, that (A) such suspension and termination shall not affect or impair either party's obligations with respect to any Shares placed or sold hereunder prior to the receipt of such notice; (B) if the Company suspends or terminates any sale of Shares after the Agent confirms such sale to the Company, the Company shall still be obligated to comply with Section 3(b)(v) with respect to such Shares; and (C) if the Company defaults in its obligation to deliver Shares on a Settlement Date, the Company agrees that it will hold the Agent harmless against any loss, claim, damage or expense (including, without limitation, penalties, interest and reasonable legal fees and expenses), as incurred, arising out of or in connection with such default by the Company. The parties hereto

acknowledge and agree that, in performing its obligations under this Agreement, the Agent may borrow Common Shares from stock lenders in the event that the Company has not delivered Shares to settle sales as required by subsection (v) above, and may use the Shares to settle or close out such borrowings. The Company agrees that no such notice shall be effective against the Agent unless it is made to the persons identified in writing by the Agent pursuant to Section 3(b)(i).

(vii) No Guarantee of Placement, Etc. The Company acknowledges and agrees that (A) there can be no assurance that the Agent will be successful in placing Shares; (B) the Agent will incur no liability or obligation to the Company or any other Person if it does not sell Shares; and (C) the Agent shall be under no obligation to purchase Shares on a principal basis pursuant to this Agreement, except as otherwise specifically agreed by the Agent and the Company.

(viii) Material Non-Public Information. Notwithstanding any other provision of this Agreement, the Company and the Agent agree that the Company shall not deliver any Issuance Notice to the Agent, and the Agent shall not be obligated to place any Shares, during any period in which the Company is in possession of material non-public information.

(c) Fees. As compensation for services rendered, the Company shall pay to the Agent, on the applicable Settlement Date, the Selling Commission for the applicable Issuance Amount (including with respect to any suspended or terminated sale pursuant to Section 3(b)(vi)) by the Agent deducting the Selling Commission from the applicable Issuance Amount.

(d) Expenses. The Company agrees to pay all costs, fees and expenses incurred in connection with the performance of its obligations hereunder and in connection with the transactions contemplated hereby, including without limitation (i) all expenses incident to the issuance and delivery of the Shares (including all printing and engraving costs); (ii) all fees and expenses of the registrar and transfer agent of the Shares; (iii) all

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necessary issue, transfer and other stamp taxes in connection with the issuance and sale of the Shares; (iv) all fees and expenses of the Company's counsel, independent public or certified public accountants and other advisors; (v) all costs and expenses incurred in connection with the preparation, printing, filing, shipping and distribution of the Registration Statement (including financial statements, exhibits, schedules, consents and certificates of experts), the Prospectus, any Free Writing Prospectus prepared by or on behalf of, used by, or referred to by the Company, and all amendments and supplements thereto, and this Agreement; (vi) all filing fees, attorneys' fees and expenses incurred by the Company or the Agent in connection with qualifying or registering (or obtaining exemptions from the qualification or registration of) all or any part of the Shares for offer and sale under the state securities or blue sky laws, and, if requested by the Agent, preparing and printing a "**Blue Sky Survey**" or memorandum, and any supplements thereto, advising the Agent of such qualifications, registrations, determinations and exemptions; (vii) the reasonable fees and disbursements of the Agent's counsel, including the reasonable fees and expenses of counsel for the Agent in connection with, FINRA review, if any, and approval of the Agent's participation in the offering and distribution of the Shares; (viii) the filing fees incident to FINRA review, if any; and (ix) the fees and expenses associated with listing the Shares on the Principal Market. The fees and disbursements of the Agent's counsel pursuant to subsections (vi) and (vii) above shall

not exceed (A) $75,000 in connection with the entry into this Agreement, (B) $25,000 in connection with each Triggering Event Date (as defined below) involving the filing of a Form 10-K on which the Company is required to provide a certificate pursuant to Section 4(o), (C) $15,000 in connection with each other Triggering Event Date on which the Company is required to provide a certificate pursuant to Section 4(o), and (D) $40,000 for each program "refresh" (i.e. filing of a new registration statement, prospectus or prospectus supplement relating to the Shares and/or an amendment of this Agreement) executed pursuant to this Agreement.

Section 4. ADDITIONAL COVENANTS

The Company covenants and agrees with the Agent as follows, in addition to any other covenants and agreements made elsewhere in this Agreement:

(a) Exchange Act Compliance. During the Agency Period, the Company shall (i) file, on a timely basis, with the Commission all reports and documents required to be filed under Section 13, 14 or 15 of the Exchange Act in the manner and within the time periods required by the Exchange Act; and (ii) either (A) include in its quarterly reports on Form 10-Q and its annual reports on Form 10-K, a summary detailing, for the relevant reporting period, (1) the number of Shares sold through the Agent pursuant to this Agreement and (2) the net proceeds received by the Company from such sales or, in the Company's sole discretion, (B) prepare a prospectus supplement containing, or include in such other filing permitted by the Securities Act or Exchange Act (each an "**Interim Prospectus Supplement**"), such summary information and, at least once a quarter and subject to this Section 4, file such Interim Prospectus Supplement pursuant to Rule 424(b) under the Securities Act (and within the time periods required by Rule 424(b) and Rule 430B under the Securities Act).

(b) Securities Act Compliance. After the date of this Agreement, the Company shall promptly advise the Agent in writing (i) of the receipt of any comments of, or requests for additional or supplemental information from, the Commission; (ii) of the time and date of any filing of any post-effective amendment to the Registration Statement, any Rule 462(b) Registration Statement or any amendment or supplement to the Prospectus, or any Free Writing Prospectus; (iii) of the time and date that any post-effective amendment to the Registration Statement or any Rule 462(b) Registration Statement becomes effective; and (iv) of the issuance by the Commission of any stop order suspending the effectiveness of the Registration Statement or any post-effective amendment thereto, any Rule 462(b) Registration Statement or any amendment or supplement to the Prospectus or of any order preventing or suspending the use of any Free Writing Prospectus or the Prospectus, or of any proceedings to remove, suspend or terminate from listing or quotation the Common Shares from any securities exchange upon which they are listed for trading or included or designated for quotation, or of the threatening or initiation of any proceedings for any of such purposes. If the Commission shall enter any such stop order at any time, the Company will use its best efforts to obtain the lifting of such order at the earliest possible moment. Additionally, the Company agrees that it shall comply with the provisions of Rule 424(b) and Rule 433, as applicable, under the Securities Act and will use its reasonable efforts to confirm that any filings made by the Company under such Rule 424(b) or Rule 433 were received in a timely manner by the Commission.

(c) Amendments and Supplements to the Prospectus and Other Securities Act Matters. If any event shall occur or condition exist as a result of which it is necessary to amend or supplement the Prospectus so that the Prospectus does not include an untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances when the Prospectus is delivered to a purchaser, not misleading, or if in the opinion of the Agent or counsel for the Agent it is otherwise necessary to amend or supplement the Prospectus to comply with applicable law, including the Securities Act, the Company agrees (subject to Section 4(d) and 4(f)) to promptly prepare, file with the Commission and furnish at its own expense to the Agent, amendments or supplements to the Prospectus so that the statements in the Prospectus as so amended or supplemented will not include an untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances when the Prospectus is delivered to a purchaser, be misleading or so that the Prospectus, as amended or supplemented, will comply with applicable law including the Securities Act. Neither the Agent's consent to, or delivery of, any such amendment or supplement shall constitute a waiver of any of the Company's obligations under Sections 4(d) and 4(f). Notwithstanding the foregoing,

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the Company shall not be required to file such amendment or supplement if there is no pending Issuance Notice and the Company believes that it is in its best interests not to file such amendment or supplement.

(d) Agent's Review of Proposed Amendments and Supplements.Prior to amending or supplementing the Registration Statement (including any registration statement filed under Rule 462(b) under the Securities Act) or the Prospectus (excluding any amendment or supplement through incorporation of any report filed under the Exchange Act), the Company shall furnish to the Agent for review, a reasonable amount of time prior to the proposed time of filing or use thereof, a copy of each such proposed amendment or supplement, and the Company shall not file or use any such proposed amendment or supplement without the Agent's prior consent, and to file with the Commission within the applicable period specified in Rule 424(b) under the Securities Act any prospectus required to be filed pursuant to such Rule.

(e) Use of Free Writing Prospectus. Neither the Company nor the Agent has prepared, used, referred to or distributed, or will prepare, use, refer to or distribute, without the other party's prior written consent, any "written communication" that constitutes a "free writing prospectus" as such terms are defined in Rule 405 under the Securities Act with respect to the offering contemplated by this Agreement (any such free writing prospectus being referred to herein as a "**Free Writing Prospectus**").

(f) Free Writing Prospectuses. The Company shall furnish to the Agent for review, a reasonable amount of time prior to the proposed time of filing or use thereof, a copy of each proposed free writing prospectus or any amendment or supplement thereto to be prepared by or on behalf of, used by, or referred to by the Company and the Company shall not file, use or refer to any proposed free writing prospectus or any amendment or supplement thereto without the Agent's consent. The Company shall furnish to the Agent, without charge, as many copies of any free writing prospectus prepared by or on behalf of, or used by the Company, as the Agent may reasonably request. If at any time when a prospectus is required by the Securities Act (including, without limitation, pursuant to Rule 173(d)) to be delivered in connection with sales

of the Shares (but in any event if at any time through and including the date of this Agreement) there occurred or occurs an event or development as a result of which any free writing prospectus prepared by or on behalf of, used by, or referred to by the Company conflicted or would conflict with the information contained in the Registration Statement or included or would include an untrue statement of a material fact or omitted or would omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances prevailing at that subsequent time, not misleading, the Company shall promptly amend or supplement such free writing prospectus to eliminate or correct such conflict or so that the statements in such free writing prospectus as so amended or supplemented will not include an untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances prevailing at such subsequent time, not misleading, as the case may be; *provided, however*, that prior to amending or supplementing any such free writing prospectus, the Company shall furnish to the Agent for review, a reasonable amount of time prior to the proposed time of filing or use thereof, a copy of such proposed amended or supplemented free writing prospectus and the Company shall not file, use or refer to any such amended or supplemented free writing prospectus without the Agent's consent.

(g) Filing of Agent Free Writing Prospectuses. The Company shall not take any action that would result in the Agent or the Company being required to file with the Commission pursuant to Rule 433(d) under the Securities Act a free writing prospectus prepared by or on behalf of the Agent that the Agent otherwise would not have been required to file thereunder.

(h) Copies of Registration Statement and Prospectus. After the date of this Agreement through the last time that a prospectus is required by the Securities Act (including, without limitation, pursuant to Rule 173(d)) to be delivered in connection with sales of the Shares, the Company agrees to furnish the Agent with copies (which may be electronic copies) of the Registration Statement and each amendment thereto, and with copies of the Prospectus and each amendment or supplement thereto in the form in which it is filed with the Commission pursuant to the Securities Act or Rule 424(b) under the Securities Act, both in such quantities as the Agent may reasonably request from time to time; and, if the delivery of a prospectus is required under the Securities Act or under the blue sky or securities laws of any jurisdiction at any time on or prior to the applicable Settlement Date for any period set forth in an Issuance Notice in connection with the offering or sale of the Shares and if at such time any event has occurred as a result of which the Prospectus as then amended or supplemented would include an untrue statement of a material fact or omit to state any material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made when such Prospectus is delivered, not misleading, or, if for any other reason it is necessary during such same period to amend or supplement the Prospectus or to file under the Exchange Act any document incorporated by reference in the Prospectus in order to comply with the Securities Act or the Exchange Act, to notify the Agent and to request that the Agent suspend offers to sell Shares (and, if so notified, the Agent shall cease such offers as soon as practicable); and if the Company decides to amend or supplement the Registration Statement or the Prospectus as then amended or supplemented, to advise the Agent promptly by telephone (with confirmation in writing) and to prepare and cause to be filed promptly with the Commission an amendment or supplement to the Registration Statement or the Prospectus as then amended or supplemented

that will correct such statement or omission or effect such compliance; provided, however, that if during such same period the Agent is required to deliver a prospectus in respect of transactions in the Shares, the Company shall promptly prepare and file with the Commission such an amendment

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or supplement.

(i) Blue Sky Compliance. The Company shall cooperate with the Agent and counsel for the Agent to qualify or register the Shares for sale under (or obtain exemptions from the application of) the state securities or blue sky laws or Canadian provincial securities laws of those jurisdictions designated by the Agent, shall comply with such laws and shall continue such qualifications, registrations and exemptions in effect so long as required for the distribution of the Shares. The Company shall not be required to qualify as a foreign corporation or to take any action that would subject it to general service of process in any such jurisdiction where it is not presently qualified or where it would be subject to taxation as a foreign corporation. The Company will advise the Agent promptly of the suspension of the qualification or registration of (or any such exemption relating to) the Shares for offering, sale or trading in any jurisdiction or any initiation or threat of any proceeding for any such purpose, and in the event of the issuance of any order suspending such qualification, registration or exemption, the Company shall use its best efforts to obtain the withdrawal thereof at the earliest possible moment.

(j) Earnings Statement. As soon as practicable, the Company will make generally available to its security holders and to the Agent an earnings statement (which need not be audited) covering a period of at least twelve months beginning with the first fiscal quarter of the Company occurring after the date of this Agreement which shall satisfy the provisions of Section 11(a) of the Securities Act and Rule 158 under the Securities Act; provided that the Company will be deemed to have furnished such statement to its security holders and the Agent to the extent they are filed on EDGAR or any successor system.

(k) Listing; Reservation of Shares. (a) The Company will maintain the listing of the Shares on the Principal Market; and (b) the Company will reserve and keep available at all times, free of preemptive rights, Shares for the purpose of enabling the Company to satisfy its obligations under this Agreement.

(l) Transfer Agent. The Company shall engage and maintain, at its expense, a registrar and transfer agent for the Shares.

(m) Due Diligence. During the term of this Agreement, the Company will reasonably cooperate with any reasonable due diligence review conducted by the Agent in connection with the transactions contemplated hereby, including, without limitation, providing information and making available documents and senior corporate officers, during normal business hours and at the Company's principal offices, as the Agent may reasonably request from time to time.

(n) Representations and Warranties. The Company acknowledges that each delivery of an Issuance Notice and each delivery of Shares on a Settlement Date shall be deemed to be (i) an affirmation to the Agent that the representations and warranties of the Company contained in or made pursuant to this Agreement are true and correct as of the date of such Issuance Notice or of such Settlement Date, as the case may be, as though made at and as of each such date, except

as may be disclosed in the Prospectus (including any documents incorporated by reference therein and any supplements thereto); and (ii) an undertaking that the Company will advise the Agent if any of such representations and warranties will not be true and correct as of the Settlement Date for the Shares relating to such Issuance Notice, as though made at and as of each such date (except that such representations and warranties shall be deemed to relate to the Registration Statement and the Prospectus as amended and supplemented relating to such Shares).

(o) Deliverables at Triggering Event Dates; Certificates. The Company agrees that on or prior to the date of the first Issuance Notice and, during the term of this Agreement after the date of the first Issuance Notice, upon:

(A) the filing of the Prospectus or the amendment or supplement of any Registration Statement or Prospectus (other than a prospectus supplement relating solely to an offering of securities other than the Shares or a prospectus filed pursuant to Section 4(a)(ii)(B)), by means of a post-effective amendment, sticker or supplement, but not by means of incorporation of documents by reference into the Registration Statement or Prospectus;

(B) the filing with the Commission of an annual report on Form 10-K or a quarterly report on Form 10-Q (including any Form 10-K/A or Form 10-Q/A containing amended financial information or a material amendment to the previously filed annual report on Form 10-K or quarterly report on Form 10-Q), in each case, of the Company; or

(C) the filing with the Commission of a current report on Form 8-K of the Company containing amended financial information (other than information "furnished" pursuant to Item 2.02 or 7.01 of Form 8-K or to provide disclosure pursuant to Item 8.01 of Form 8-K relating to reclassification of certain properties as discontinued operations in accordance with Statement of Financial Accounting Standards No. 144) that is material to the offering of securities of the Company in the Agent's reasonable discretion;

(any such event, a "**Triggering Event Date**"), the Company shall furnish the Agent (but in the case of clause (C) above only if the Agent reasonably determines that the information contained in such current report on Form 8-K of the Company is material) with a certificate as of the Triggering Event

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Date, in the form and substance satisfactory to the Agent and its counsel, substantially similar to the form previously provided to the Agent and its counsel, modified, as necessary, to relate to the Registration Statement and the Prospectus as amended or supplemented, (A) confirming that the representations and warranties of the Company contained in this Agreement are true and correct, (B) that the Company has performed all of its obligations hereunder to be performed on or prior to the date of such certificate and as to the matters set forth in Section 5(a)(iii) hereof, and (C) containing any other certification that the Agent shall reasonably request. The requirement to provide a certificate under this Section 4(o) shall be waived for any Triggering Event Date occurring at a time when no Issuance Notice is pending or a suspension is in effect, which waiver shall continue until the earlier to occur of the date the Company delivers instructions for the sale of Shares hereunder (which for such calendar quarter shall be considered a Triggering Event Date) and the next occurring Triggering Event Date.

Notwithstanding the foregoing, if the Company subsequently decides to sell Shares following a Triggering Event Date when a suspension was in effect and did not provide the Agent with a certificate under this Section 4(o), then before the Company delivers the instructions for the sale of Shares or the Agent sells any Shares pursuant to such instructions, the Company shall provide the Agent with a certificate in conformity with this Section 4(o) dated as of the date that the instructions for the sale of Shares are issued.

(p) Legal Opinions. On or prior to the date of the first Issuance Notice and on or prior to each Triggering Event Date with respect to which the Company is obligated to deliver a certificate pursuant to Section 4(o) for which no waiver is applicable and excluding the date of this Agreement, a negative assurances letter and the written legal opinion of Cooley LLP, counsel to the Company, and Seed Intellectual Property Group LLP, intellectual property counsel to the Company, each dated the date of delivery, in form and substance reasonably satisfactory to the Agent and its counsel, substantially similar to the form previously provided to the Agent and its counsel, modified, as necessary, to relate to the Registration Statement and the Prospectus as then amended or supplemented. In lieu of such opinions for subsequent periodic filings, in the discretion of the Agent, the Company may furnish a reliance letter from such counsel to the Agent, permitting the Agent to rely on a previously delivered opinion letter, modified as appropriate for any passage of time or Triggering Event Date (except that statements in such prior opinion shall be deemed to relate to the Registration Statement and the Prospectus as amended or supplemented as of such Triggering Event Date).

(q) Comfort Letter. On or prior to the date of the first Issuance Notice and on or prior to each Triggering Event Date with respect to which the Company is obligated to deliver a certificate pursuant to Section 4(o) for which no waiver is applicable and excluding the date of this Agreement, the Company shall cause each of (i) Ernst & Young LLP, the independent registered public accounting firm who has audited the financial statements included or incorporated by reference in the Registration Statement and (ii) BDO USA, LLP, the Company's former independent registered public accounting firm who audited the financial statements for the year-ended December 31, 2022, which at the time of this Agreement, is included or incorporated by reference in the Registration Statement, to furnish the Agent a comfort letter, dated the date of delivery, in form and substance reasonably satisfactory to the Agent and its counsel, substantially similar to the form previously provided to the Agent and its counsel; provided, however, that any such comfort letter will only be required on the Triggering Event Date specified to the extent that it contains financial statements filed with the Commission under the Exchange Act and incorporated or deemed to be incorporated by reference into a Prospectus; and provided further, that no such comfort letter will be required to be furnished by BDO USA, LLP to the Agent on or prior to the date of the first Issuance Notice or on or prior to each Triggering Event Date if the Company's most recent annual report on Form 10-K, as of such date, does not contain an audit report of BDO USA, LLP. If requested by the Agent, the Company shall also cause a comfort letter to be furnished to the Agent within ten (10) Trading Days of the date of occurrence of any material transaction or event requiring the filing of a current report on Form 8-K containing material amended financial information of the Company, including the restatement of the Company's financial statements. The Company shall be required to furnish no more than one comfort letter hereunder per calendar quarter.

(r) Secretary's Certificate. On or prior to the date of the first Issuance Notice, the Company shall furnish the Agent a certificate executed by the Secretary of the Company, signing in such capacity, dated the date of delivery (i) certifying that attached thereto are true and complete copies of the resolutions duly adopted by the Board of Directors of the Company authorizing the execution and delivery of this Agreement and the consummation of the transactions contemplated hereby (including, without limitation, the issuance of the Shares pursuant to this Agreement), which authorization shall be in full force and effect on and as of the date of such certificate, (ii) certifying and attesting to the office, incumbency, due authority and specimen signatures of each Person who executed this Agreement for or on behalf of the Company and (iii) containing any other certification that the Agent shall reasonably request.

(s) Agent's Own Account; Clients' Account. The Company consents to the Agent trading, in compliance with applicable law, in the Common Shares for the Agent's own account and for the account of its clients at the same time as sales of the Shares occur pursuant to this Agreement.

(t) Investment Limitation. The Company shall not invest, or otherwise use the proceeds received by the Company from its sale of the Shares in such a manner as would require the Company or any of its subsidiaries to register as an investment company under the Investment Company Act.

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(u) Market Activities. The Company will not take, directly or indirectly, any action designed to or that might be reasonably expected to cause or result in stabilization or manipulation of the price of the Shares or any other reference security, whether to facilitate the sale or resale of the Shares or otherwise, and the Company will, and shall cause each of its Affiliates to, comply with all applicable provisions of Regulation M. If the limitations of Rule 102 of Regulation M ("**Rule 102**") do not apply with respect to the Shares or any other reference security pursuant to any exception set forth in Section (d) of Rule 102, then promptly upon notice from the Agent (or, if later, at the time stated in the notice), the Company will, and shall cause each of its Affiliates to, comply with Rule 102 as though such exception were not available but the other provisions of Rule 102 (as interpreted by the Commission) did apply. The Company shall promptly notify the Agent if it no longer meets the requirements set forth in Section (d) of Rule 102.

(v) Notice of Other Sale. Without the written consent of the Agent, the Company will not, directly or indirectly, (i) offer to sell, sell, contract to sell, grant any option to sell or otherwise dispose of any Common Shares or securities convertible into or exchangeable for Common Shares (other than Shares hereunder), warrants or any rights to purchase or acquire Common Shares, during the period beginning on the third Trading Day immediately prior to the date on which any Issuance Notice is delivered to the Agent hereunder and ending on the third Trading Day immediately following the Settlement Date with respect to Shares sold pursuant to such Issuance Notice; (ii) effect a reverse stock split, recapitalization, share consolidation, reclassification or similar transaction affecting the outstanding Common Shares; or (iii) enter into any other "at the market" or continuous equity transaction offer to sell, sell, contract to sell, grant any option to sell or otherwise dispose of any Common Shares (other than the Shares

offered pursuant to this Agreement) or securities convertible into or exchangeable for Common Shares, warrants or any rights to purchase or acquire, Common Shares prior to the termination of this Agreement; provided, however, that such restrictions will not be required in connection with the Company's (i) issuance or sale of Common Shares, options to purchase Common Shares or Common Shares issuable upon the exercise of options or other equity awards pursuant to any employee or director share option, incentive or benefit plan, share purchase or ownership plan, long-term incentive plan, dividend reinvestment plan, inducement award under Principal Market rules or other compensation plan of the Company or its subsidiaries, as in effect on the date of this Agreement, (ii) issuance or sale of Common Shares issuable upon exchange, conversion or redemption of securities or the exercise or vesting of warrants, options or other equity awards outstanding at the date of this Agreement and (iii) modification of any outstanding options, warrants of any rights to purchase or acquire Common Shares.

Section 5. CONDITIONS TO DELIVERY OF ISSUANCE NOTICES AND TO SETTLEMENT

(a) Conditions Precedent to the Right of the Company to Deliver an Issuance Notice and the Obligation of the Agent to Sell Shares. The right of the Company to deliver an Issuance Notice hereunder is subject to the satisfaction, on the date of delivery of such Issuance Notice, and the obligation of the Agent to use its commercially reasonable efforts to place Shares during the applicable period set forth in the Issuance Notice is subject to the satisfaction, on each Trading Day during the applicable period set forth in the Issuance Notice, of each of the following conditions:

(i) Accuracy of the Company's Representations and Warranties; Performance by the Company. The Company shall have delivered the certificate required to be delivered pursuant to Section 4(o) on or before the date on which delivery of such certificate is required pursuant to Section 4(o). The Company shall have performed, satisfied and complied with all covenants, agreements and conditions required by this Agreement to be performed, satisfied or complied with by the Company at or prior to such date, including, but not limited to, the covenants contained in Section 4(p), Section 4(q) and Section 4(r).

(ii) No Injunction. No statute, rule, regulation, executive order, decree, ruling or injunction shall have been enacted, entered, promulgated or endorsed by any court or governmental authority of competent jurisdiction or any self-regulatory organization having authority over the matters contemplated hereby that prohibits or directly and materially adversely affects any of the transactions contemplated by this Agreement, and no proceeding shall have been commenced that may have the effect of prohibiting or materially adversely affecting any of the transactions contemplated by this Agreement.

(iii) Material Adverse Changes. Except as disclosed in the Prospectus and the Time of Sale Information, (a) in the judgment of the Agent there shall not have occurred any Material Adverse Change; and (b) there shall not have occurred any downgrading, nor shall any notice have been given of any intended or potential

downgrading or of any review for a possible change that does not indicate the direction of the possible change, in the rating accorded any securities of the Company or any of its subsidiaries by any "nationally recognized statistical rating organization" as such term is defined for purposes of Section 3(a)(62) of the Exchange Act.

(iv) No Suspension of Trading in or Delisting of Common Shares; Other Events. The trading of the Common Shares (including without limitation the Shares) shall not have been suspended by the Commission, the Principal Market or FINRA and the Common Shares (including without limitation the Shares) shall have been approved for listing or quotation on and shall not have been delisted from the Nasdaq Stock Market, the New York Stock Exchange or any of their constituent markets. There shall not have occurred (and be continuing in the case of occurrences under clauses (i) and (ii) below) any of the following: (i) trading or quotation in any of the

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Company's securities shall have been suspended or limited by the Commission or by the Principal Market or trading in securities generally on either the Principal Market shall have been suspended or limited, or minimum or maximum prices shall have been generally established on any of such stock exchanges by the Commission or the FINRA; (ii) a general banking moratorium shall have been declared by any of federal or New York, authorities; or (iii) there shall have occurred any outbreak or escalation of national or international hostilities or any crisis or calamity, or any change in the United States or international financial markets, or any substantial change or development involving a prospective substantial change in United States' or international political, financial or economic conditions, as in the judgment of the Agent is material and adverse and makes it impracticable to market the Shares in the manner and on the terms described in the Prospectus or to enforce contracts for the sale of securities.

(b) Documents Required to be Delivered on each Issuance Notice Date. The Agent's obligation to use its commercially reasonable efforts to place Shares hereunder shall additionally be conditioned upon the delivery to the Agent on or before the Issuance Notice Date of a certificate in form and substance reasonably satisfactory to the Agent, executed by the Chief Executive Officer, President or Chief Financial Officer of the Company, to the effect that all conditions to the delivery of such Issuance Notice shall have been satisfied as at the date of such certificate (which certificate shall not be required if the foregoing representations shall be set forth in the Issuance Notice).

(c) No Misstatement or Material Omission. Agent shall not have advised the Company that the Registration Statement, the Prospectus or the Times of Sales Information, or any amendment or supplement thereto, contains an untrue statement of fact that in the Agent's reasonable opinion is material, or omits to state a fact that in the Agent's reasonable opinion is material and is required to be stated therein or is necessary to make the statements therein not misleading.

Section 6. INDEMNIFICATION AND CONTRIBUTION

(a) Indemnification of the Agent. The Company agrees to indemnify and hold harmless the Agent, its officers and employees, and each person, if any, who controls the Agent within the meaning of the Securities Act or the Exchange Act against any loss, claim, damage, liability or expense, as incurred, to which the Agent or such officer, employee or controlling person may become subject, under the Securities Act, the Exchange Act, other federal or state statutory law or regulation, or the laws or regulations of foreign jurisdictions where Shares have been offered or sold or at common law or otherwise (including in settlement of any litigation), insofar as such loss, claim, damage, liability or expense (or actions in respect thereof as contemplated below) arises out of or is based upon: (i) any untrue statement or alleged untrue statement of a material fact contained in the Registration Statement, or any amendment thereto, including any information deemed to be a part thereof pursuant to Rule 430B under the Securities Act, or the omission or alleged omission therefrom of a material fact required to be stated therein or necessary to make the statements therein not misleading; or (ii) any untrue statement or alleged untrue statement of a material fact contained in any Free Writing Prospectus that the Company has used, referred to or filed, or is required to file, pursuant to Rule 433(d) of the Securities Act or the Prospectus (or any amendment or supplement thereto), or the omission or alleged omission therefrom of a material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading, and to reimburse the Agent and each such officer, employee and controlling person for any and all expenses (including the reasonable and documented fees and disbursements of counsel chosen by the Agent) as such expenses are reasonably incurred by the Agent or such officer, employee or controlling person in connection with investigating, defending, settling, compromising or paying any such loss, claim, damage, liability, expense or action; provided, however, that the foregoing indemnity agreement shall not apply to any loss, claim, damage, liability or expense to the extent, but only to the extent, arising out of or based upon any untrue statement or alleged untrue statement or omission or alleged omission made in reliance upon and in conformity with written information furnished to the Company by the Agent expressly for use in the Registration Statement, any such Free Writing Prospectus or the Prospectus (or any amendment or supplement thereto), it being understood and agreed that the only such information furnished by the Agent to the Company consists of the information set forth in the information described in Section 6(b) below. The indemnity agreement set forth in this Section 6(a) shall be in addition to any liabilities that the Company may otherwise have.

(b) Indemnification of the Company, its Directors and Officers. The Agent agrees to indemnify and hold harmless the Company, each of its directors, each of its officers who signed the Registration Statement and each person, if any, who controls the Company within the meaning of the Securities Act or the Exchange Act against any loss, claim, damage, liability or expense, as incurred, to which the Company or any such director, officer or controlling person may become subject, under the Securities Act, the Exchange Act, or other federal or state statutory law or regulation, or the laws or regulations of foreign jurisdictions where Shares have been offered or sold or at common law or otherwise (including in settlement of any litigation), arises out of or is based upon (i) any untrue statement or alleged untrue statement of a material fact contained in the Registration Statement, or any amendment thereto, including any

information deemed to be a part thereof pursuant to Rule 430B under the Securities Act, or the omission or alleged omission therefrom of a material fact required to be stated therein or necessary to make the statements therein not misleading; or (ii) any untrue statement or alleged untrue statement of a material fact contained in any Free Writing Prospectus that the Company has used, referred to or filed, or is required to file, pursuant to Rule 433(d) of the Securities Act or the Prospectus (or any amendment or supplement thereto), or the omission or alleged omission therefrom of a material

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fact necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading; but only to the extent arising out of or based upon any untrue statement or alleged untrue statement or omission or alleged omission made in reliance upon and in conformity with written information furnished to the Company by the Agent expressly for use in the Registration Statement, any such Free Writing Prospectus or the Prospectus (or any amendment or supplement thereto), it being understood and agreed that the only such information furnished by the Agent to the Company consists of the information set forth in the first sentence of the ninth paragraph under the caption “Plan of Distribution” in the Prospectus, and to reimburse the Company and each such director, officer and controlling person for any and all expenses (including the fees and disbursements of counsel chosen by the Company) as such expenses are reasonably incurred by the Company or such officer, director or controlling person in connection with investigating, defending, settling, compromising or paying any such loss, claim, damage, liability, expense or action. The indemnity agreement set forth in this Section 6(b) shall be in addition to any liabilities that the Agent or the Company may otherwise have.

(c) Notifications and Other Indemnification Procedures. Promptly after receipt by an indemnified party under this Section 6 of notice of the commencement of any action, such indemnified party will, if a claim in respect thereof is to be made against an indemnifying party under this Section 6, notify the indemnifying party in writing of the commencement thereof, but the omission to so notify the indemnifying party will not relieve it from any liability which it may have to any indemnified party for contribution or otherwise than under the indemnity agreement contained in this Section 6 or to the extent it is not prejudiced as a proximate result of such failure. In case any such action is brought against any indemnified party and such indemnified party seeks or intends to seek indemnity from an indemnifying party, the indemnifying party will be entitled to participate in, and, to the extent that it shall elect, jointly with all other indemnifying parties similarly notified, by written notice delivered to the indemnified party promptly after receiving the aforesaid notice from such indemnified party, to assume the defense thereof with counsel reasonably satisfactory to such indemnified party; provided, however, if the defendants in any such action include both the indemnified party and the indemnifying party and the indemnified party shall have reasonably concluded based on the advice of counsel that a conflict may arise between the positions of the indemnifying party and the indemnified party in conducting the defense of any such action or that there may be legal defenses available to it and/or other indemnified parties which are different from or additional to those available to the indemnifying party, the indemnified party or parties shall have the right to select separate counsel to assume such legal defenses and to otherwise participate in the defense of such action on behalf of such indemnified party or parties. Upon receipt of notice from the indemnifying party to such indemnified party of such indemnifying party’s election so to assume the defense of such action and approval by the indemnified party of counsel, the indemnifying

party will not be liable to such indemnified party under this Section 6 for any legal or other expenses subsequently incurred by such indemnified party in connection with the defense thereof unless (i) the indemnified party shall have employed separate counsel in accordance with the proviso to the preceding sentence (it being understood, however, that the indemnifying party shall not be liable for the fees and expenses of more than one separate counsel (together with local counsel), representing the indemnified parties who are parties to such action), which counsel (together with any local counsel) for the indemnified parties shall be selected by the Agent (in the case of counsel for the indemnified parties referred to in Section 6(a)above), (ii) the indemnifying party shall not have employed counsel satisfactory to the indemnified party to represent the indemnified party within a reasonable time after notice of commencement of the action or (iii) the indemnifying party has authorized in writing the employment of counsel for the indemnified party at the expense of the indemnifying party, in each of which cases the fees and expenses of counsel shall be at the expense of the indemnifying party and shall be paid as they are incurred.

(d) Settlements. The indemnifying party under this Section 6 shall not be liable for any settlement of any proceeding effected without its written consent, but if settled with such consent or if there be a final judgment for the plaintiff, the indemnifying party agrees to indemnify the indemnified party against any loss, claim, damage, liability or expense by reason of such settlement or judgment. Notwithstanding the foregoing sentence, if at any time an indemnified party shall have requested an indemnifying party to reimburse the indemnified party for fees and expenses of counsel as contemplated by Section 6(c) hereof, the indemnifying party agrees that it shall be liable for any settlement of any proceeding effected without its written consent if (i) such settlement is entered into more than 45 days after receipt by such indemnifying party of the aforesaid request; and (ii) such indemnifying party shall not have reimbursed the indemnified party in accordance with such request prior to the date of such settlement. No indemnifying party shall, without the prior written consent of the indemnified party, effect any settlement, compromise or consent to the entry of judgment in any pending or threatened action, suit or proceeding in respect of which any indemnified party is or could have been a party and indemnity was or could have been sought hereunder by such indemnified party, unless such settlement, compromise or consent includes an unconditional release of such indemnified party from all liability on claims that are the subject matter of such action, suit or proceeding.

(e) Contribution. If the indemnification provided for in this Section 6 is for any reason held to be unavailable to or otherwise insufficient to hold harmless an indemnified party in respect of any losses, claims, damages, liabilities or expenses referred to therein, then each indemnifying party shall contribute to the aggregate amount paid or payable by such indemnified party, as incurred, as a result of any losses, claims, damages, liabilities or expenses referred to therein (i) in such proportion as is appropriate to reflect the relative benefits received by the Company, on the one hand, and the Agent, on the other hand, from the offering of the Shares pursuant to this Agreement; or (ii) if the allocation provided by clause (i) above is not permitted by applicable law, in such proportion as is appropriate to reflect not only the relative benefits referred to in clause (i) above but also the relative fault of the Company, on the one hand, and the Agent, on the other hand, in connection with the statements or omissions which resulted in such

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losses, claims, damages, liabilities or expenses, as well as any other relevant equitable considerations. The relative benefits received by the Company, on the one hand, and the Agent, on the other hand, in connection with the offering of the Shares pursuant to this Agreement shall be deemed to be in the same respective proportions as the total gross proceeds from the offering of the Shares (before deducting expenses) received by the Company bear to the total commissions received by the Agent. The relative fault of the Company, on the one hand, and the Agent, on the other hand, shall be determined by reference to, among other things, whether any such untrue or alleged untrue statement of a material fact or omission or alleged omission to state a material fact relates to information supplied by the Company, on the one hand, or the Agent, on the other hand, and the parties' relative intent, knowledge, access to information and opportunity to correct or prevent such statement or omission.

The amount paid or payable by a party as a result of the losses, claims, damages, liabilities and expenses referred to above shall be deemed to include, subject to the limitations set forth in Section 6(c), any legal or other fees or expenses reasonably incurred by such party in connection with investigating or defending any action or claim. The provisions set forth in Section 6(c) with respect to notice of commencement of any action shall apply if a claim for contribution is to be made under this Section 6(e); *provided, however,* that no additional notice shall be required with respect to any action for which notice has been given under Section 6(c) for purposes of indemnification.

The Company and the Agent agree that it would not be just and equitable if contribution pursuant to this Section 6(e) were determined by pro rata allocation or by any other method of allocation which does not take account of the equitable considerations referred to in this Section 6(e).

Notwithstanding the provisions of this Section 6(e), the Agent shall not be required to contribute any amount in excess of the agent fees received by the Agent in connection with the offering contemplated hereby. No person guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the Securities Act) shall be entitled to contribution from any person who was not guilty of such fraudulent misrepresentation. For purposes of this Section 6(e), each officer and employee of the Agent and each person, if any, who controls the Agent within the meaning of the Securities Act or the Exchange Act shall have the same rights to contribution as the Agent, and each director of the Company, each officer of the Company who signed the Registration Statement, and each person, if any, who controls the Company with the meaning of the Securities Act and the Exchange Act shall have the same rights to contribution as the Company.

Section 7. TERMINATION & SURVIVAL

(a) Term. Subject to the provisions of this Section 7, the term of this Agreement shall continue from the date of this Agreement until the end of the Agency Period, unless earlier terminated by the parties to this Agreement pursuant to this Section 7.

(b) Termination; Survival Following Termination.

(i) Either party may terminate this Agreement prior to the end of the Agency Period, by giving written notice as required by this Agreement, upon notice to the other party; provided that, (A) if the Company terminates this Agreement after the Agent confirms to the Company any sale of Shares, the Company shall remain obligated to comply with Section 3(b)(v) with respect to such Shares and (B) Section 2, Section 6, Section 7 and Section 8 shall survive termination of this Agreement. If termination shall occur prior to the Settlement Date for any sale of Shares, such sale shall nevertheless settle in accordance with the terms of this Agreement.

(ii) In addition to the survival provision of Section 7(b)(i), the respective indemnities, agreements, representations, warranties and other statements of the Company, of its officers and of the Agent set forth in or made pursuant to this Agreement will remain in full force and effect, regardless of any investigation made by or on behalf of the Agent or the Company or any of its or their partners, officers or directors or any controlling person, as the case may be, and, anything herein to the contrary notwithstanding, will survive delivery of and payment for the Shares sold hereunder and any termination of this Agreement.

Section 8. MISCELLANEOUS

(a) Press Releases and Disclosure. The Company may issue a press release describing the material terms of the transactions contemplated hereby as soon as practicable following the date of this Agreement, and may file with the Commission a Current Report on Form 8-K, with this Agreement attached as an exhibit thereto, describing the material terms of the transactions contemplated hereby, and the Company shall consult with the Agent prior to making such disclosures, and the parties hereto shall use all commercially reasonable efforts, acting in good faith, to agree upon a text for such disclosures that is reasonably satisfactory to all parties hereto. No party hereto shall issue thereafter any press release or like public statement (including, without limitation, any disclosure required in reports filed with the Commission pursuant to the Exchange Act) related to this Agreement or any of the transactions contemplated hereby without the prior written approval of the other party hereto, except as may be necessary or appropriate in the reasonable opinion of the party seeking to make disclosure to comply with the requirements of applicable law or stock exchange rules. If any such press release or like public statement is so required, the party making such disclosure shall consult with the other party prior to making such disclosure, and the parties shall use all commercially reasonable efforts, acting in good faith, to agree upon a text for such disclosure that is reasonably satisfactory to all parties hereto.

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(b) No Advisory or Fiduciary Relationship.The Company acknowledges and agrees that (i) the transactions contemplated by this Agreement, including the determination of any fees, are arm's-length commercial transactions between the Company and the Agent, (ii) when acting as a principal under this Agreement, the Agent is and has been acting solely as a principal and is not the agent or fiduciary of the Company, or its stockholders, creditors, employees or any other party, (iii) the Agent has not assumed nor will assume an advisory or fiduciary responsibility in

favor of the Company with respect to the transactions contemplated hereby or the process leading thereto (irrespective of whether the Agent has advised or is currently advising the Company on other matters) and the Agent does not have any obligation to the Company with respect to the transactions contemplated hereby except the obligations expressly set forth in this Agreement, (iv) the Agent and its respective affiliates may be engaged in a broad range of transactions that involve interests that differ from those of the Company, and (v) the Agent has not provided any legal, accounting, regulatory or tax advice with respect to the transactions contemplated hereby and the Company has consulted its own legal, accounting, regulatory and tax advisors to the extent it deemed appropriate.

(c) Research Analyst Independence. The Company acknowledges that the Agent's research analysts and research departments are required to and should be independent from their respective investment banking divisions and are subject to certain regulations and internal policies, and as such the Agent's research analysts may hold views and make statements or investment recommendations and/or publish research reports with respect to the Company or the offering that differ from the views of their respective investment banking divisions. The Company understands that the Agent is a full service securities firm and as such from time to time, subject to applicable securities laws, may effect transactions for its own account or the account of its customers and hold long or short positions in debt or equity securities of the companies that may be the subject of the transactions contemplated by this Agreement.

(d) Notices. All communications hereunder shall be in writing and shall be mailed, hand delivered or telecopied and confirmed to the parties hereto as follows:

If to the Agent:

Jefferies LLC
520 Madison Avenue
New York, NY 10022
Attention: General Counsel

with a copy (which shall not constitute notice) to:

Latham & Watkins LLP
12670 High Bluff Drive
San Diego, CA 92130
Facsimile: (858) 523-5450
Attention: Cheston J. Larson, Esq.

If to the Company:
Travere Therapeutics, Inc.
3611 Valley Centre Drive, Suite 300
San Diego, CA 92130
Attention: Chief Executive Officer.

with a copy (which shall not constitute notice) to:

Cooley LLP
10265 Science Center Drive
San Diego, CA 92121
Facsimile: (858) 550-6420
Attention: Jason L. Kent, Esq. and Asa M. Henin

Any party hereto may change the address for receipt of communications by giving written notice to the others in accordance with this Section 8(d).

(e) Successors. This Agreement will inure to the benefit of and be binding upon the parties hereto, and to the benefit of the employees, officers and directors and controlling persons referred to in Section 6, and in each case their respective successors, and no other person will have any

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right or obligation hereunder. The term “successors” shall not include any purchaser of the Shares as such from the Agent merely by reason of such purchase.

(f) Partial Unenforceability. The invalidity or unenforceability of any Article, Section, paragraph or provision of this Agreement shall not affect the validity or enforceability of any other Article, Section, paragraph or provision hereof. If any Article, Section, paragraph or provision of this Agreement is for any reason determined to be invalid or unenforceable, there shall be deemed to be made such minor changes (and only such minor changes) as are necessary to make it valid and enforceable.

(g) Governing Law Provisions. This Agreement shall be governed by and construed in accordance with the internal laws of the State of New York applicable to agreements made and to be performed in such state. Any legal suit, action or proceeding arising out of or based upon this Agreement or the transactions contemplated hereby may be instituted in the federal courts of the United States of America located in the Borough of Manhattan in the City of New York or the courts of the State of New York in each case located in the Borough of Manhattan in the City of New York (collectively, the “**Specified Courts**”), and each party irrevocably submits to the exclusive jurisdiction (except for proceedings instituted in regard to the enforcement of a judgment of any such court, as to which such jurisdiction is non-exclusive) of such courts in any such suit, action or proceeding. Service of any process, summons, notice or document by mail to such party’s address set forth above shall be effective service of process for any suit, action or other proceeding brought in any such court. The parties irrevocably and unconditionally waive any objection to the laying of venue of any suit, action or other proceeding in the Specified Courts and irrevocably and unconditionally waive and agree not to plead or claim in any such court that any such suit, action or other proceeding brought in any such court has been brought in an inconvenient forum.

(h) General Provisions. This Agreement constitutes the entire agreement of the parties to this Agreement and supersedes all prior written or oral and all contemporaneous oral agreements, understandings and negotiations with respect to the subject matter hereof. This

Agreement may be executed in two or more counterparts, each one of which shall be an original, with the same effect as if the signatures thereto and hereto were upon the same instrument, and may be delivered by facsimile transmission or by electronic delivery of a portable document format (PDF) file. This Agreement may not be amended or modified unless in writing by all of the parties hereto, and no condition herein (express or implied) may be waived unless waived in writing by each party whom the condition is meant to benefit. The Article and Section headings herein are for the convenience of the parties only and shall not affect the construction or interpretation of this Agreement.

[Signature Page Immediately Follows]

If the foregoing is in accordance with your understanding of our agreement, kindly sign and return to the Company the enclosed copies hereof, whereupon this instrument, along with all counterparts hereof, shall become a binding agreement in accordance with its terms

Very truly yours,

TRAVERE THERAPEUTICS, INC.

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

The foregoing Agreement is hereby confirmed and accepted by the Agent in New York, New York as of the date first above written.

JEFFERIES LLC

By: /s/ Donald Lynaugh
Name: Donald Lynaugh
Title: Managing Director

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EXHIBIT A

ISSUANCE NOTICE

[Date]

Jefferies LLC
520 Madison Avenue
New York, New York 10022

Attn: [_____]

Reference is made to the Amended and Restated Open Market Sale Agreement between Travere Therapeutics, Inc. (the "**Company**") and Jefferies LLC (the "**Agent**") dated as of October [31], 2024. The Company confirms that all conditions to the delivery of this Issuance Notice are satisfied as of the date hereof.

Date of Delivery of Issuance Notice (determined pursuant to Section 3(b)(i)): _____

Issuance Amount (equal to the total Sales Price for such Shares):

$

Number of days in selling period:

First date of selling period:

Last date of selling period:

Settlement Date(s) if other than standard T+1 settlement:

Floor Price Limitation (in no event less than $1.00 without the prior written consent of the Agent, which consent may be withheld in the Agent's sole discretion): $ ____ per share

Comments:

_____

By:
Name:
Title:

Schedule A

Notice Parties

The Agent

Michael Brinkman (mbrinkman@jefferies.com)

Michael Magarro (mmagarro@jefferies.com)

Donald Lynaugh (dlynaugh@jefferies.com)

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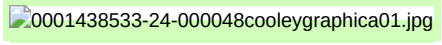
Exhibit 5.1



Jason L. Kent
+1 212 479-6044
jkent@cooley.com

October 31, 2024

Travere Therapeutics, Inc.
3611 Valley Centre Drive, Suite 300
San Diego, CA 92130

Ladies and Gentlemen:

We have acted as counsel to Travere Therapeutics, Inc., a Delaware corporation (the "***Company***"), in connection with the offering by the Company of shares of its common stock, par value $0.0001 per share (the "***Common Stock***"), have an aggregate offering price of up to $100 million (the "***Shares***") pursuant to a Registration Statement on Form S-3 (File No. 333-281194) (the "***Registration Statement***"), filed with the Securities and Exchange Commission (the "***Commission***") under the Securities Act of 1933, as amended (the "***Securities Act***"), the prospectus included in the Registration Statement (the "***Base Prospectus***"), and the prospectus supplement relating to the Shares to be filed with the Commission pursuant to Rule 424(b) under the Securities Act (together with the Base Prospectus, the "***Prospectus***"). The Shares are to be sold by the Company in accordance with the Amended and Restated Open Market Sale Agreement, dated October 31, 2024, by and between the Company and Jefferies LLC (the "***Agreement***").

In connection with this opinion, we have examined and relied upon (a) the Registration Statement and the Prospectus, (b) the Agreement, (c) the Company's certificate of incorporation and bylaws, each as currently in effect, and (d) such other records, documents, opinions, certificates, memoranda and instruments as in our judgment are necessary or appropriate to enable us to render the opinion expressed below. We have assumed the genuineness of all signatures; the authenticity of all documents submitted to us as originals; the conformity to originals of all documents submitted to us as copies; the accuracy, completeness and authenticity of certificates of public officials; and the due authorization, execution and delivery, of all documents by all persons other than the Company where authorization, execution and delivery are prerequisites to the effectiveness thereof. As to certain factual matters, we have relied upon a certificate of an officer of the Company and have not independently verified such matters.

In rendering this opinion, we have also assumed (i) that each sale of Shares will be duly authorized by the Board of Directors of the Company, a duly authorized committee thereof or a person or body pursuant to an authorization granted in accordance with Section 152 of the General Corporation Law of the State of Delaware (the "***DGCL***"), and (ii) that no more than 7,500,000 Shares will be sold under the Agreement pursuant to the Prospectus and (iii) that the price at which the Shares are sold for a consideration per share not less than the par value of the Shares. We express no opinion to the extent that future issuances of securities of the Company, anti-dilution adjustments to outstanding securities of the Company or other matters cause the number of shares of Common Stock issuable under the Agreement to exceed the number of shares of Common Stock available for issuance by the Company.

Our opinion is expressed solely with respect to the DGCL. We express no opinion to the extent that any other laws are applicable to the subject matter hereof and express no opinion and provide no assurance as to compliance with any federal or state securities law, rule or regulation.

On the basis of the foregoing, in reliance thereon and subject to the qualifications set forth herein, we are of the opinion that the Shares, when sold and issued against payment therefor in accordance with the Agreement, the Registration Statement and the Prospectus, will be validly issued, fully paid and nonassessable.

This opinion is limited to the matters expressly set forth in this letter, and no opinion has been or should be implied, or may be inferred, beyond the matters expressly stated. This opinion speaks only as to law and facts in effect or existing as of the date hereof and we have no obligation or responsibility to update or supplement this letter to reflect any facts or circumstances that may hereafter come to our attention or any changes in law that may hereafter occur.

We consent to the reference to our firm under the heading "Legal Matters" in the Prospectus and to the filing of this opinion as an exhibit to the Company's Quarterly Report on Form 10-Q to be filed with the Commission for incorporation by reference into the Registration Statement. In giving such consents, we do not thereby admit that we are in the category of persons whose consent is required under Section 7 of the Securities Act, or the rules and regulations of the Commission thereunder.

Sincerely,

Cooley LLP

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By: /s/ Jason L. Kent

Jason L. Kent

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EXHIBIT 10.1

**CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY [***],
HAS BEEN OMITTED BECAUSE IT IS BOTH (I) NOT MATERIAL AND
(II) IS THE TYPE OF INFORMATION THAT THE REGISTRANT TREATS AS PRIVATE OR CONFIDENTIAL.**

AMENDMENT NO. 5 TO SUBLICENSE AGREEMENT

THIS AMENDMENT NO. 5 TO SUBLICENSE AGREEMENT (the "**Amendment**") is made and entered into as of March 20, 2018 ("**Amendment Effective Date**") and amends the Sublicense Agreement effective as of February 16, 2012, as amended pursuant to that certain Amendment to Sublicense Agreement dated December 11, 2012, Amendment No. 2 to Sublicense Agreement dated January 7, 2013, Amendment No. 3 to Sublicense Agreement dated February 27, 2015 and Amendment No. 4 to Sublicense Agreement dated September 17, 2015 (the "**Sublicense Agreement**") by and between Ligand Pharmaceuticals Incorporated, a corporation organized under the laws of Delaware and having a place of business at **3911 Sorrento Valley Boulevard, Suite 110, San Diego, CA 92121** and its wholly owned subsidiary, Pharmacopeia, LLC (as successor in interest to Pharmacopeia Drug Discovery Inc.) ("**PCOP**"), a limited liability company organized under the laws of Delaware and having a place of business at **3911 Sorrento Valley Boulevard, Suite 110, San Diego, CA 92121** (collectively, Ligand Pharmaceuticals Incorporated and PCOP shall be known as "**Ligand**") and Retrophin Inc., a corporation organized under the laws of Delaware and having a place of business at **3721 Valley Centre Drive, Suite 200, San Diego, CA 92130** ("**Retrophin**").

BACKGROUND

WHEREAS Ligand and Retrophin have previously entered into the Sublicense Agreement pursuant to which Ligand sublicensed to Retrophin rights under the License Agreement dated March 27, 2006 between PCOP and Bristol-Myers Squib Company (the "Upstream License"); and

WHEREAS, Ligand and Retrophin desire to amend certain terms of the Sublicense Agreement as set forth herein.

NOW, THEREFORE, in consideration of the foregoing premises and the mutual covenants contained herein, the parties, intending to be legally bound, agree as follows:

1. **Capitalized Terms**. The capitalized terms used herein and not otherwise defined shall have the same definitions as provided in the Sublicense Agreement.
2. **Amendments**.
   a) Section 6.1.3 of the Sublicense Agreement is hereby amended to read as follows:

> "6.1.3 File for Approval for at least one (1) Orphan Licensed Product ("**Approval Submission**") no later than [...***...] ("**Filing Deadline**");
>
> [...***...].

   b) Section 8.2.1 of the Sublicense Agreement is hereby amended to read as follows:

> "8.2.1 Development Milestone Payments. Retrophin shall make milestone payments to Ligand upon achievement of each of the milestone events in the amounts set forth below in Table 1. The first milestone payment shall be payable by Retrophin to Ligand within thirty (30) days of execution of the Agreement. Notwithstanding Section 15.4 or any other provision herein, the last milestone payment shall be payable by Retrophin to Ligand upon the Closing of Retrophin's Exit Transaction. Subject to Section 8.2.2, the remainder of the milestone payments set forth below, with the exception of the milestone payment for Initiation of the first Phase 3 Trial for the first Licensed Product, will be payable by Retrophin to Ligand within thirty (30) days of the achievement of the specified milestone event with respect to each Licensed Compound. The milestone for Initiation of the first Phase 3 Trial for the first Licensed Product will be payable by Retrophin to Ligand within ten (10) days of the execution of Amendment No. 5 by both Parties. The milestone payments shall not be refundable or returnable in any event, nor shall they be creditable against royalties or other payments.

*** Certain Confidential Information Omitted

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Table 1

Milestone Event	Milestone Payment
Execution of Agreement	$1.15 million
The earlier of (a) December 31, 2012 or (b) initiation of the first Phase 2 Trial for a Licensed Product	$1.3 million (the "**Second Milestone**"); provided, that if the Second Milestone is received by Ligand (a) prior to or on January 31, 2012, Retrophin shall make an additional $50,000 payment simultaneously with the payment of the Second Milestone (for an aggregate payment of $1.35 million), (b) after January 31, 2013 but prior to or on February 28, 2013, Retrophin shall make an additional $100,000 payment simultaneously with the payment of the Second Milestone (for an aggregate payment of $1.4 million), and (c) after February 28, 2013 but prior to or on March 31, 2013, Retrophin shall make an additional $150,000 payment of the Second Milestone (for an aggregate payment of $1.45 million) (the additional payment, an "**Additional Payment**") [1]

[1] If the Second Milestone and any Additional Payment is not received by Ligand on or before March 31, 2013, Ligand shall have the right to terminate the Agreement pursuant to Section 13.2.2 with immediate effect as of March 31, 2013 by providing written notice to Retrophin, notwithstanding (a) the cure period for the failure to make a payment when due set out in said Section 13.2.2 (Breach) or (b) the provisions to Section 13.2.4 (Disputed Breach). In addition, and for clarity, the provisions of Section 13.4 (Effect of Termination) shall be operative, including, without limitation, the provisions of subsections (c), (k), and (m) related to amounts then due and payable."

Milestone Event	Milestone Payment
At or prior to Initiation of the first Phase 3 Trial for the first Licensed Product	$4.6 million
[...***...]	[...***...]
[...***...]	[...***...]
[...***...]	[...***...]
[...***...]	[...***...]
[...***...]	[...***...]
[...***...]	[...***...]
[...***...]	[...***...]
[...***...]	[...***...]
[...***...]	[...***...]
[...***...]	[...***...]

In the event that a milestone event is achieved that triggers a development milestone payment as set forth above, if the preceding milestone events have not occurred such that the previous development milestone payments have not been previously paid, all such previous development milestone payments shall become due and payable upon achievement of such milestone event. For example, if a Phase 3 Trial is initiated that triggers a development milestone payment as set forth above without a Phase 2 Trial supporting such Phase 3 Trial being previously initiated (and consequently the applicable initiation of Phase 2 Trial milestone payment has not been previously paid to Ligand), in addition to the milestone payment for the initiation of the Phase 3 Trial, Retrophin shall also pay to Ligand the applicable milestone payment for the initiation of a Phase 2 Trial."

3. No Other Amendments. Except as provided herein, the Sublicense Agreement shall continue in full force and effect.

4. Governing Law. This Amendment shall be governed by, enforced, and shall be construed in accordance with the laws of the State of New York without regard to its conflicts of law provisions.

*** Certain Confidential Information Omitted

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5. Counterparts. This Amendment may be executed in counter-parts with the same effect as if both Parties had signed the same document. All such counterparts shall be deemed an original, shall be construed together and shall constitute one and the same instrument.

IN WITNESS WHEREOF, the Parties have executed this Amendment to Sublicense Agreement through their duly authorized representatives to be effective as of the Amendment Effective Date.

LIGAND PHARMACEUTICALS RETROPHIN, INC. INCORPORATED

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By: /s/ Charles S. Berkman By: /s/ Stephen Aselage Name: Charles S. Berkman Name: Stephen Aselage Title: Sr. VP, General Counsel & Secretary Title: CEO

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: August 1, 2024 October 31, 2024

/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;

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

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

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

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

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

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

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

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

Date: August 1, 2024 October 31, 2024

/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 June 30, 2024 September 30, 2024 (the "Report"), the undersigned officer of the Company hereby certifies pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to such officer's knowledge:

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

2. The information contained in the Report, fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: August 1, 2024 October 31, 2024

/s/ Eric Dube

Eric Dube

Chief Executive Officer

(Principal Executive Officer)

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Exhibit 32.2

CERTIFICATION OF

CHIEF FINANCIAL OFFICER

PURSUANT TO 18 U.S.C. SECTION 1350

AS ADOPTED PURSUANT TO

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the accompanying Quarterly Report on Form 10-Q of Travere Therapeutics, Inc. (the "Company"), for the period ending June 30, 2024 September 30, 2024 (the "Report"), the undersigned officer of the Company hereby certifies pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to such officer's knowledge:

1. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
2. The information contained in the Report, fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: August 1, 2024 October 31, 2024

/s/ Christopher Cline

Christopher Cline

Chief Financial Officer

(Principal Financial Officer)

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