UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

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

For the Fiscal Year Ended December 31, 2024

☐ ***Transition Report Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934***

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)

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**

Securities registered pursuant to Section 12(g) of the Act: **None**

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. ☑ Yes ☐ No

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. ☐ Yes ☑ No

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 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 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 7(a)(2)(B) of the Securities Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☑

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to §240.10D-1(b). ☐

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

State the aggregate market value of the voting and non-voting common equity held by non-affiliates computed by reference to the price at which the common equity was last sold, or the average bid and asked price of such common equity, as of the last business day of the registrant's most recently completed second fiscal quarter. $624,140,482.

The number of shares of outstanding common stock, par value $0.0001 per share, of the registrant as of February 18, 2025 was 88,739,826.

DOCUMENTS INCORPORATED BY REFERENCE: Portions of the Proxy Statement for the registrant's 2025 Annual Meeting of Stockholders, to be filed within 120 days after the conclusion of the registrant's fiscal year ended December 31, 2024, are incorporated by reference into Part III of this Annual Report on Form 10-K.

FORM 10-K REPORT INDEX

CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS

Certain information contained in this Annual Report on Form 10-K of Travere Therapeutics, Inc., a Delaware corporation (the “Company”) include forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. The statements herein which are not historical reflect our current expectations and projections about the Company’s future results, performance, liquidity, financial condition, prospects and opportunities and are based upon information currently available to the Company's management and is subject to its interpretation of what are believed to be significant factors affecting the Company's business, including many assumptions regarding future events. Such forward-looking statements include statements regarding, among other things:

- the estimated addressable U.S. patient population for FILSPARI® (sparsentan) in Immunoglobulin A nephropathy (IgAN);
- estimated patient populations related to our other products and products in development;
- expectations regarding our pivotal Phase 3 HARMONY Study to support the potential approval of pegtibatinase for the treatment of classical homocystinuria (HCU), including our ability to restart enrollment in 2026;
- expectations regarding the potential for sparsentan in focal segmental glomerulosclerosis (FSGS) and related matters, including our planned submission of a supplemental new drug application (sNDA) for FSGS around the end of the first quarter of 2025;
- our ability to produce, sustain and expand sales of our products;
- our ability to develop, acquire and/or introduce new products including expectations with regard to clinical trials and preclinical studies;
- expectations regarding potential future milestone and royalty payments;
- our projected future sales, profitability, savings and other financial metrics;
- our future financing plans;
- our anticipated needs for working capital;
- the anticipated trends in our industry;
- acquisitions of other companies or assets that we might undertake in the future;
- our operations in the United States and abroad, and the domestic and foreign regulatory, economic and political conditions; and
- competition existing today or that will likely arise in the future.

Forward-looking statements, which involve assumptions and describe our future plans, strategies and expectations, are generally identifiable by use of the words “may,” “should,” “expect,” “anticipate,” “estimate,” “believe,” “intend,” “seek,” or “project” or the negative of these words or other variations on these words or comparable terminology. Actual results, performance, liquidity, financial condition and results of operations, prospects and opportunities could differ materially from those expressed in, or implied by, these forward-looking statements as a result of various risks, uncertainties and other factors, including the ability to raise sufficient capital to continue the Company’s operations. Actual events or results may differ materially from those discussed in forward-looking statements as a result of various factors, including, without limitation, the risks outlined under “Risk Factors” and matters described in this Annual Report generally. 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 Annual Report on Form 10-K, and while we believe such information provides a reasonable basis for these 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 investors are cautioned not to unduly rely upon these statements.

In light of these risks and uncertainties, there can be no assurance that the forward-looking statements contained in this Annual Report will in fact occur. Potential investors should not place undue reliance on any forward-looking statements. Except as expressly required by the federal securities laws, there is no undertaking to publicly update or revise any forward-looking statements, whether as a result of new information, future events, changed circumstances or any other reason.

The specific discussions in this Annual Report about the Company include financial projections and future estimates and expectations about the Company’s business. The projections, estimates and expectations are presented in this Annual Report only as a guide about future possibilities and do not represent actual amounts or assured events. All the projections and estimates are based exclusively on the Company management’s own

assessment of the business, the industry in which it works and the economy at large and other operational factors, including capital resources and liquidity, financial condition, fulfillment of contracts and opportunities. The actual results may differ significantly from the projections.

Potential investors should not make an investment decision based solely on the Company’s projections, estimates or expectations.

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 I of this Annual Report on Form 10-K and should be carefully considered, together with other information in this Annual Report on Form 10-K and our other filings with the SEC before making investment decisions regarding our common stock.

- Our future prospects are highly dependent upon our ability to successfully develop and execute commercialization strategies for our products, including FILSPARI, and to attain market acceptance among physicians, patients and healthcare payers.
- Our clinical trials are expensive and time-consuming and may fail to demonstrate the safety and efficacy of our product candidates.
- Success in nonclinical testing and early clinical trials does not ensure that later clinical trials will be successful.
- Communications and/or feedback from regulatory authorities related to our current or planned future clinical trials does not guarantee any particular outcome from or timeline for regulatory review, and expedited regulatory review pathways may not actually lead to faster development or approval.
- In order to operate our business and increase adoption and sales of our products, we need to continue to develop our commercial organization, including maintaining a highly experienced and skilled workforce with qualified sales representatives.
- Interim, topline and preliminary data from our clinical trials that we announce or publish may change materially as more patient data become available and audit and verification procedures are complete.
- We face substantial generic and other competition, and our operating results will suffer if we fail to compete effectively.
- Healthcare reform initiatives, unfavorable pricing regulations, and changes in reimbursement practices of third-party payers or patients' access to insurance coverage could affect the pricing of and demand for our products.
- We are dependent on third parties to manufacture and distribute our products.
- The market opportunities for our products and product candidates may be smaller than we believe they are.
- Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval or commercialization.
- We do not currently have patent protection for certain of our commercial products. If we are unable to obtain and maintain protection for the intellectual property relating to our technology and products, their value will be adversely affected.
- We expect to rely on orphan drug status to develop and commercialize certain of our products and product candidates, but our orphan drug designations may not confer marketing exclusivity or other expected commercial benefits.
- If we are unable to obtain and maintain coverage and adequate reimbursement from governments or third-party payers for any products that we may develop or if we are unable to obtain acceptable prices for those products, our prospects for generating revenue and achieving profitability will suffer.
- We will likely experience fluctuations in operating results and could incur substantial losses.
- Negative publicity regarding any of our products could impair our ability to market any such product and may require us to spend time and money to address these issues.
- We may need substantial funding and may be unable to raise capital when needed.
- We may not receive some or all of the potential milestone and/or royalty payments from our corporate and licensing transactions.
- We may be unable to successfully integrate new products or businesses we may acquire.
- We may become involved in litigation matters, which could result in substantial costs, divert management's attention and otherwise have a material adverse effect on our business, operating results or financial condition.

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

PART I

In this Annual Report on Form 10-K, unless the context requires otherwise, the terms “we”, “our”, “us”, “Travere” and the “Company” refer to Travere Therapeutics, Inc., a Delaware corporation, as well as our direct and indirect subsidiaries.

We own or have rights to various trademarks used in our business, including those referenced in the subsection of Item 1 below titled “Trademarks”. Our logos and trademarks are the property of Travere Therapeutics, Inc. All other brand names or trademarks appearing in this report are the property of their respective holders.

ITEM 1. BUSINESS

Those statements in the following discussion that are not historical in nature should be considered forward-looking statements that are inherently uncertain. Actual results and the timing of the events may differ materially from those contained in these forward-looking statements due to a number of factors, including those discussed in the “Cautionary Statement Regarding Forward-Looking Statements” and “Risk Factors” set forth elsewhere in this Annual Report.

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. In September 2024, the U.S. Food and Drug Administration (the FDA) granted full approval to our lead development program, FILSPARI (sparsentan), which is indicated to slow kidney function decline in adults with primary IgAN who are at risk of disease progression. IgAN is a rare progressive kidney disease and the most common type of primary glomerulonephritis worldwide. FILSPARI had previously been granted accelerated approval in February 2023 based on the surrogate marker of proteinuria. Full approval was based on positive long-term confirmatory results from the PROTECT Study demonstrating that FILSPARI significantly slowed kidney function decline over two years compared to irbesartan. Sparsentan is also in late-stage development for FSGS. FSGS is a rare kidney disease and leading cause of kidney failure with no approved treatment options. In February 2025, we announced that we had completed a Type C meeting with the FDA and that we plan to submit an sNDA around the end of the first quarter of 2025 seeking traditional approval of FILSPARI for FSGS. We are also advancing pegtibatinase, a novel investigational enzyme replacement therapy for the treatment of HCU, a genetic disorder caused by a deficiency in a pivotal enzyme essential to the body. We are conducting a pivotal Phase 3 study to support the potential approval of pegtibatinase as the first disease modifying therapy for HCU. In addition, we continue to evaluate potential opportunities to expand our pipeline and approved products through licenses and acquisitions of products in areas that will serve rare disease patients with serious unmet medical need and that we believe offer attractive growth characteristics. Our research and development efforts are at the forefront of our mission to address the unmet needs of patients and we support this innovation by reinvesting revenues from our commercialized products. We are committed to ensuring broad access and educational and diagnostic support for patients.

Our Strategy

Our vision is to become a leading biopharmaceutical company dedicated to the delivery of innovation and hope to patients in the global rare disease community. In order to achieve our vision, we intend to:

- **Focus on developing products to treat rare diseases characterized by severe unmet medical needs.** We believe that our research, development, and commercialization capabilities in rare disease represent distinct competitive advantages. We leverage our development capabilities in rare disease to focus on advancing therapeutic candidates with life-changing potential. Given these capabilities, the well-established regulatory model and the ability to demonstrate clinical effects in small clinical studies, we believe that we can successfully bring new therapies to patients living with severe unmet medical needs.

- **Leverage our commercialization expertise to effectively deliver the therapies we develop.** Our strategy is to maximize the benefit and value of our commercial products through commercial execution and expertise needed to successfully support rare disease patients. Our approach may vary depending on the product and will be based on a number of factors including capital necessary to execute on each option, size of the market and terms of potential collaboration and/or licensing offers from other pharmaceutical and biotechnology companies with respect to jurisdictions outside the United States.

- **Develop a sustainable pipeline by employing disciplined decision criteria in the evaluation of potential in-licensing candidates.** We seek to build a sustainable product pipeline by employing multiple therapeutic approaches and by developing or acquiring orphan drug candidates. We seek to augment our internally developed pipeline projects by selectively and strategically acquiring pipeline assets that will add value to the portfolio. We continue to evaluate potential in-licensing, out-licensing and other potential relationships with other pharmaceutical or biotechnology companies. We intend to mitigate risk by employing rigorous decision criteria, favoring therapeutic candidates that have undergone at least some clinical study. Our decision to acquire rights to a therapeutic candidate also depends on the

scientific merits of the available clinical data; the identifiable orphan patient population; the economic terms of any proposed acquisition of rights; the projected amount of capital required to develop the therapeutic candidate; and the economic potential of the therapeutic candidate, should it be commercialized. We believe this strategy minimizes our clinical development risk and allows us to accelerate the development and potential commercialization of current and future therapeutic candidates.

- **Listen to patients.** Leadership in rare disease demands attention beyond innovative medicines. By listening to patients and leaders in the rare disease community, including those who have traditionally been underserved, we are focusing on barriers that prevent some patients from accessing the incredible innovation that our industry is delivering, including access to clinical trials and rare disease specialists.

- **Support earlier diagnosis.** We support efforts in furtherance of enabling earlier diagnosis. The growth of our commercial business reflects the strong capabilities of our commercial and medical teams to work across multiple medical specialties to help patients find a diagnosis.

Our Pipeline and Approved Products

We have a diversified pipeline designed to address areas of high unmet need in rare kidney and metabolic diseases. We invest revenues from our commercial portfolio into our pipeline with the goal of delivering new treatments for diseases with limited or no approved therapies.

The following table summarizes the status of our clinical programs, preclinical programs and approved products, each of which is described in further detail below.

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

2 On February 11, 2025, we announced that we had completed a Type C meeting with the FDA and that we plan to submit an sNDA around the end of the first quarter of 2025 seeking traditional approval of FILSPARI for FSGS.

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

FILSPARI® (sparsentan)

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

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

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

benefit of FILSPARI on absolute eGFR accrued over time and by Week 110 resulted in a 3.8 mL/min/1.73 m^2 difference in the mean change from baseline between FILSPARI and irbesartan.

Results from the PROTECT Study showed that FILSPARI was well tolerated with a clearly defined safety profile that has been consistent across all clinical trials conducted to date.

FILSPARI is a dual endothelin angiotensin receptor antagonist (DEARA). Pre-clinical data have shown that blockade of both endothelin type A and angiotensin II type 1 pathways in forms of rare chronic kidney disease, reduces proteinuria, protects podocytes and prevents glomerulosclerosis and mesangial cell proliferation. FILSPARI has been granted seven years of Orphan Drug Exclusivity in the U.S. (running from the date of accelerated approval) for the reduction of proteinuria in adults with primary IgAN at risk of rapid disease progression, and has been granted a separate seven years of Orphan Drug Exclusivity in the U.S. (running from the date of full approval) to slow kidney function decline in adults with primary IgAN who are at risk for disease progression, excluding the use provided for in the aforementioned Orphan Drug Exclusivity granted in connection with the accelerated approval.

IgAN is characterized by hematuria, proteinuria, and variable rates of progressive renal failure. With an estimated prevalence of up to 150,000 people in the United States and greater numbers in Europe and Asia, IgAN is the most common primary glomerular disease. Most patients are diagnosed between the ages of 16 and 35, with up to 40% progressing to kidney failure within 15 years. FILSPARI is the first non-immunosuppressive therapy approved for IgAN and is the only oral, once-daily, non-immunosuppressive therapy approved for this condition that directly targets glomerular injury in the kidney by blocking two critical pathways of IgAN disease progression (endothelin-1 and angiotensin II). We estimate more than 70,000 patients in the United States to be addressable under FILSPARI's full approval indication.

Data to support the approval of FILSPARI was generated from the Phase 3 PROTECT Study, the largest head-to-head interventional study to date in IgAN. It is a global, randomized, multicenter, double-blind, parallel-arm, active-controlled clinical trial that evaluated the safety and efficacy of 400mg of sparsentan, compared to 300mg of irbesartan, in 404 patients ages 18 years and up with IgAN and persistent proteinuria despite available angiotensin converting enzyme (ACE) inhibitor or angiotensin receptor blockers (ARB) therapy, and is currently ongoing in the open label extension phase of the study.

FILSPARI is available only through a risk evaluation and mitigation strategy (REMS) approved by the FDA, 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. The Company submitted an sNDA for a potential modification to the frequency of liver monitoring for FILSPARI; the sNDA has been accepted for review by the FDA and assigned a Prescription Drug User Fee Act (PDUFA) target action date of August 28, 2025.

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 countries. CSL Vifor submitted an application for full regulatory approval in the second 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 November 2024, the Medicines and Healthcare products Regulatory Agency (MHRA) approved FILSPARI in the United Kingdom.

In January 2024, we announced our entry into an exclusive licensing agreement with Renalys Pharma, Inc. (Renalys), to bring sparsentan, for the treatment of IgAN, to patients in Japan and other countries in Asia. Renalys 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 registration study of sparsentan in Japan to support potential approval of sparsentan in Japan. In July 2024, Renalys announced that the first patient was dosed in the study, and in January 2025, Renalys announced achievement of full enrollment in the study. Results from the urine protein/creatinine ratio (UP/C) endpoint in the study are expected in the second half of 2025 to support a submission for approval to PMDA. In December 2024, Renalys announced that sparsentan received Orphan Drug Designation from the Japanese Ministry of Health, Labour and Welfare for the indication of primary IgA nephropathy as of November 27, 2024. 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 pre-specified secondary endpoints on proteinuria and exploratory endpoints, including renal outcomes, which trended favorably for sparsentan. In addition, a review of the safety results through 108 weeks of treatment indicate sparsentan was generally well-tolerated and the overall safety profile in the study to date was generally consistent between treatment groups.

In December 2023, we announced that we completed a planned Type C meeting with the FDA to discuss results from the Phase 3 DUPLEX Study of sparsentan in FSGS. The FDA acknowledged the high unmet need for approved therapies as well as the challenges in studying FSGS but indicated that the two-year results from the Phase 3 DUPLEX Study alone were not sufficient to support an sNDA submission. The FDA acknowledged the work being done by the larger nephrology community to better understand proteinuria and eGFR as endpoints in clinical trials of FSGS and indicated a willingness to continue to engage with us on a potential path forward for sparsentan in FSGS following our consideration of additional evidence. Subsequently, a collaborative international effort referred to as the PARASOL project was initiated with a goal to define the quantitative relationships between short-term changes in biomarkers (proteinuria and GFR) and long-term outcomes in order to support the use of alternative proteinuria-based endpoints as a basis for accelerated and traditional approval. The PARASOL project is led by several patient advocacy organizations focused on glomerular diseases, with participation from regulators and industry representatives. The principal finding from PARASOL was that in FSGS, reduction in proteinuria over 24 months is strongly associated with a reduction in the risk of kidney failure, and responder definitions based on thresholds of proteinuria are both biologically plausible and strongly supported by epidemiological data. Following the 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 scheduled a Type C meeting with the FDA to discuss a potential regulatory pathway for a sparsentan FSGS indication. In February 2025, we announced that we completed a Type C meeting with the FDA and that we plan to submit an sNDA around the end of the first quarter of 2025 seeking traditional approval of FILSPARI for FSGS. The sNDA will be based on existing data from the Phase 3 DUPLEX and Phase 2 DUET studies of FILSPARI.

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.

Under the terms of our exclusive license to CSL Vifor, CSL Vifor is responsible for all commercialization activities in its licensed territories. We remain responsible for the clinical development of sparsentan in the applicable territories. If sparsentan receives marketing authorization in any of the territories covered by the exclusive license to Renalys, Renalys will be responsible for all development, regulatory matters, and commercialization activities in such licensed territories. We will retain all rights to sparsentan in the United States and rest of world outside of the territories licensed to CSL Vifor and Renalys, provided that CSL Vifor has a right of negotiation to expand the licensed territories into Canada, 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. We estimate that there are approximately 7,000 to 10,000 addressable HCU patients globally. Pegtibatinase has been granted Rare Pediatric Disease, Fast Track and Breakthrough Therapy designations by the FDA, as well as orphan drug designation in the United States and European Union.

In December 2021, we announced positive topline results from the Phase 1/2 COMPOSE Study, a double blind, randomized, placebo-controlled dose escalation study to assess its safety, tolerability, pharmacokinetics, pharmacodynamics and clinical effects in patients with classical HCU. Pegtibatinase demonstrated dose-dependent reductions in total homocysteine (tHcy) during the 12 weeks of treatment, and in the highest dose cohort to date evaluating 1.5 mg/kg of pegtibatinase twice weekly (BIW), treatment with pegtibatinase resulted in rapid and sustained reductions in total homocysteine (tHcy) through 12 weeks of treatment, including a 55.1% mean relative reduction in tHcy from baseline as well as maintenance of tHcy below a clinically meaningful threshold of 100 µmol. Additionally, in a dose-dependent manner in the study to date, methionine levels were substantially reduced and cystathionine levels were substantially elevated following treatment with pegtibatinase, suggesting that pegtibatinase acts in a manner similar to the native CBS enzyme.

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

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

In September 2024, we announced a voluntary pause of enrollment in the Phase 3 HARMONY Study. The voluntary enrollment pause enables us to work to address necessary process improvements in manufacturing scale-up to support commercial scale manufacturing as well as full enrollment in the HARMONY Study. Patients currently enrolled in pegtibatinase studies continue to receive study medication from small scale batches which are unaffected by the scale-up process. Currently enrolled patients will be able to continue on study medication as scheduled for the duration of the trials in which they are participating. 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 are making progress on necessary process improvements in manufacturing scale-up and currently anticipate that we should be in position to restart enrollment in the Phase 3 HARMONY Study in 2026.

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

Preclinical Program:

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 U.S. is estimated to be 10,000 to 12,000, indicating that there may be as many as 4,000 to 5,000 affected individuals with cystinuria in the U.S. that would be candidates for Thiola or Thiola EC.

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

In May 2021, a generic option for the 100mg version of the original formulation of Thiola (tiopronin tablets) became available and in June 2022, a second option for the 100mg version of the original formulation of Thiola (tiopronin tablets) was approved. These generic versions of the original formulation of Thiola have impacted our sales, and these or additional generic versions of either formulation could have a material adverse impact on sales. As of December 31, 2024, several generic options for the 100mg and 300mg versions of Thiola EC have been approved by the FDA and become available. Accordingly, Thiola EC is subject to generic competition.

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". This transaction was consummated on August 31, 2023.

Competition

The pharmaceutical and biotechnology industries are intensely competitive and subject to rapid and significant technological change. Many of our competitors are larger than our company and have substantially greater financial, marketing and technical resources than we have.

The development and commercialization of new products to treat rare diseases is highly competitive, and we expect considerable competition from major pharmaceutical, biotechnology and specialty pharmaceutical companies. As a result, there are, and will likely continue to be, extensive research and substantial financial resources invested in the discovery and development of new orphan drug products.

Our competition will be determined in part by the potential indications for which therapies are developed and ultimately approved by regulatory authorities. The speed with which we can develop products, complete preclinical testing, clinical trials, approval processes, and supply commercial quantities to market are expected to be important competitive factors. We expect that competition among products approved for sale will be based on various factors, including product efficacy, safety, reliability, availability, price, reimbursement, patent position, and regulatory exclusivity.

FILSPARI/Sparsentan

IgAN

FILSPARI (sparsentan) is the first non-immunosuppressive therapy approved for IgAN and is the only oral, once-daily, non-immunosuppressive medication that directly targets glomerular injury in the kidney by blocking two critical pathways of IgAN disease progression (endothelin-1 and angiotensin II). FILSPARI received accelerated approval in the U.S. in February 2023 and full approval in September 2024 and is indicated to slow kidney function decline in adults with primary IgAN who are at risk for disease progression. In April 2024, the European Commission granted conditional marketing authorization (“CMA”) for FILSPARI for the treatment of adults with primary IgAN with a urine protein excretion ≥1.0 g/day (or urine protein-to-creatinine ratio ≥0.75 g/g).

The IgAN treatment landscape is evolving rapidly. Historically, traditional standard of care has been renin-angiotensin-aldosterone system (RAAS) blockade with immunosuppression. RAAS blockade is also commonly used in patients with significant proteinuria or rapidly progressive glomerulonephritis. In August 2024, Kidney Disease Improving Global Outcomes (“KDIGO”) published draft guidelines for public comment that include updated recommendations for the treatment of IgAN. The draft KDIGO guidelines recommend FILSPARI as a foundational kidney-targeted therapy for IgAN patients who are at risk of progressive kidney function loss, and advocate for lowering the targeted proteinuria level for all IgAN patients to under 0.5 g/day, or preferably under 0.3 g/day. The draft KDIGO guidelines suggest that combination treatment should be initiated simultaneously in most cases where patients are at risk of progressive kidney function decline. FILSPARI is the only oral, once-daily, non-immunosuppressive medication that directly targets glomerular injury in the kidney by blocking two critical pathways of IgAN disease progression: endothelin-1 and angiotensin II. This aligns with the draft KDIGO guidelines' emphasis on early diagnosis, aggressive proteinuria reduction, and the use of combination therapies to manage IgAN.

In addition to FILSPARI, other therapies currently available in the IgAN market include RAAS blockade therapies, and IgAN-indicated treatments including Calliditas’ Tarpeyo (budesonide delayed-release capsules), and Novartis’ iptacopan (Fabhalta) (a Factor B complement inhibitor currently approved under accelerated approval). In addition, interim proteinuria data from Novartis’ Phase 3 IgAN study (ALIGN) with atrasentan (an endothelin receptor antagonist, or ERA) read out in 2024, and the company disclosed that their NDA was submitted to FDA in the second quarter of 2024, and that a potential approval could take place in the first half of 2025. Based on public sources, the confirmatory portions of the Phase 3 studies of iptacopan and atrasentan are expected to begin reading out in the fourth quarter of 2025 or in 2026. Additionally, Novartis has an ongoing Phase 3 IgAN study with zigakibart (an anti-APRIL monoclonal antibody (mAb)).

Based on public sources, multiple companies have programs in clinical and/or pre-clinical development for the treatment of IgAN or related conditions, including several Phase 3 IgAN programs that are ongoing and/or enrolling in the United States, Europe, and parts of Asia: (1) Otsuka

Corporation with sibeprenlimab (anti-APRIL mAb), (2) Roche with sefaxersen (Factor B complement inhibitor) (licensed via Ionis Pharmaceuticals), (3) Vera Therapeutics with atacicept (BAFF / APRIL inhibitor), (4) AstraZeneca with ravulizumab (Ultomiris) (C5 complement inhibitor), and (5) Vertex Pharmaceuticals Inc. with povetacicept (BAFF / APRIL inhibitor). RemeGen is reportedly conducting a Phase 3 IgAN study with telitacicept in China only. In 2025, we are anticipating at least two additional Phase 3 IgAN studies to be initiated, including Biogen's felzartamab (anti-CD38) and Takeda Pharmaceutical Company's mezagitamab (anti-CD38).

In October 2024, Otsuka announced that sibeprenlimab (anti-APRIL mAb) met its primary endpoint at the prespecified interim analysis and potentially will submit for accelerated approval in 2025.

In 2021-2023, there were global regulatory label expansions of two SGLT2 inhibitors, AstraZeneca's Farxiga/Forxiga and Boehringer Ingelheim and Eli Lilly's Jardiance, in chronic kidney disease, which could potentially be complementary to sparsentan for the treatment of IgAN and/or FSGS. Additionally, although patients diagnosed with IgAN are not part of the label (nor were they included in the initial pivotal studies), Bayer's non-steroidal mineralocorticoid receptor antagonist, Kerendia, could be used in patients with diabetic kidney disease and theoretically, those with concurrent IgAN and is currently studied in patients with chronic kidney disease without diabetes. Finally, there are endothelin receptor antagonists, or ERAs, that are developed for chronic kidney disease (CKD) and CKD-related conditions which could potentially be used (if approved) in CKD patients without a specific IgAN diagnosis. Idorsia Pharmaceuticals' aprocitentan (branded as Tryvio in the U.S. and Jeraygo in Europe) was approved for the treatment of resistant hypertension by the FDA and EMA, in March 2024 and June 2024, respectively; the pivotal study was in patients with resistant hypertension, including those with Stage 3 & 4 CKD. In November 2023, AstraZeneca initiated a registrational fixed-dose combination (FDC) study with zibotentan and Farxiga in CKD patients with high proteinuria. Patients with IgAN are not excluded from the study.

FSGS

Currently, there are no products approved by the FDA or the European Commission for the treatment of FSGS. The current standard of care for FSGS includes steroids, ACE/ARBs, calcineurin inhibitors, dialysis, and renal transplant. Moreover, as an adjuvant apheresis device which works to remove lipoproteins from blood, the Liposorber® LA-15 System (Kaneka Pharma America LLC) is FDA-approved for the treatment of adult and pediatric patients with Nephrotic Syndrome associated with primary FSGS when standard options are unsuccessful or post-transplant with FSGS recurrence. A post-approval study with this device is ongoing.

Dimerix is conducting a Phase 3 study in pediatric and adult FSGS patients with their CCR2 blocker, DMX-200. Dimerix has indicated that an interim analysis will be made available in the second half of 2025.

Additionally, Vertex Pharmaceuticals is conducting the Phase 3 portion of their Phase 2/3 study in adult and pediatric patients with APOL1-mediated kidney disease, which can include a subset of FSGS patients with known APOL1 mutations (G1/G1, G2/G2, or G1/G2).

Based on public sources, several other companies have programs in clinical and/or pre-clinical development for the treatment of FSGS or related conditions.

Pegtibatinase

Current treatment options for homocystinuria (HCU) are limited to protein-restricted diet and supplemental use of vitamin B6 and betaine.

According to public sources, Syntis Bio, Inc. has a pre-clinical development program for a microbiome therapy for the management of HCU. Additionally, in July 2024, Innorna announced that they received FDA's Rare Pediatric Disease Designation for IN022 (pre-clinical) for the treatment of HCU.

Based on public sources, there are other pre-clinical development programs in HCU that may enter the clinic.

Thiola and Thiola EC (tiopronin)

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. As of December 31, 2024, several generic options for the 100mg and 300mg versions of Thiola EC have been approved by the FDA and become available. Accordingly, Thiola EC is subject to generic competition. Thiola also faces potential competition from compounded formulations and additional generic entrants.

In addition, certain penicillamine agents including but not limited to Cuprimine and Depen are FDA approved for the treatment of cystinuria. Additional generic versions of penicillamine have been approved by the FDA and some have entered the market. Captopril is not FDA approved for the treatment of cystinuria but has been prescribed for patients with cystinuria. Advicenne Pharma is developing a microtablet formulation containing potassium citrate monohydrate and potassium bicarbonate, for the potential oral treatment of cystinuria.

Based on public sources, there are other preclinical assets in development that may enter the clinic for the treatment of cystinuria.

Licenses and Royalties

Ligand License Agreement

In 2012, we entered into a license agreement with Ligand Pharmaceuticals, Inc. ("Ligand"), granting us a worldwide sublicense for the development, manufacture and commercialization of FILSPARI (sparsentan). Under the license agreement, Ligand granted us a sublicense under certain of its patents and other intellectual property in connection with the development and commercialization of sparsentan. Under the license agreement, Ligand is obligated to transfer to us certain information, records, regulatory filings, materials and inventory controlled by Ligand and relating to or useful for developing sparsentan. We must use commercially reasonable efforts to develop and commercialize sparsentan in specified major market countries and other countries in which we believe it is commercially reasonable to develop and commercialize such products.

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 December 31, 2024, we have capitalized $47.2 million for contractual milestones achieved under the Ligand License Agreement, including $5.8 million for the year ended December 31, 2024. Pursuant to the Ligand License Agreement, we are 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. We began incurring costs associated with such royalties following the February 2023 approval of FILSPARI (sparsentan). For the years ended December 31, 2024 and 2023, we capitalized $20.3 million and $4.4 million, respectively, to intangible assets for royalties owed on net sales of FILSPARI.

Under the terms of the license agreement, BMS has a right of first negotiation and Ligand has a right of second negotiation with respect to any license arrangement for a licensed compound, except to the extent such rights may be waived.

The license agreement will continue until neither party has any further payment obligations under the agreement and is expected to continue for approximately 10 to 20 years from the effective date. Ligand may terminate the license agreement due to (i) our insolvency, (ii) our material uncured breach of the agreement, (iii) our failure to use commercially reasonable efforts to develop and commercialize sparsentan as described above or (iv) certain other conditions. We may terminate the license agreement due to a material uncured breach of the agreement by Ligand.

Mission License Agreement

In 2014, we entered into a license agreement with Mission Pharmacal, 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. We paid Mission an up-front license fee of $3.0 million and over the term of the agreement will pay the greater of $2.0 million, representing the guaranteed minimum royalty, or 20% of our net sales of Thiola in the United States and Canada during each calendar year.

In October 2015, the license agreement was amended to allow for us to secure enough API to ensure an adequate level of safety stock to prevent an interruption in the supply of Thiola and to prepare for a reformulation development project.

In March 2016, the license agreement was amended to, among other things, include a new formulation development project for tiopronin tablets.

In November 2017, we amended the license agreement to extend the term through May 2029.

In November 2018, the license agreement was amended to remove all territorial restrictions on our license rights. As consideration for the expanded territory, we paid an up-front fee of $0.3 million and will pay the greater of $0.1 million, representing the guaranteed minimum royalty, or 20% of our Thiola net sales generated outside of the United States during each calendar year.

Intellectual Property

The proprietary nature of, and protection for, our product candidates and our discovery programs, processes and know-how are important to our business. We have sought patent protection in the United States and certain other jurisdictions for sparsentan, where available and when appropriate. Our policy is to pursue, maintain and defend patent rights, whether developed internally or licensed from third parties, and to protect the technology, inventions and improvements that are commercially important to the development of our business.

Our commercial success will depend in part on obtaining and maintaining patent protection and for our current and future product candidates, their use in treating particular diseases, and the methods used to develop and manufacture them, as well as successfully defending these patents against third-party challenges. Our ability to stop third parties from making, using, selling, offering to sell, or importing our products depends on the extent to which we have rights under valid and enforceable patents that cover these activities. We cannot be sure that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications filed by us in the future, nor can we be sure that any of our existing patents or any patents that may be granted to us in the future will be commercially useful in protecting our product candidates, discovery programs and processes.

FILSPARI

As of February 1, 2025, our patent portfolio for FILSPARI (sparsentan) was comprised of six distinct patent families, one of which is exclusively licensed from Ligand (the “Ligand patent family”). The other five patent families are owned by Travere (the "Travere patent families"). We previously had one additional licensed patent family related to sparsentan that was owned by BMS, exclusively licensed to Ligand and sub-licensed to us by Ligand. However, the BMS patent family expired in 2019 and therefore is no longer in force.

The Ligand patent family is directed to methods of using sparsentan in the treatment of various diseases, including glomerulosclerosis and IgAN. As of December 31, 2024, this patent family included two U.S. patents (U.S. Patent No. 9,662,312, which we refer to herein as the ‘312 patent, and U.S. Patent No. 9,993,461, which we refer to herein as the ‘461 patent), a pending U.S. application, 18/797,336, filed August 25, 2024, two European patents (European Patent No. EP2732818, which we refer to herein as the European ‘818 patent, and European Patent No. EP3222277, which we refer to herein as the European ‘277 patent), a pending European application, and two granted Hong Kong patents. The ‘312 patent and the European ‘818 patent claim the use of sparsentan for treating glomerulosclerosis. The ‘461 patent and the European '277 patent each claim both the use of sparsentan for treating glomerulosclerosis and the use of sparsentan for treating IgAN. The U.S. and foreign patents in this patent family have a stated expiration date in March 2030, which may potentially be extended in the United States via a pending patent term extension application. In November 2020, a third party filed an opposition to the European ‘277 patent. In March 2024, our patent claims were upheld by the European Patent Office Board of Opposition and in May the opponent filed an appeal thereto. We are continuing to vigorously defend the European '277 against the opposition. If we were to be unsuccessful in doing so, we would expect to rely on the data and/or marketing exclusivity that may be available in the EU, as described below under “Regulatory Exclusivity.”

The first Travere patent family is directed to methods of using sparsentan in the treatment of various kidney diseases, including focal segmental glomerulosclerosis (FSGS) and IgAN, e.g., by achieving a specified urine protein-to-creatinine ratio. As of December 31, 2024, this patent family was comprised of a granted U.S. patent (U.S. Patent No. 10,864,197, which we refer to herein as the ‘197 patent) and pending patent applications in the U.S., Australia, Brazil, Canada, China, the European Patent Office, Hong Kong, Japan, Korea, New Zealand and South Africa. The ‘197 patent claims use of sparsentan for treating Alport syndrome and has a stated expiration date in October 2037. The pending patent claims in this patent family are directed at the use of sparsentan for treating FSGS and IgAN.

The second Travere patent family is directed to methods of using sparsentan for treating hearing loss associated with Alport syndrome. As of December 31, 2024, this patent family was comprised of a granted U.S. patent (U.S. Patent No. 11,207,299) and pending patent applications in the U.S., Australia Brazil, Canada, China, the European Patent Office, Hong Kong, Israel, Japan, Korea, Mexico, New Zealand and South Africa.

The third Travere patent family is comprised of a pending international patent application, related to methods of treating a kidney disease or disorder comprising sparsentan, and a SGLT2 inhibitor.

The fourth Travere patent family is comprised of a pending international patent application, related to methods of treating IgA mediated diseases or disorders comprising sparsentan.

The fifth Travere patent family is comprised of a pending international patent application, related to methods of treating IgA mediated kidney diseases or disorders comprising sparsentan.

The sixth Travere patent family is comprised of a pending provisional patent application directed to additional methods of use of sparsentan for treating FSGS and IgAN.

The U.S. and some countries outside the U.S. similarly offer forms of patent term extension or restoration. For example, Supplementary Protection Certificates are available to extend the life of a European patent up to an additional five years (subject to a 15-year cap from European Medicines Agency (EMA) approval) and in Japan patent terms can be extended up to five years.

We have filed an application for patent term extension of the ‘461 patent, which may be extended under the provisions of the Hatch-Waxman Act. Patent term extensions or Supplemental Protection Certificates (SPCs) also may be available in certain foreign jurisdictions upon regulatory approval.

In the EU, a conditional marketing authorization (“CMA”) for FILSPARI was issued by the European Commission in April 2024 (the ”FILSPARI CMA”). Because there has been no prior European approval for FILSPARI, we and Vifor are pursuing SPCs throughout the European Economic Area (EEA) based on this FILSPARI CMA.

Likewise, it is possible, if sparsentan achieves regulatory approval elsewhere in the world, depending upon local law and regulation, patent term extensions or SPCs may be pursued in additional jurisdictions.

Pegtibatinase

As of December 31, 2024, our patent portfolio for pegtibatinase was comprised of six distinct patent families, which we obtained upon our acquisition of Orphan Technologies Limited (now Travere Therapeutics Switzerland GmbH). Orphan Technologies obtained the rights to the first five patent families under an exclusive license agreement with the University of Colorado, while the sixth patent family was owned by Orphan Technologies. The first three patent families are owned by the University of Colorado and exclusively licensed to Travere Therapeutics Switzerland GmbH (the “CU patent families”). The first CU patent family is directed to human cystathionine β-synthase variants and methods for their production, the second CU patent family is directed to methods of purifying human cystathionine β-synthase variants, and the third CU patent family is directed to compositions comprising human cystathionine β-synthase variants and methods of treating homocystinuria. The next two families are co-owned by the University of Colorado and Travere Therapeutics Switzerland GmbH, with the University of Colorado’s interest exclusively licensed to Travere Therapeutics Switzerland GmbH (the “co-owned patent families”). The first co-owned patent family is directed to methods of pegylating human cystathionine β-synthase variants, while the second co-owned patent family is directed to pharmaceutical formulations comprising pegylated human cystathionine β-synthase variants and their use in treating homocystinuria. Lastly, the sixth patent family is owned by Travere Therapeutics Switzerland GmbH (the “Travere patent family”). The Travere patent family is directed to methods for treating human cystathionine β-synthase deficiency in patients with elevated homocysteine levels.

Thiola

Our patent portfolio for Thiola is comprised of a patent family which is exclusively licensed from Mission Pharmacal (the “Mission patent family”). The Mission patent family is directed to a new formulation of Thiola, known as Thiola EC. As of December 31, 2024, this patent family included a granted U.S. patent (U.S. Patent No. 11,458,104, which we refer to herein as the ‘104 patent) and a pending U.S. patent application. The '104 patent claims a method for treating cystinuria by administering a formulation of tiopronin with food.

Regulatory Exclusivity

If we obtain marketing approval for sparsentan for the treatment of FSGS in the United States or in certain jurisdictions outside of the United States, we may be eligible for regulatory exclusivity for the approved therapy. For example, in the United States, an FDA-approved therapy may be eligible to receive five years of new chemical entity ("NCE") exclusivity or, for drugs granted an orphan designation by the FDA, seven years of orphan drug exclusivity ("Orphan Drug Exclusivity" or "ODE"). In addition, both the five-year NCE period and the seven-year ODE period may be extended by six months upon submission of satisfactory written reports to the FDA of clinical studies conducted in pediatric populations.

Likewise, if we obtain marketing approval for pegtibatinase in the United States or in certain jurisdictions outside of the United States, we may be eligible for regulatory exclusivity for the approved product. Pegtibatinase is a biologic product and therefore its application for approval would be via a biologic license application (“BLA”). In the United States, an FDA-approved biologic product may be eligible to receive twelve years of regulatory exclusivity. In addition, the twelve-year BLA exclusivity period may be extended by six months upon submission of satisfactory written reports to the FDA of clinical studies conducted in pediatric populations.

In the European Union and European Free Trade Association ("EFTA") countries, innovative medicinal products approved by the European Commission may receive eight years of data exclusivity and 10 years of market exclusivity. The 10-year period of market exclusivity may extend to 11 years if, during the eight-year period of data exclusivity, the product receives marketing authorization for a second therapeutic that provides a significant clinical benefit in comparison to existing therapies. Additionally, upon approval by the European Commission, orphan drugs may receive 10 years of market exclusivity or, in the case of orphan drugs for which a pediatric investigational plan (PIP) has been completed, 12 years of market exclusivity. This period of market exclusivity 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. The regulatory exclusivity periods run from the date of European Commission approval. There can be no assurance that we will qualify for any such regulatory exclusivity, or that any such exclusivity will prevent competitors from seeking approval solely on the basis of their own studies. See “Government Regulation” below.

FILSPARI

FILSPARI (sparsentan) received orphan drug designation in the United States and in the European Union for the treatment of IgAN in 2021 and FSGS in 2015. FILSPARI has been granted seven years of Orphan Drug Exclusivity in the U.S. (running from the date of accelerated approval) for the reduction of proteinuria in adults with primary IgAN at risk of rapid disease progression, and has been granted a separate seven years of Orphan Drug Exclusivity in the U.S. (running from the date of full approval) to slow kidney function decline in adults with primary IgAN who are at risk for disease progression, excluding the use provided for in the aforementioned Orphan Drug Exclusivity granted in connection with the accelerated approval.

Thiola

Thiola does not have regulatory exclusivity in the United States.

Trademarks

Our trademark portfolio includes both Travere-owned and Travere-licensed trademarks and is comprised of various U.S. and foreign registered trademarks and pending trademark applications relating to our company name, our commercial products (FILSPARI, Thiola, and Thiola EC), and sparsentan.

More specifically, as of December 31, 2024, our trademark portfolio included registered U.S. and foreign trademarks for the wordmark “Travere Therapeutics” and its logo, registered U.S. and foreign trademarks relating to FILSPARI (sparsentan), registered U.S. trademarks for both the wordmark “TOTALCARE”, and its logo, a registered trademark for both the wordmark “TOTAL CARE HUB” and its logo, and a registered trademark for “In Rare for Life”. In addition, under our license agreement with Mission we have an exclusive license to use Mission’s trademarks related to Thiola and Thiola EC, including three registered U.S. trademarks and one registered Canadian trademark for the mark “THIOLA”, and one registered U.S. trademark for the mark "Thiola EC", in the United States and Canada.

Trade Secrets

In addition to patents, we rely on trade secrets and know-how to develop and maintain our competitive position. We seek to protect our proprietary data and processes, in part, by confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors, and partners. These agreements are designed to protect our proprietary information. We also seek to preserve the integrity and confidentiality of our data, trade secrets and know-how by maintaining physical security of our premises and physical and electronic security of our information technology systems. Trade secrets and know-how can be difficult to protect. Consequently, we anticipate that trade secrets and know-how will, over time, be disseminated within the industry through independent development, the publication of journal articles, and the movement of personnel skilled in the art from academic to industry scientific positions.

Manufacturing

STA Pharmaceutical Hong Kong Limited manufactures the active pharmaceutical ingredient for FILSPARI. Catalent Pharma Solutions manufactures FILSPARI and performs primary packaging. PCI Pharma Services performs secondary packaging and serialization for FILSPARI. Mission Pharmacal manufactures Thiola and Thiola EC.

We intend to continue to use our financial resources to accelerate development of our therapeutic candidates rather than establishing our own manufacturing facilities. We intend to meet our preclinical and clinical trial manufacturing requirements by establishing relationships with third-party manufacturers and other service providers to perform these services for us. Because we rely on these third parties, we have personnel with pharmaceutical development and manufacturing experience who are responsible for maintaining our third-party manufacturing relationships.

Should any of our therapeutic candidates obtain marketing approval, we anticipate establishing relationships with third-party manufacturers and other service providers in connection with the commercial production of our products. We have some flexibility in securing other manufacturers to produce our therapeutic candidates; however, our alternatives may be limited due to proprietary technologies or methods used in the manufacture of some of our therapeutic candidates.

Sales, Marketing and Distribution

In 2024, we continued to utilize our specialty sales force to market our FDA-approved products in the U.S. Through our deep understanding of patient and healthcare provider needs, we believe we are able to:

- serve patients living with rare disease that have limited treatment options;
- drive optimum performance of our marketed products;
- educate and train healthcare providers about our products and the diseases for which they are approved to treat;
- support access to and reimbursement coverage for our products without significant restrictions;
- support compliant use by providing patients with support services and disease education, to the extent and in the manner permitted under applicable laws, to help them utilize our products in a manner consistent with the label and maximize the benefits of treatment; and
- successfully launch new treatment options once approved.

Our U.S. commercial initiatives are designed to support patients living with rare diseases and clinicians treating these patients. We commercialize our products in the United States with a relatively small specialty sales force. Nephrologists are the primary call point for FILSPARI. The primary call points for Thiola and Thiola EC include urologists and nephrologists.

Our sales force is differentiated by its high level of experience, averaging more than 20 years in pharmaceutical sales including over five years of experience in rare disease. Our commercial management and operations team also has an average of more than 20 years of pharmaceutical experience focused on specialty and rare disease.

Our marketing and patient access teams, supported by third-party agencies with rare disease experience, drive our commercialization and disease awareness efforts in the United States. Specifically, we implement a variety of industry accepted programs to educate physicians, including direct-to-physician contact by sales representatives, peer-to-peer educational programs, and participation in targeted medical convention programs.

We distribute FILSPARI in the United States through two direct to patient pharmacies, and operate Travere TotalCare, pursuant to which we provide our comprehensive patient support services. This patient support program for FILSPARI in the United States provides services, assistance and resources that help patients understand IgAN, manage the insurance process, fill their prescriptions and initiate treatment.

We distribute our other products, Thiola and Thiola EC, through one direct to patient pharmacy, Eversana, who also provides our comprehensive patient support services in the United States. This patient support program includes a case-managed approach to patient education, insurance verification and reimbursement support, co-pay and other financial assistance for eligible patients, monitoring and support of adherence, and 24/7 access to pharmacist counseling.

In April 2024, we and CSL Vifor, with whom we entered into a license and collaboration agreement ("License Agreement") in September 2021, announced that the European Commission 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. Additionally, in October 2024 Swissmedic granted temporary marketing authorization for FILSPARI for the same patient population in Switzerland, and in November 2024, the Medicines and Healthcare products Regulatory Agency (MHRA) approved FILSPARI in the United Kingdom. CSL Vifor is responsible for all commercialization activities in such licensed territories. We remain responsible for the clinical development of sparsentan and will retain all rights to sparsentan in the United States and rest of world outside of the licensed territories, provided that CSL Vifor has a right of negotiation to expand the licensed territories into Canada, China, Brazil and/or Mexico.

In January 2024, we announced our entry into an exclusive licensing agreement with Renalys, to bring sparsentan for the treatment of IgAN to patients in Japan and other countries in Asia. 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, Renalys plans to initiate an open label registration study of sparsentan in Japan in the second quarter of 2024 to support potential approval of sparsentan in Japan. 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 agreement, Renalys will be responsible for development, regulatory matters, and commercialization in the licensed territories.

Medical Affairs

We have a global medical affairs team located in the United States and Europe which supports data dissemination, education, external stakeholder engagement and data generation in therapeutic areas relevant to our pipeline and commercial assets. The responsibilities of our medical affairs personnel include execution of real-world evidence studies, scientific exchange with external stakeholders, medical communication through scientific publications and presentations at medical congresses, providing medical information support to HCPs and patients related to our pipeline and our post-approval clinical commitments, organizing medical advisory boards to obtain input from experts and practitioners, and supporting grants for investigator sponsored research and independent medical education programs on medical topics relevant to our products and diseases.

Government Regulation

Regulation by government authorities in the United States and foreign countries is a significant factor in the development, manufacture and marketing of proposed products and in ongoing research and product development activities. All pipeline products will require regulatory approval by competent regulatory authorities prior to commercialization. In particular, human therapeutic products are subject to rigorous preclinical studies and clinical trials and other approval procedures of the FDA and similar regulatory authorities in foreign countries. Various federal and state statutes and regulations also govern or influence analytical testing, manufacturing, safety evaluation, labeling, storage, inspection and record-keeping related to such products and their marketing. The process of obtaining these approvals and the subsequent compliance with appropriate federal and state statutes and regulations require the expenditure of substantial time and financial and human resources.

FDA Drug Approval Process

In the United States, pharmaceutical products (drugs and biologics) are subject to extensive regulation by the FDA. The Federal Food, Drug, and Cosmetic Act, and other federal and state statutes and regulations, govern, among other things, the research, development, testing, manufacture, storage, recordkeeping, approval, labeling, promotion and marketing, distribution, post-approval monitoring and reporting, sampling and import and export of pharmaceutical products. Failure to comply with applicable U.S. requirements may subject a company to a variety of administrative or judicial sanctions, such as FDA refusal to approve pending new drug or new biologic license applications, or NDAs and BLAs, respectively, warning

or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties and criminal prosecution. Firms are also subject to potential inspection by FDA.

We cannot market a drug product candidate in the United States until the drug has received FDA approval. The key steps required before a drug may be marketed in the United States generally include the following:

- completion of extensive preclinical laboratory tests, animal studies, toxicology, pharmacology, and formulation studies in accordance with the FDA's GLP regulations;
- submission to the FDA of an IND, and oversight by an Institutional Review Board, for human clinical testing, which must become effective before human clinical trials may begin;
- performance of adequate and well-controlled human clinical trials (phase 1-3) in accordance with Good Clinical Practices ("GCP") requirements to establish the safety and efficacy of the drug for each proposed indication;
- submission to the FDA of an NDA or BLA after completion of the required pivotal clinical trials;
- satisfactory completion of any FDA pre-approval inspection of the manufacturing facility or facilities at which the active pharmaceutical ingredient, or API, and finished drug product are produced and tested to assess compliance with current Good Manufacturing Practices ("cGMPs"); and
- FDA review and approval of the NDA or BLA.

Satisfaction of FDA pre-market approval requirements typically takes many years and the actual time required may vary substantially based upon the type, complexity and novelty of the product or disease.

Preclinical tests include laboratory evaluation of product chemistry, formulation and toxicity, as well as animal trials to assess the characteristics and potential safety and efficacy of the product. The conduct of the preclinical tests must comply with federal regulations and requirements, including good laboratory practices. The results of preclinical testing are submitted to the FDA as part of an IND along with other information, including information about product chemistry, manufacturing and controls and a proposed clinical trial protocol. Long-term preclinical tests, such as animal tests of reproductive toxicity and carcinogenicity, may continue after the IND is submitted.

A 30-day waiting period after the submission of each IND is required prior to the commencement of clinical testing in humans. If the FDA has neither commented on nor questioned the IND within this 30-day period, the clinical trial proposed in the IND may begin. If the FDA raises concerns or questions about the conduct of the trial, such as whether human research subjects will be exposed to an unreasonable health risk, the IND sponsor must resolve any outstanding FDA concerns or questions before clinical trials can proceed.

Clinical trials involve the administration of the investigational new drug or biologic to healthy volunteers or patients under the supervision of a qualified investigator. Clinical trials must be conducted in compliance with federal regulations, including GCP requirements, as well as under protocols detailing the objectives of the trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Each protocol and subsequent protocol amendments must be submitted to the FDA as part of the IND. Certain studies must also be posted on clinicaltrials.gov.

The FDA may order the temporary, or permanent, discontinuation of a clinical trial at any time, or impose other sanctions, if it believes that the clinical trial either is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial patients. The study protocol and informed consent information for patients in clinical trials also must be submitted for approval to an institutional review board, or IRB, for approval at each site at which the clinical trial will be conducted. An IRB also may require the clinical trial at its site to be halted, either temporarily or permanently, for failure to comply with the IRB's requirements, or may impose other conditions.

Clinical trials to support NDAs for marketing approval are typically conducted in three sequential phases, but the phases may overlap. In Phase 1, the initial administration of the drug to healthy human subjects or patients, the drug is tested to assess pharmacological actions, side effects associated with increasing doses and, if possible, early evidence on effectiveness. Phase 2 usually involves trials in a limited patient population to determine metabolism, pharmacokinetics, the effectiveness of the drug for a particular indication, dosage tolerance and optimum dosage, and to identify common adverse effects and safety risks. If a drug demonstrates evidence of effectiveness and an acceptable safety profile in Phase 2 evaluations, Phase 3 clinical trials, also called pivotal trials, are undertaken to obtain the additional information about clinical efficacy and safety in a larger number of patients, typically at geographically dispersed clinical trial sites, to permit the FDA to evaluate the overall benefit-risk relationship of the drug and to provide adequate information for the labeling of the drug. In most cases the FDA requires two adequate and well controlled Phase 3 clinical trials to demonstrate the efficacy of the drug. A single Phase 3 clinical trial with other confirmatory evidence may be sufficient in rare instances where the study is a large multicenter trial demonstrating internal consistency and a statistically very persuasive finding of a clinically meaningful effect on mortality, irreversible morbidity or prevention of a disease with a potentially serious outcome and where confirmation of the result in a second Phase 3 trial would be impractical or unethical.

After completion of the required clinical testing, an NDA or BLA submission is prepared and submitted to the FDA. FDA approval of the submission is required before marketing of the product in the United States may begin. The submission must include the results of all preclinical, clinical and other testing and a compilation of data relating to the product's pharmacology, chemistry, manufacture and controls. The cost of preparing and submitting a submission is substantial. The submission of most NDAs and BLAs is additionally subject to a substantial application user fee, and the sponsor of an approved NDA or BLA is also subject to an annual program user fee. These fees typically are increased annually.

The FDA has 60 days from its receipt of an NDA or BLA to determine whether the application will be accepted for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review. Once the submission is accepted for filing, the FDA begins an in-depth review. The FDA has agreed to certain performance goals in the review of NDAs and BLAs. Most such applications for standard review drug products are reviewed within 10 months of filing; most applications for priority review drugs are reviewed within six months of filing. Priority review can be applied to drugs to treat serious conditions that the FDA determines offer significant improvement in safety or effectiveness. The review process for both standard and priority review may be extended by the FDA for three additional months to consider certain late-submitted information, or information intended to clarify information already provided in the submission.

The FDA also may refer applications for novel drug products, or drug products that present difficult questions of safety or efficacy, to an advisory committee—typically a panel that includes clinicians and other experts—for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations.

Before approving an NDA or BLA, the FDA typically will inspect one or more clinical sites to assure compliance with GCPs. Additionally, the FDA may inspect the facility or the facilities at which the drug is manufactured. The FDA will not approve the product unless compliance with cGMPs is satisfactory and the submission contains data that provide substantial evidence that the drug is safe and effective in the indication studied.

After the FDA evaluates the NDA or BLA and the manufacturing facilities, it issues either an approval letter or a complete response letter. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing, or information, in order for the FDA to reconsider the application. If, or when, those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA or BLA, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included.

An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. As a condition of approval, the FDA may require a REMS to ensure that the benefits of the drug outweigh the potential risks. REMS can include a medication guide, a communication plan for healthcare professionals and elements to assure safe use, such as special training and certification requirements for individuals who prescribe or dispense the drug, requirements that patients enroll in a registry and other measures that the FDA deems necessary to assure the safe use of the drug. The requirement for REMS can materially affect the potential market and profitability of the drug. Moreover, product approval may require substantial post-approval testing and surveillance to monitor the drug's safety or efficacy. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing.

Changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, require submission and FDA approval of a new NDA or NDA supplement before the change can be implemented. An NDA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing NDA supplements as it does in reviewing NDAs. Such supplements typically are reviewed within 10 months of receipt.

Orphan Drugs

Under the Orphan Drug Act, the FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition—generally a disease or condition that affects fewer than 200,000 individuals in the U.S. Orphan drug designation must be requested before submitting an NDA. After the FDA confers orphan drug status, the generic identity of the drug and its potential orphan indication are disclosed publicly by the FDA. Orphan drug designation in and of itself does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. The first NDA applicant to receive FDA approval for a particular active ingredient to treat a particular indication with FDA orphan drug designation is entitled to a seven-year exclusive marketing period in the U.S. for that product, for that indication. During the seven-year exclusivity period, the FDA may not approve any other applications to market the same drug for the same disease, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity. Orphan drug exclusivity does not prevent FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition. Prior to FDA approval, orphan designation provides incentives for sponsors including tax credits for clinical research expenses, the opportunity to obtain government grant funding to support clinical research, and an exemption from FDA user fees.

Expedited Development and Review Programs

A sponsor may seek to develop and obtain approval of its products under programs designed to accelerate the development, FDA review and approval of new products that meet certain criteria.

Fast track is a process designed by the FDA to facilitate the development of drugs to treat serious conditions through expediting their review. The purpose is to get important new drugs to patients earlier. Fast Track addresses a broad range of serious conditions. Determining whether a condition is serious is a matter of judgment, but generally is based on whether the drug will have an impact on such factors as survival, day-to-day functioning, or the likelihood that the condition, if left untreated, will progress from a less severe condition to a more serious one.

A drug that receives Fast Track designation is eligible for some or all of the following:

- more frequent meetings with FDA to discuss the drug's development plan and ensure collection of appropriate data needed to support drug approval;
- more frequent written communication from FDA about such things as the design of the proposed clinical trials and use of biomarkers;
- eligibility for Accelerated Approval and Priority Review, if relevant criteria are met; and
- Rolling Review, which means that a drug company can submit completed sections of its NDA or BLA for review by FDA, rather than waiting until every section is completed before the entire application can be reviewed. NDA or BLA review usually does not begin until the drug company has submitted the entire application to the FDA.

Once a drug receives Fast Track designation, early and frequent communication between the FDA and a drug company is encouraged throughout the entire drug development and review process. The frequency of communication assures that questions and issues are resolved quickly, often leading to earlier drug approval and access by patients.

Breakthrough therapy designation is available if the product is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over currently approved therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. If the FDA designates a breakthrough therapy, it may take actions appropriate to expedite the development and review of the application, which may include holding meetings with the sponsor and the review team throughout the development of the therapy; providing timely advice to, and interactive communication with, the sponsor regarding the development of the product candidate to ensure that the development program to gather the nonclinical and clinical data necessary for approval is as efficient as practicable; involving senior managers and experienced review staff, as appropriate, in a collaborative, cross- disciplinary review; assigning a cross-disciplinary project lead for the FDA review team to facilitate an efficient review of the development program and to serve as a scientific liaison between the review team and the sponsor; and considering alternative clinical trial designs when scientifically appropriate, which may result in smaller trials or more efficient trials that require less time to complete and may minimize the number of patients exposed to a potentially less efficacious treatment. Breakthrough therapy designation comes with the benefits of Fast Track designation, which means that the sponsor may submit sections of the NDA or BLA for review on a rolling basis.

Accelerated Approval

Under the FDA’s accelerated approval regulations, FDA may approve a drug or biologic for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments.

In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions, or survives. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A drug candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint with regular reporting to FDA on the status of such trials. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, will allow FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by FDA.

The Hatch-Waxman Amendments: Orange Book Listing

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent whose claims cover the applicant’s product or its use for the relevant application. Upon approval of a drug, each of the patents listed in the application for the drug is then published in the FDA’s Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential generic competitors in support of approval of an ANDA. An ANDA provides for marketing of a drug product that has the same active ingredients in the same strengths and dosage form as the listed drug and has been shown through bioequivalence testing to be therapeutically equivalent to the listed drug. Other than the requirement for bioequivalence testing, ANDA applicants are not required to conduct, or submit results of, preclinical or clinical tests to prove the safety or effectiveness of their drug product. Drugs approved in this way are commonly

referred to as “generic equivalents” to the listed drug, and can often be substituted by pharmacists under prescriptions written for the original listed drug.

The ANDA applicant is required to certify to the FDA concerning all patents listed for the approved product in the FDA’s Orange Book. Specifically, the applicant must certify that: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or (iv) the listed patent is invalid or will not be infringed by the new product. The ANDA applicant may also 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. If the applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

A certification that the new product will not infringe the already approved product’s listed patents, or that such patents are invalid, is called a Paragraph IV certification. If the ANDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. Unless the submission of the ANDA pre-dates the listing of the patent in the Orange Book, the filing of a patent infringement lawsuit within 45 days of the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit, or a decision in the infringement case that is favorable to the ANDA applicant.

The ANDA application also will not be approved until any applicable non-patent exclusivity listed in the Orange Book for the referenced product has expired.

Post-Approval Requirements

Once an NDA is approved, a product will be subject to certain post-approval requirements. For instance, the FDA closely regulates the post-approval marketing and promotion of drugs, including standards and regulations for direct-to-consumer advertising, off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the internet and social media. Drugs may be marketed only for the approved indications and in accordance with the provisions of the approved labeling.

Adverse event reporting and submission of periodic reports is required following FDA approval of an NDA. The FDA also may require post-marketing testing, known as Phase 4 testing, REMS, surveillance to monitor the effects of an approved product, or restrictions on the distribution or use of the product. In addition, quality-control, drug manufacturing, packaging and labeling procedures must continue to conform to cGMPs after approval. Drug manufacturers and certain of their subcontractors are required to register their establishments with the FDA and certain state agencies. Registration with the FDA subjects entities to periodic unannounced inspections by the FDA, during which the agency inspects manufacturing facilities to assess compliance with cGMPs. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality-control to maintain compliance with cGMPs. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market and/or product recalls;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of product approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

Pricing and Reimbursement

A portion of our product demand for our approved therapies comes from patients covered under Medicaid, Medicare and other federal and state government-related programs such as TRICARE and the Department of Veterans Affairs, or the VA. As required by Federal regulations, we provide rebates and discounts in connection with these programs.

Our commercial success depends in significant part on the extent to which coverage and adequate reimbursement for these products will be available from third-party payers, including government health administration authorities, private health insurers and other organizations. Third-party payers determine which medications they will cover and establish reimbursement levels. Even if a third-party payer covers a particular product, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. Patients who are prescribed medications for the treatment of their conditions, and their prescribing physicians, generally rely on third-party payers to reimburse all or part of the costs associated with prescription therapies. Patients are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover all or a significant portion of the cost of our products. Therefore, coverage and adequate reimbursement is critical to product

acceptance. 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.

Government authorities and other third-party payers have and are developing methods of controlling healthcare costs, such as by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payers are requiring that therapeutic companies provide them with predetermined discounts/rebates from list prices as a condition of coverage, are using restrictive formularies and preferred therapy lists to leverage greater discounts in competitive classes, and are challenging the prices charged for medical products. Third party payers also are carefully evaluating the medical necessity and cost-effectiveness of medical products and services, in addition to a product's safety and efficacy, which may require us to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products. Further, no uniform policy requirement for coverage and reimbursement for therapies exists among third-party payers in the United States. Therefore, coverage and reimbursement for therapies can differ significantly from payer to payer. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payer separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

In addition, it is possible that future legislation in the U.S. and other jurisdictions could be enacted which could potentially influence the coverage and reimbursement rates for our products and also could further impact the levels of discounts and rebates paid to federal and state government entities, as well as commercial payers. Any legislation that influences these areas could impact, in a significant way, our ability to generate revenues from sales of products that, if successfully developed, we bring to market.

There have been a number of enacted or proposed legislative and regulatory changes affecting the healthcare system and pharmaceutical industry that could affect our commercial success. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, (collectively, the “PPACA”) was signed into law, which intended to, among other things, broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against healthcare fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes, rebates and fees on pharmaceutical and medical device manufacturers and impose additional health policy reforms. There have been executive, judicial and Congressional challenges and amendments to certain aspects of the PPACA. For example, on August 16, 2022, the Inflation Reduction Act of 2022 (“IRA”) was signed into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in PPACA marketplaces through plan year 2025. The IRA also eliminates the "donut hole" under the Medicare Part D program beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost and creating a new manufacturer discount program. The IRA, discussed in more detail below, also established new pricing pressures, including government price setting for certain therapies and mandatory rebates on price increases over inflation.

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, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee on Deficit Reduction did not achieve a targeted deficit reduction of at least $1.2 trillion for fiscal years 2012 through 2021, triggering the legislation’s automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect beginning on April 1, 2013 and, due to subsequent legislative amendments, will stay in effect until 2032, unless additional Congressional action is taken.

There has been increasing legislative and enforcement interest in the United States with respect to therapy pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and numerous proposed and enacted legislation at both the state and federal levels designed to, among other things, bring more transparency to therapy pricing, reduce the cost of prescription therapies under Medicare, expedite generic competition, review the relationship between pricing and manufacturer patient programs, institute therapy re-importation, and reform government program reimbursement methodologies for therapies. For example, the IRA, among other things (i) directs the U.S. Department of Health and Human Services (HHS) to negotiate the price of certain high-expenditure, single-source drugs that have been on the market for at least 7 years covered under Medicare (the “Medicare Drug Price Negotiation Program”), and (ii) imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation. These provisions began to take effect progressively in fiscal year 2023. On August 15, 2024, HHS announced the agreed-upon price of the first ten drugs that were subject to price negotiations, although the Medicare Drug Price Negotiation Program is currently subject to legal challenges. On January 17, 2025, HHS selected fifteen additional drugs covered under Part D for price negotiation in 2025. Each year thereafter more Part B and Part D products will become subject to the Medicare Drug Price Negotiation Program. Further, on December 7, 2023, an initiative to control the price of prescription drugs through the use of march-in rights under the Bayh-Dole Act was announced. 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.

We expect that the PPACA, the IRA, as well as other federal and state healthcare reform measures that have been and may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any of our products, and could seriously affect our future revenues. In addition, it is possible that future legislation in the United States and other jurisdictions could be enacted which could potentially impact the reimbursement rates for the products we are developing and may develop in the future and also could further impact the levels of discounts and rebates paid to federal and state government entities. Any legislation that impacts these areas could impact, in a significant way, our ability to generate revenues from sales of products that, if successfully developed, we bring to market.

Health Care Regulatory Laws

In addition to FDA marketing restrictions and regulation of pharmaceutical products, several other types of state and federal laws have been applied to restrict and regulate certain business practices in the pharmaceutical industry in recent years. These laws include, without limitation, anti-kickback statutes and false claims laws, data privacy and security laws, and transparency laws regarding payments or other items of value provided to healthcare providers.

The federal healthcare 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 healthcare item or service reimbursable under Medicare, Medicaid, or other federally financed healthcare programs. This statute has been interpreted broadly to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers, and formulary managers, among others, on the other. Although there are a number of statutory exceptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exceptions and safe harbors are drawn narrowly, and practices that involve remuneration that may induce prescribing, purchases, or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the anti-kickback statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all its facts and circumstances. Several courts have interpreted the statute's intent requirement to mean that if any one purpose of an arrangement involving remuneration is to induce referrals of federal healthcare covered business, the federal anti-kickback statute has been violated. Additionally, the PPACA amended the federal anti-kickback statute to a stricter standard 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. In addition, the PPACA codified case law that a claim including items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the federal civil False Claims Act.

Federal false claims laws, including the civil 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 have a false claim paid. This includes claims made to programs where the federal government reimburses, such as Medicaid, as well as programs where the federal government is a direct purchaser, such as when it purchases off the Federal Supply Schedule. The False Claims Act contains qui tam provisions, which allow a private individual, or relator, to bring a civil action on behalf of the federal government alleging that the defendant submitted a false claim to the federal government, and to share in any monetary recovery. In recent years, the number of suits brought by private individuals has increased dramatically. For example, pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly inflating drug prices they report to pricing services, which in turn were used by the government to set Medicare and Medicaid reimbursement rates, and for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. In addition, certain marketing practices, including off-label promotion, may also violate federal false claims laws.

Also, many states have similar fraud and abuse statutes or regulations, including state anti-kickback and false claims laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payer.

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 you 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.

In addition, we are subject to data privacy and security regulation by both the federal government and the states in which we conduct our business. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), and their respective implementing regulations, imposes specified requirements relating to the privacy, security and transmission of individually identifiable health information. Among other things, HITECH, through its implementing regulations, makes certain of HIPAA's privacy and security standards directly applicable to business associates, defined as a person or organization, other than a member of a covered entity's workforce, that creates, receives, maintains or transmits protected health information for or on behalf of a covered entity for a function or activity regulated by HIPAA and their covered subcontractors.

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 for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to annually report information related to certain payments or other transfers of value made or distributed to physicians and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, physicians (defined to include to include doctors, dentists, optometrists, podiatrists and chiropractors), other healthcare professionals (such as physician assistants and nurse practitioners), and teaching hospitals and to report annually certain ownership and investment interests held by physicians and their immediate family members.

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. Additionally, states that have not implemented these types of regulations are considering similar proposals. Compliance with these laws is difficult and time consuming, and companies that do not comply with these state laws face civil penalties.

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.

Foreign Regulation

In addition to regulations in the United States, we are subject to a variety of foreign regulations governing clinical trials and commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product, we must obtain approval by the comparable regulatory authorities of foreign countries before we can commence clinical trials and approval of foreign countries or economic areas, such as the European Union, before we may market products in those countries or areas. The approval process and requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly from place to place, and the time may be longer or shorter than that required for FDA approval.

Clinical Trials and Marketing Authorization in the European Union

In the EU, clinical trials are governed by the Clinical Trials Regulation (EU) No 536/2014, or CTR, which entered into application on January 31, 2022 repealing and replacing the former Clinical Trials Directive 2001/20, or CTD. The CTR is intended to harmonize and streamline clinical trial authorizations, simplify adverse-event reporting procedures, improve the supervision of clinical trials and increase transparency. Specifically, the Regulation, which is directly applicable in all EU Member States, introduces a streamlined application procedure through a single-entry point, the "EU portal", the Clinical Trials Information System, or CTIS; a single set of documents to be prepared and submitted for the application; as well as simplified reporting procedures for clinical trial sponsors. A harmonized procedure for the assessment of applications for clinical trials has been introduced and is divided into two parts. Part I assessment is led by the competent authorities of a reference Member State selected by the trial sponsor and relates to clinical trial aspects that are considered to be scientifically harmonized across EU Member States. This assessment is then submitted to the competent authorities of all concerned Member States in which the trial is to be conducted for their review. Part II is assessed separately by the competent authorities and Ethics Committees in each concerned EU Member State. Individual EU Member States retain the power to authorize the conduct of clinical trials on their territory. The CTR foresaw a three-year transition period that ended on January 31, 2025. Since this date, all new or ongoing trials are subject to the provisions of the CTR.

In all cases, clinical trials must be conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki. Medicines used in clinical trials must be manufactured in accordance with the guidelines on cGMP and in a GMP licensed facility, which can be subject to GMP inspections.

Under European Union regulatory systems, a company may not market a medicinal product without a marketing authorization.

In the EU, medicinal products can only be commercialized after a related marketing authorization ("MA"), has been granted. To obtain an MA for a product in the EU, an applicant must submit a Marketing Authorization Application ("MAA"), either under a centralized procedure administered by the EMA or one of the procedures administered by the competent national authorities of EU Member States: (i) the national procedure, (ii) the mutual recognition procedure; or (iii) the decentralized procedure. The submission strategy for a given product will depend on the nature of the product, the target indication(s), the history of the product and the marketing plan. The centralized procedure provides for the grant of a single MA by the European Commission that is valid throughout the European Economic Area (which is comprised of the 27 EU Member States plus Norway, Iceland and Liechtenstein). The centralized procedure is compulsory for certain medicinal products which are produced by biotechnology processes, advanced therapy medicinal products, products which are designated as orphan medicinal products and products with a new active substance indicated for the treatment of HIV/AIDS, cancer, neurodegenerative diseases, diabetes, auto-immune and other immune dysfunctions and viral diseases. For products with a new active substance indicated for the treatment of other diseases and products that are highly innovative or for which a centralized process is in the interest of patients, authorization through the centralized procedure is optional on related approval.

Under the centralized procedure, the EMA's Committee for Human Medicinal Products ("CHMP") conducts the initial assessment of a product. Under the centralized procedure, the maximum timeframe for the evaluation of an MAA by the EMA is 210 days. This excludes so-called clock stops, during which additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP. At the end of the review period, the CHMP provides an opinion to the European Commission. If this opinion is favorable, the Commission may then adopt a decision to grant a marketing authorization. In exceptional cases, the CHMP might perform an accelerated review of an MAA in no more than 150 days, excluding clock stops. This is usually when the product targets an unmet medical need and is expected to be of major interest from the point of view of public health and, in particular, from the viewpoint of therapeutic innovation. The CHMP can, however, revert to the standard time limit for the centralized procedure if it considers that it is no longer appropriate to conduct an accelerated assessment.

Unlike the centralized authorization procedure, the decentralized MA procedure requires a separate application to, and leads to separate approval by, the competent authorities of each EU Member State in which the product is to be marketed. This application is identical to the application that would be submitted to the EMA for authorization through the centralized procedure. The reference EU Member State prepares a draft assessment and drafts of the related materials within 120 days after receipt of a valid application. The resulting assessment report is submitted to the concerned EU Member States who, within 90 days of receipt, must decide whether to approve the assessment report and related materials. If a concerned EU Member State cannot approve the assessment report and related materials due to concerns relating to a potential serious risk to public health, disputed elements may be referred to the Heads of Medicines Agencies' Coordination Group for Mutual Recognition and Decentralised Procedures – Human, or CMDh, for review. The subsequent decision of the European Commission is binding on all EU Member States.

The mutual recognition procedure allows companies that have a medicinal product already authorized in one EU Member State to apply for this authorization to be recognized by the competent authorities in other EU Member States. Like the decentralized procedure, the mutual recognition procedure is based on the acceptance by the competent authorities of the EU Member States of the MA of a medicinal product by the competent authorities of other EU Member States. The holder of a national MA may submit an application to the competent authority of an EU Member State requesting that this authority recognize the MA delivered by the competent authority of another EU Member State.

An MA has, in principle, an initial validity of five years. The MA may be renewed after five years on the basis of a re-evaluation of the risk-benefit balance by the EMA or by the competent authority of the EU Member State in which the original MA was granted. To support the application, the MA holder must provide the EMA or the competent authority with a consolidated version of the Common Technical Document providing up-to-date data concerning the quality, safety and efficacy of the product, including all variations introduced since the MA was granted, at least nine months before the MA ceases to be valid. The European Commission or the competent authorities of the EU Member States may decide on justified grounds relating to pharmacovigilance, to proceed with one further five-year renewal period for the MA. Once subsequently definitively renewed, the MA shall be valid for an unlimited period. Any authorization which is not followed by the actual placing of the medicinal product on the EU market (for a centralized MA) or on the market of the authorizing EU Member State within three years after authorization ceases to be valid (the so-called sunset clause).

In the EU, a "conditional" MA may be granted in cases where all the required safety and efficacy data are not yet available. The European Commission may grant a conditional MA for a medicinal product if it is demonstrated that all of the following criteria are met: (i) the benefit-risk balance of the medicinal product is positive; (ii) it is likely that the applicant will be able to provide comprehensive data post-authorization; (iii) the medicinal product fulfils an unmet medical need; and (iv) the benefit of the immediate availability to patients of the medicinal product is greater than the risk inherent in the fact that additional data are still required. The conditional MA is subject to conditions to be fulfilled for generating the missing data or ensuring increased safety measures. It is valid for one year and must be renewed annually until all related conditions have been fulfilled. Once any pending studies are provided, the conditional MA can be converted into a traditional MA. However, if the conditions are not fulfilled within the timeframe set by the EMA and approved by the European Commission, the MA will cease to be renewed.

An MA may also be granted "under exceptional circumstances" where the applicant can show that it is unable to provide comprehensive data on efficacy and safety under normal conditions of use even after the product has been authorized and subject to specific procedures being introduced. These circumstances may arise in particular when the intended indications are very rare and, in the state of scientific knowledge at that time, it is not

possible to provide comprehensive information, or when generating data may be contrary to generally accepted ethical principles. Like a conditional MA, an MA granted in exceptional circumstances is reserved to medicinal products intended to be authorized for treatment of rare diseases or unmet medical needs for which the applicant does not hold a complete data set that is required for the grant of a standard MA. However, unlike the conditional MA, an applicant for authorization in exceptional circumstances is not subsequently required to provide the missing data. Although the MA "under exceptional circumstances" is granted definitively, the risk-benefit balance of the medicinal product is reviewed annually, and the MA will be withdrawn if the risk-benefit ratio is no longer favorable.

Pediatric Development in the European Union

In the EU, Regulation (EC) No 1901/2006 provides that all MAAs for new medicinal products have to include the results of trials conducted in the pediatric population, in compliance with a pediatric investigation plan, or PIP, agreed with the EMA's Pediatric Committee, or PDCO. The PIP sets out the timing and measures proposed to generate data to support a pediatric indication of the medicinal product for which MA is being sought. The PDCO can grant a deferral of the obligation to implement some or all of the measures provided in the PIP until there are sufficient data to demonstrate the efficacy and safety of the product in adults. Further, the obligation to provide pediatric clinical trial data can be waived by the PDCO when these data are not needed or appropriate because the product is likely to be ineffective or unsafe in children, the disease or condition for which the product is intended occurs only in adult populations, or when the product does not represent a significant therapeutic benefit over existing treatments for pediatric patients. Once the MA is obtained in all EU Member States and study results are included in the product information, even when negative, the product is eligible for a six-month extension to the Supplementary Protection Certificate, or SPC, if any is in effect at the time of authorization or, in the case of orphan medicinal products, a two-year extension of orphan market exclusivity.

An innovator company enjoys a period of "data exclusivity" during which their pre-clinical and clinical trials data may not be referenced in the regulatory filings of another company (typically a generic company) for the same drug substance.

Upon receiving an MA, innovative medicinal products are generally entitled to receive eight years of data exclusivity and 10 years of market exclusivity. Data exclusivity, if granted, prevents regulatory authorities in the EU from referencing the innovator's data to assess a generic application or biosimilar application for eight years from the date of authorization of the innovative product, after which a generic or biosimilar MAA can be submitted, and the innovator's data may be referenced. The market exclusivity period prevents a successful generic or biosimilar applicant from commercializing its product in the EU until 10 years have elapsed from the initial MA of the reference product in the EU. The overall ten-year period may, occasionally, be extended for a further year to a maximum of 11 years if, during the first eight years of those ten years, the MA holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. However, there is no guarantee that a product will be considered by the EU's regulatory authorities to be a new chemical/biological entity, and products may not qualify for data exclusivity.

Orphan Designation in the European Union

In the EU, Regulation (EC) No. 141/2000, as implemented by Regulation (EC) No. 847/2000 provides that a medicinal product can be designated as an orphan medicinal product by the European Commission if its sponsor can establish that: (i) the product is intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions; (ii) either (a) such conditions affect not more than five in 10,000 persons in the EU when the application is made, or (b) the product without the benefits derived from orphan status, would not generate sufficient return in the EU to justify the necessary investment in developing the medicinal product; and (iii) there exists no satisfactory authorized method of diagnosis, prevention, or treatment of the condition that has been authorized in the EU, or even if such method exists, the product will be of significant benefit to those affected by that condition.

Regulation (EC) No 847/2000 sets out further provisions for implementation of the criteria for designation of a medicinal product as an orphan medicinal product. An application for the designation of a medicinal product as an orphan medicinal product must be submitted at any stage of development of the medicinal product but before filing of an MAA. An MA for an orphan medicinal product may only include indications designated as orphan. For non-orphan indications treated with the same active pharmaceutical ingredient, a separate marketing authorization has to be sought.

Orphan medicinal product designation entitles an applicant to incentives such fee reductions or fee waivers, protocol assistance, and access to the centralized marketing authorization procedure. Upon grant of a marketing authorization, orphan medicinal products are entitled to a ten-year period of market exclusivity for the approved therapeutic indication, which means that the EMA cannot accept another marketing authorization application or accept an application to extend for a similar product and the European Commission cannot grant a marketing authorization for the same indication for a period of ten years. The period of market exclusivity is extended by two years for orphan medicinal products that have also complied with an agreed PIP. No extension to any supplementary protection certificate can be granted on the basis of pediatric studies for orphan indications. Orphan medicinal product designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

The period of market exclusivity 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, an MA may be granted to a similar medicinal product with the same

orphan indication during the 10 year period if: (i) if the applicant consents to a second original orphan medicinal product application, (ii) if the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities; or (iii) if 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.

Post-authorization Requirements

Where an MA is granted in relation to a medicinal product in the EU, the holder of the MA is required to comply with a range of regulatory requirements applicable to the manufacturing, marketing, promotion and sale of medicinal products. Similar to the United States, both MA holders and manufacturers of medicinal products are subject to comprehensive regulatory oversight by the EMA, the European Commission and/or the competent regulatory authorities of the individual EU Member States. The holder of an MA must establish and maintain a pharmacovigilance system and appoint an individual qualified person for pharmacovigilance who is responsible for oversight of that system. Key obligations include expedited reporting of suspected serious adverse reactions and submission of periodic safety update reports, or PSURs.

All new MAAs must include a risk management plan, or RMP, describing the risk management system that the company will put in place and documenting measures to prevent or minimize the risks associated with the product. The regulatory authorities may also impose specific obligations as a condition of the MA. Such risk- minimization measures or post-authorization obligations may include additional safety monitoring, more frequent submission of PSURs or the conduct of additional clinical trials or post-authorization safety studies.

In the EU, the advertising and promotion of medicinal products are subject to both EU and EU Member States' laws governing promotion of medicinal products, interactions with physicians and other healthcare professionals, misleading and comparative advertising and unfair commercial practices. General requirements for advertising and promotion of medicinal products, such as direct-to-consumer advertising of prescription medicinal products are established in EU law. However, the details are governed by regulations in individual EU Member States and can differ from one country to another. For example, applicable laws require that promotional materials and advertising in relation to medicinal products comply with the product's Summary of Product Characteristics, or SmPC, which may require approval by the competent national authorities in connection with an MA. The SmPC is the document that provides information to physicians concerning the safe and effective use of the product. Promotional activity that does not comply with the SmPC is considered off-label and is prohibited in the EU.

Other Laws and Regulatory Processes

We are subject to a variety of financial disclosure and securities trading regulations as a public company in the United States, including laws relating to the oversight activities of the Securities and Exchange Commission ("SEC"), and Nasdaq rules under which our stock is listed. In addition, the Financial Accounting Standards Board ("FASB"), the SEC, and other bodies that have jurisdiction over the form and content of our accounts, our financial statements and other public disclosures are constantly considering and interpreting proposals and existing pronouncements designed to ensure that companies best display relevant and transparent information relating to their respective businesses.

Our present and future business has been and will continue to be subject to various other laws and regulations. Various laws, regulations and recommendations relating to safe working conditions, laboratory practices, the experimental use of animals, and the purchase, storage, movement, import and export and use and disposal of hazardous or potentially hazardous substances used in connection with our research work are or may be applicable to our activities. Certain agreements entered into by us involving exclusive license rights or acquisitions may be subject to national antitrust regulatory control, the effect of which cannot be predicted. The extent of government regulation which might result from future legislation or administrative action also cannot be predicted with accuracy.

Employees and Human Capital Management

As of January 31, 2025, we had 385 full-time employees, with most of those based in the United States and a small number outside of the United States. We consider the intellectual capital and well-being of our employees to be an important driver of our business and key to our future success. The biopharmaceutical industry is very competitive and we believe that our future success largely depends upon our continued ability to attract, develop and retain highly skilled employees as our operations expand, as well as our continued focus on our culture and patient centricity. Our workforce primarily consists of college-educated workers with experience in the biopharmaceutical industry, many of whom have advanced degrees. Our employees primarily focus on our drug development and commercialization efforts, including sales and general and administrative operational support of those functions. Currently, we rely on third-party contract manufacturers and conduct our discovery research efforts via collaborations and/or contracted third-party engagements. None of our employees in the United States are represented by a labor union or covered by collective bargaining agreements. We consider our current employee relations to be good.

We are committed to cultivating a workplace where individuals of all backgrounds feel valued, supported, and empowered to reach their fullest potential. We desire to have our organization be representative of the communities we serve, while fostering a culture of inclusivity and a strong sense of belonging. We know from our experience over more than a decade that rare diseases affect individuals across all demographics, and there are clear disparities in diagnosis, access to care, and support that remain significant barriers for many. By fostering inclusivity within our workforce, we are better able to incorporate diverse viewpoints, which we believe positively influences our ability to achieve our objectives and ultimately impact patient outcomes – this is key to operating within our mission, and successfully helping the patients we aim to serve. In line with embracing this

inclusivity, we support a number of initiatives that directly impact our human capital, workforce and community, such as efforts focused on professional development, cultural awareness, and engagement. Additionally, we provide information about these efforts on our website, though the information on our website is not incorporated in this annual report on Form 10-K.

We strive to provide compensation, benefits and support services that help meet the varying needs of our employees. In the United States, our total compensation package includes competitive pay, including opportunities for performance-based bonuses; comprehensive healthcare benefits; paid time off and paid holidays, and the opportunity for equity ownership through our equity incentive plan and our employee stock purchase plan, as well as a suite of wellness focused offerings. We also sponsor a 401(k) plan that includes a discretionary matching contribution. A similar package of benefits is provided to our employees outside of the United States, subject to regional differences.

By focusing on employee retention, engagement and development opportunities, we believe we also improve our ability to support our clinical trials, our pipeline, our business and our operations. We value the growth and professional development of our employees. We do this through clear organizational, team, and individual goal setting, performance measurement, customized professional development, and employee training and development sessions on various topics. Our success also depends on our ability to respond to the needs of employees. We do this by listening to our employees through many avenues, including formal engagement initiatives, such as surveys, as well as informal listening sessions hosted by senior leaders, including our CEO and our human resources department. The response rate for our latest employee engagement survey, which was conducted in November 2024, was 84%. During 2024 we continued to focus on ways to maximize a people-centered, inclusive, and recognition-based company culture, building on initiatives that have been implemented over the last several years. Also during 2024, we continued to enhance our hybrid workforce program that provides a variety of virtual and in-person collaboration opportunities. In 2024, we solicited feedback on ways to enhance employees' experience, with a focus on engagement and collaboration, and expect to implement new offerings in 2025. As we continue to navigate through new ways of working, we take pride in the role we play in helping to ensure our employees are as productive and engaged as possible and their physical and emotional health, well-being and safety remains a key priority. We are committed to providing a safe and healthy working environment for our employees and to avoiding adverse impact to the environment and the communities in which we do business.

Available Information

We were incorporated in the state of Delaware in February 2011. Our website address is travere.com. We post links on our website to the following filings as soon as reasonably practicable after they are electronically filed with or furnished to the SEC: annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K, proxy statements, and any amendments to those reports filed or furnished pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934, as amended. All such filings are available through our website free of charge. The SEC also maintains an internet site at www.sec.gov that contains reports, proxy and information statements, and other information regarding issuers that file electronically with the SEC.

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.

Risks Related to the Commercialization of Our Products

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

Our ability to generate significant product revenues and to achieve commercial success in the near-term will depend almost entirely on our ability to successfully commercialize our products in the United States, including FILSPARI (sparsentan) to slow kidney function decline in adults with primary IgAN who are at risk of disease progression, which was granted full approval by the FDA in September 2024. FILSPARI had previously been granted accelerated approval in February 2023 based on the surrogate marker of proteinuria. As a product for a rare disease that had no previously-approved non-immunosuppressive treatment, the successful launch and commercialization of FILSPARI is subject to many risks. There are numerous examples of unsuccessful product launches and failures to meet high expectations of market potential, including by pharmaceutical companies with more experience and resources than we have. While we have established our commercial team and U.S. sales force, we will need to continue to train and further develop the team in order to successfully coordinate the ongoing launch and commercialization of FILSPARI in the United States. There are many factors that could cause the launch and commercialization of FILSPARI to be unsuccessful, including a number of factors that are outside our control. Because no non-immunosuppressive product had previously been approved by the FDA for the treatment of

IgAN, it is difficult to estimate FILSPARI's market potential or the time it will take to increase patient and physician awareness of FILSPARI and change current treatment paradigms.

The commercial success of FILSPARI depends on the extent to which patients and physicians accept and adopt FILSPARI for IgAN patients. For example, if the addressable patient population suffering from primary IgAN is smaller than we estimate, if it proves difficult to educate physicians as to the availability and potential benefits of FILSPARI, or if physicians are unwilling to prescribe or patients are unwilling to take FILSPARI, the commercial potential of FILSPARI will be limited. We also do not know how physicians, patients and payers will respond to the pricing of FILSPARI, the updated, full approval label, clinical practice guidelines and any future changes thereto, 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 such as Cholbam, are subject to immediate competition from compounded and generic entrants, as the ANDA and/or NDA for these drug products have no remaining or current patent or non-patent exclusivity. In April 2021, a generic option for the 100mg version of the original formulation of Thiola (tiopronin tablets) was approved by the FDA and an additional generic option of the original formulation of Thiola (tiopronin tablets) was approved in June 2022 and during the year ended December 31, 2022, we experienced a decrease in total net product revenues compared to the year ended December 31, 2021, which was due in part to competition from generic tiopronin tablets (100mg version of the original formulation). Additional generic versions of Thiola may be approved in the future. As of December 31, 2024, several generic options for the 100mg and 300mg versions of Thiola EC have been approved by the FDA and become available. Our future net product revenues from Thiola and/or Thiola EC may be materially impacted by competition from existing or additional generic versions of Thiola or Thiola EC.

In addition, there have been a number of recent regulatory and legislative initiatives designed to encourage generic competition for pharmaceutical products, including expedited review procedures for generic manufacturers, proposed "skinny label" legislation, and incentives designed to spur generic competition of branded drugs. In particular, the FDA and the U.S. Federal Trade Commission ("FTC") have been focused on brand companies' denial of drug supply to potential generic competitors for testing. In December 2019, the CREATES Act was enacted, which provides a legislatively defined private right of action under which generic companies can bring suit against companies who refuse access to product for the bioequivalence testing needed to support approval of a generic product.

In 2020, we completed our response to a civil investigative demand from the FTC related to the marketing, sale, distribution and pricing of our products, including Thiola. While the investigation remains open, at this time the FTC has not indicated that it has additional questions for us and has not initiated any claim or proceeding against us relating to these matters.

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

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

The Drug Price Competition and Patent Term Restoration Act (commonly referred to as the "Hatch-Waxman Act") requires an ANDA applicant seeking FDA approval of its proposed generic product prior to the expiration of an Orange Book-listed patent (as defined below) to certify that the applicant believes that the patent is invalid, unenforceable and/or will not be infringed by the manufacture, use or sale of the drug for which the application has been submitted (a paragraph IV certification) and notify the NDA and patent holder of such certification (a paragraph IV notice). Upon

receipt of a paragraph IV notice, the Hatch-Waxman Act allows the patent holder, with proper basis, to bring an action for patent infringement against the ANDA filer, asking that the proposed generic product not be approved until after the patent expires. For ANDAs that are filed ("received") after the listing of the patent in the Orange Book, if the patent holder commences a lawsuit within 45 days from receipt of the paragraph IV notice, the Hatch-Waxman Act provides a 30-month stay during which time the FDA cannot finally approve the generic's application. If the litigation is resolved in favor of the ANDA applicant during the 30-month stay period, the stay is lifted and the FDA may finally approve the ANDA if it is otherwise ready for approval. For ANDAs that are filed ("received") before the listing of the patent in the Orange Book, the 30-month stay provision of the Hatch-Waxman Act does not apply. It also may be possible, depending on the approved label, for an ANDA applicant to elect to submit a section viii statement certifying that its proposed ANDA label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent.

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

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

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

Economic, social, and congressional pressure may result in individuals and government entities increasingly seeking to achieve cost savings through mechanisms that limit coverage or payment for our products. For example, state Medicaid programs are increasingly requesting manufacturers to pay supplemental rebates and are requiring prior authorization for use of drugs. Managed care organizations continue to seek price discounts and, in some cases, to impose restrictions on the coverage of particular drugs. Government efforts to reduce Medicaid expenses may lead to increased use of managed care organizations by Medicaid programs. This may result in managed care organizations influencing prescription decisions for a larger segment of the population and a corresponding constraint on prices and reimbursement for our products.

In addition, patients' access to employer sponsored insurance coverage may be negatively impacted by economic factors that result in increased rates of unemployment. To the extent patients taking our approved therapies become unemployed and experience a reduction to, or increased costs associated with, their insurance coverage, demand for our products could decline, which could have a material adverse effect on our sales and profitability, either as a result of decreased sales of our products and/or increased provision by us of free product to uninsured or commercially insured patients. The extent and duration of this potential impact on our business is currently unknown.

We are dependent on third parties to manufacture and distribute our products.

We have no manufacturing capabilities and rely on third-party manufacturers who are currently sole source suppliers for manufacturing of FILSPARI and Thiola. The facilities used by our third-party manufacturers must be approved by the FDA and comparable foreign regulatory authorities. Our dependence on third parties for the manufacture of our products may harm our profit margin on the sale of products and our ability to deliver products on a timely and competitive basis. Because we are ultimately responsible for ensuring that our API and finished products are manufactured in accordance with cGMP regulations and similar regulatory requirements outside the United States, it is critical that we maintain effective management practices and oversight with respect to our third-party manufacturers, including routine auditing. If our third-party manufacturers are unable to manufacture to specifications or in compliance with applicable regulatory requirements, our ability to commercialize our products will be adversely impacted and could affect our ability to gain market acceptance for our products and negatively impact our revenues.

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

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

substantially similar terms, distribution of FILSPARI and/or Thiola could become disrupted, resulting in lost revenues, provider dissatisfaction, and/or patient dissatisfaction.

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

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

Moreover, to obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost effectiveness of our product candidate to other available therapies. This Health Technology Assessment ("HTA") of medicinal products is becoming an increasingly common part of the pricing and reimbursement procedures in some EU Member States, including those representing the larger markets. The HTA process is the procedure to assess therapeutic, economic and societal impact of a given medicinal product in the national healthcare systems of the individual country. The outcome of an HTA will often influence the pricing and reimbursement status granted to these medicinal products by the competent authorities of individual EU Member States. The extent to which pricing and reimbursement decisions are influenced by the HTA of the specific medicinal product currently varies between EU Member States. In December 2021, Regulation No 2021/2282 on HTA amending Directive 2011/24/EU, was adopted in the EU. This Regulation, 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. In October 2024, we received an invoice from the government authority for approximately €5.6 million (approximately $6.2 million), which we paid while we continue to pursue an appeal of the decision and the amount paid. While we cannot predict the amount that we may ultimately need to repay following ongoing review and future potential appeal proceedings, from 2015 through 2020, the period during which we had sales of Kolbam in France, our aggregate revenues from sales of Kolbam in France attributable to all purchasers/payers were approximately $8 million. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels or subject to re-assessment and recoupment procedures, our prospects for generating revenue outside of the United States, if any, could be adversely affected and our business may suffer.

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

Regulatory authorities in some jurisdictions, including the United States and the EU, may designate drugs for relatively small patient populations as orphan drugs, providing eligibility for orphan drug exclusivity upon regulatory approval if certain jurisdictional-specific conditions are met. For example, FILSPARI has been granted orphan drug designation for the treatment of IgAN and has been awarded seven years of orphan drug exclusivity in the United States (running from the date of accelerated approval) to reduce proteinuria in adults with primary IgAN at risk of rapid disease progression, and has been granted a separate seven years of Orphan Drug Exclusivity in the U.S. (running from the date of full approval) to slow kidney function decline in adults with primary IgAN who are at risk for disease progression, excluding the use provided for in the aforementioned Orphan Drug Exclusivity granted in connection with the accelerated approval. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, that product is entitled to a period of marketing exclusivity, which precludes the applicable regulatory authority from approving another marketing application for the same drug for the same indication for that time period. The applicable period is seven years in the United States and ten years in the EU or, in the case of orphan drugs for which a pediatric investigation plan has been completed, 12 years. Even though we have been awarded orphan drug designation in the United States and the EU for

sparsentan for the treatment of IgAN and FSGS and for pegtibatinase for the treatment of HCU, we may not be able to maintain it in the EU and the orphan drug designation may not result in orphan drug exclusivity in the United States for FSGS or the EU if approved. For example, if a competitive product that contains the same active moiety and treats the same disease as our product is shown to be clinically superior to our product, any orphan drug exclusivity we have obtained will not block the approval of such competitive product and we may effectively lose orphan drug exclusivity. Similarly, if a competitive product that contains the same active moiety and treats the same disease as our product candidate is approved for orphan drug exclusivity before our product candidate, we may not be able to obtain approval for our product candidate until the expiration of the competitive product's orphan drug exclusivity unless our product candidate is shown to be clinically superior to the competitive product.

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

Government agencies promulgate regulations and guidelines directly applicable to us and to our products. However, professional societies, industry groups, practice management groups, insurance carriers, physicians, private foundations and other organizations involved in various diseases or conditions from time to time may also publish guidelines or recommendations to healthcare providers, administrators and payers, and patient communities. Recommendations by government agencies or those other groups/organizations may relate to such matters as clinical guidelines, usage and reimbursement of our products by government and private payers. Recommendations or guidelines that are followed by patients, healthcare providers and payers could impact the use of our products in positive or negative ways. In addition, recommendations or guidelines may not be followed by patients, healthcare providers or 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;
- regulators may require us to conduct studies of the long-term effects associated with the use of our product candidates;
- regulators, institutional review boards or ethics committees may not authorize us to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- the FDA or any non-United States regulatory authority may impose conditions on us regarding the scope or design of our clinical trials or may require us to resubmit our clinical trial protocols to institutional review boards or ethics committees for re-inspection due to changes in the regulatory environment;
- the number of patients required for our clinical trials may be larger than we anticipate or participants may drop out of our clinical trials at a higher rate than we anticipate;
- our third-party contractors or clinical investigators may fail to comply with regulatory requirements or fail to meet their contractual obligations to us in a timely manner;
- we might have to suspend, vary or terminate one or more of our clinical trials if we, regulators or institutional review boards or ethics committees determine that the participants are being exposed to unacceptable health risks;
- regulators, institutional review boards or ethics committees may require that we hold, suspend, vary or terminate clinical research for various reasons, including noncompliance with regulatory requirements;
- the cost of our clinical trials or the anticipated commercialization costs may be greater than we anticipate;

- the supply or quality of our product candidates or other materials necessary to conduct our clinical trials may be insufficient or inadequate, or more expensive than we originally anticipated, or we may not be able to reach agreements on acceptable terms with prospective suppliers or clinical research organizations; and

- the effects of our product candidates may not be the desired effects or may include undesirable side effects or the product candidates may have other unexpected characteristics.

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

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

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

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

- be delayed in obtaining, or may not be able to obtain, marketing approval for one or more of our product candidates;

- obtain approval for indications that are not as broad as intended or entirely different than those indications for which we sought approval; and

- have the product removed from the market after obtaining marketing approval.

For example, in our pivotal Phase 3 DUPLEX Study of sparsentan in FSGS, although we achieved the pre-specified interim FSGS partial remission of proteinuria endpoint after 36 weeks of treatment, the study did not achieve the primary efficacy eGFR slope endpoint over 108 weeks of treatment. While we have continued to engage with the FDA to explore a potential path forward for an sNDA, including through a recent Type C meeting, and while we intend to file an sNDA seeking traditional approval of FILSPARI for FSGS, there is no guarantee that the FDA will accept the sNDA for filing, will grant priority review of the sNDA or grant approval of FILSPARI for FSGS. In addition, a collaborative international effort referred to as the PARASOL project was initiated in late 2023 with a goal to define the quantitative relationships between short-term changes in biomarkers (proteinuria and GFR) and long-term outcomes in order to support the use of alternative proteinuria-based endpoints as a basis for accelerated and traditional approval. Even though representatives of regulatory agencies participated in the discussions, there is no guarantee that the outcome of those discussions will be reflected in any future formal determination by such regulatory agencies. There is no guarantee that the PARASOL group will achieve its intended goal, or that, even if it does, that sparsentan will be approved for FSGS.

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

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

Success in nonclinical testing and early clinical trials does not ensure that later clinical trials will be successful. For example, while we saw trends in favor of sparsentan in the two-year confirmatory endpoint analysis in the DUPLEX Study in FSGS, the positive eGFR results from the open-label portion of the DUET study of sparsentan in FSGS were not replicated in the Phase 3 clinical trial with statistical significance. Similarly, the positive nonclinical data we have seen from pegtibatinase being tested in a mouse model of homocystinuria and the positive topline results we reported in December 2021 and May 2023 from the ongoing Phase 1/2 clinical trial of pegtibatinase may not be replicated in future studies. We cannot assure that any current or future clinical trials of sparsentan or pegtibatinase will ultimately be successful. Before obtaining regulatory approval to conduct clinical trials of our product candidates, we must conduct extensive nonclinical tests to demonstrate the safety of our product candidates in animals. Nonclinical testing is expensive, difficult to design and implement, and can take many years to complete. In addition, during the clinical development

process, additional nonclinical toxicology studies are routinely conducted concurrently with the clinical development of a product candidate. If any of our product candidates show unexpected findings in concurrent toxicology studies, we could experience potentially significant delays in, or be required to abandon, development of that product candidate. A failure of one or more of our nonclinical studies can occur at any stage of testing.

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

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

In 2018 we initiated the Phase 3 DUPLEX Study 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 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. While we have continued to engage with the FDA to explore a potential path forward for an sNDA, including through a recent Type C meeting, and while we intend to file an sNDA seeking traditional approval of FILSPARI for FSGS, there is no guarantee that the FDA will accept the sNDA for filing, will grant priority review of the sNDA or grant approval of FILSPARI for FSGS. Similarly, there is no guarantee that our collaborator CSL Vifor will be able to establish a pathway to a potential submission of sparsentan for FSGS in Europe based on the results from the DUPLEX Study, that the EMA will support an application for sparsentan in FSGS, or that sparsentan will be approved for FSGS in Europe.

In December 2023, we initiated the pivotal Phase 3 HARMONY Study to support the potential approval of pegtibatinase for the treatment of classical HCU. The HARMONY Study is a global, randomized, multi-center, double-blind, placebo-controlled Phase 3 clinical trial designed to evaluate the efficacy and safety of pegtibatinase as a novel treatment to reduce total homocysteine (tHcy) levels. In September 2024, we announced a voluntary pause of enrollment in the HARMONY Study. The voluntary enrollment pause was enacted following our determination that the desired drug substance profile was not achieved in the recent scale-up process. We continue to 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 Study will be successful or that pegtibatinase will be approved for HCU in the future, on 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.

Further, others, including regulatory authorities, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure, and any information we determine not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular therapy, therapeutic candidate or our business. If the topline data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, our product candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

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

In September 2024, the FDA granted full approval of FILSPARI to slow kidney function decline in adults with primary IgAN who are at risk of disease progression. Any future regulatory approvals that sparsentan or any of our other product candidates receives may be subject to significant limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate.

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

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

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

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

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

Risks Related to our Products and Product Candidates

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

The success of our products is dependent upon achieving and maintaining market acceptance. Commercializing products is time consuming, expensive and unpredictable. There can be no assurance that we will be able to, either by ourselves or in collaboration with our partners or through our licensees, successfully commercialize new products or current products or gain market acceptance for such products. New product candidates that appear promising in development may fail to reach the market or may have only limited or no commercial success.

Further, the discovery of significant problems with a product similar to one of our products that implicate (or are perceived to implicate) an entire class of products could have an adverse effect on sales of the affected products. Accordingly, new data about our products, or products similar to our products, could negatively impact demand for our products due to real or perceived side effects or uncertainty regarding efficacy and, in some cases, could result in product withdrawal.

Our current products, including FILSPARI, and any product candidates that receive marketing approval, that we or a collaboration partner bring to the market may not gain market acceptance by physicians, patients, third-party payers, and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and we may not become profitable. The degree of market acceptance of our current products and product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the prevalence and severity of any side effects, including any limitations or warnings contained in a product's approved labeling;
- the efficacy and potential advantages over alternative treatments;
- the pricing of our product candidates;
- the relative convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party insurance coverage and reimbursement.

As part of the NDA review process for sparsentan for IgAN, the FDA 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. The FDA recently accepted for review our sNDA efficacy supplement requesting modification of the frequency of liver monitoring during the first year that patients are taking FILSPARI, and assigned a PDUFA target action date of August 28, 2025. While we believe that the data we submitted 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 its anticipated 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 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.

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

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

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

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

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

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product or could substantially increase the costs and expenses of commercializing the product candidate, which in turn could delay or prevent us from generating significant revenues from its sale or adversely affect our reputation.

We do not currently have patent protection for certain of our commercial products. If we are unable to obtain and maintain protection for the intellectual property relating to our technology and products, their value will be adversely affected.

Our success will depend in large part on our ability to obtain and maintain protection in the United States and other countries for the intellectual property covering, or incorporated into, our technology and products. The patent situation in the field of biotechnology and pharmaceuticals generally is highly uncertain and involves complex legal, technical, scientific and factual questions. We do not have, and do not expect to obtain, patent protection for the original formulation of Thiola. Additionally, although we have a license to a granted U.S. patent covering the treatment of cystinuria by administering Thiola EC with food (U.S. Patent No. 11,458,104, "the '104 patent"), as well as a pending U.S. patent application directed to Thiola EC, certain generic manufacturers have been able to obtain “skinny-label” approvals of generic versions of tiopronin EC as described below, and the pending U.S. patent application or any future patent application may not 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 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, companies may be able to obtain up to five years patent term extension (to compensate for regulatory approval delay) for one patent covering such a product for its FDA-approved use. Such a patent, like the periods of regulatory exclusivity, also may be extended by a further six months on the basis of pediatric exclusivity if certain conditions are satisfied. While we have filed an application for patent term extension of U.S. Patent No. 9,993,461, which, if granted could extend the term of U.S. Patent No. 9,993,461 to October 2032, there is no guarantee that such patent term extension will be granted to such date, or at all. In addition, while we intend to seek pediatric exclusivity for FILSPARI based on our ongoing development efforts, which, if granted, could extend the term of such patent by an additional six months, the granting of pediatric exclusivity requires a series of regulatory interactions to reach agreement with the FDA, and there is no guarantee that our pediatric development efforts will support a path to pediatric exclusivity or that pediatric exclusivity will be granted by the FDA on a timeline that confers benefit on the term of patent coverage or regulatory exclusivity for FILSPARI, or at all.

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

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

We have 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.

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

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

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

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

In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the "PPACA"), was signed into law, which intended to, among other things, broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The PPACA revised the definition of “average manufacturer price” for reporting purposes, which could increase the amount of Medicaid drug rebates to states. The PPACA also increased the mandated Medicaid rebate from 15.1% to 23.1% of the average manufacturer price, expanded the rebate to Medicaid managed care

utilization and increased the types of entities eligible for the federal 340B drug discount program. Further, the law imposed a significant annual fee on companies that manufacture or import certain branded prescription drug products. There have been executive, judicial, Congressional, and political challenges and amendments to certain aspects of the PPACA. For example, on August 16, 2022, the Inflation Reduction Act of 2022 ("IRA") was signed into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in PPACA marketplaces through plan year 2025. The IRA also eliminates the "donut hole" under the Medicare Part D program beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost and through a newly established manufacturer discount program. It is possible that the PPACA will be subject to judicial or Congressional challenges in the future. It is unclear how any potential future healthcare reform measures of the Trump administration will impact the PPACA and our business.

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

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

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

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

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

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

Further, there has been increasing legislative and enforcement interest in the United States with respect to drug pricing practices, including several recent U.S. congressional inquiries and federal and state legislation designed to, among other things, increase drug pricing transparency, expedite generic competition, review relationships between pricing and manufacturer patient assistance programs, and reform government program drug reimbursement methodologies. For example, the IRA, among other things (i) directs HHS to negotiate the price of certain high-expenditure, single-source drugs that have been on the market for at least 7 years covered under Medicare (the "Medicare Drug Price Negotiation Program"), and (ii) imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation. These provisions began to take effect progressively starting in fiscal year 2023. On August 15, 2024, HHS announced the agreed-upon price of the first ten drugs that were subject to price

negotiations, although the Medicare Drug Price Negotiation Program is currently subject to legal challenges. On January 17, 2025, HHS selected fifteen additional drugs covered under Part D for price negotiation in 2025. Each year thereafter more Part B and Part D products will become subject to the Medicare Drug Price Negotiation Program. HHS has and will continue to issue and update guidance as these programs are implemented. Further, on December 7, 2023, an initiative to control the price of prescription drugs through the use of march-in rights under the Bayh-Dole Act was announced. On December 8, 2023, the National Institute of Standards and Technology published for comment a Draft Interagency Guidance Framework for Considering the Exercise of March-In Rights which for the first time includes the price of a product as one factor an agency can use when deciding to exercise march-in rights. While march-in rights have not previously been exercised, it is uncertain if that will continue under the new framework. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. For example, on January 5, 2024, the FDA approved Florida's Section 804 Importation Program (SIP) proposal to import certain drugs from Canada for specific state healthcare programs. It is unclear how this program will be implemented, including which drugs will be chosen, and whether it will be subject to legal challenges in the United States or Canada. Other states have also submitted SIP proposals that are pending review by the FDA. Any such approved importation plans, when implemented, may result in lower drug prices for products covered by those programs.

In addition, the current administration is pursuing policies to reduce regulations and expenditures across government including at the HHS, the FDA, Center for Medicare and Medicaid Services ("CMS") and related agencies. These actions, presently directed by executive orders or memoranda from the Office of Management and Budget, may propose policy changes that create additional uncertainty for our business. These actions may include directives to reduce agency workforce, rescinding a Biden administration executive order tasking the Center for Medicare and Medicaid Innovation to consider new payment and healthcare models to limit drug spending and eliminating the Biden administration's executive order that directed HHS to establishing an AI task force and developing a strategic plan. Additionally, in its June 2024 decision in Loper Bright Enterprises v. Raimondo ("Loper Bright"), the U.S. Supreme Court overturned the longstanding Chevron doctrine, under which courts were required to give deference to regulatory agencies' reasonable interpretations of ambiguous federal statutes. The Loper Bright decision could result in additional legal challenges to current regulations and guidance issued by federal agencies applicable to our operations, including those issued by the FDA. Finally, Congress may introduce and ultimately pass health care related legislation that could impact the drug approval process and make changes to the Medicare Drug Price Negotiation Program created under the IRA. We cannot predict which additional measures may be adopted or the impact of current and additional measures on the marketing, pricing and demand for our products, which could have a material adverse effect on our business, financial condition and results of operations.

Any reduction in reimbursement from Medicare, Medicaid or other government-funded programs may result in a similar reduction in payments from private payers. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our therapies. Additionally, we are currently unable to predict what additional legislation or regulation, if any, relating to the healthcare industry may be enacted in the future or what effect recently enacted federal legislation or any such additional legislation or regulation would have on our business, particularly in light of the upcoming U.S. Presidential and Congressional elections.

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

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

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

Several of our competitors have substantially greater financial, research and development, distribution, manufacturing and marketing experience and resources than we do and represent substantial long-term competition for us. Other companies may succeed in developing and marketing products that are more effective and/or less costly than any products that may be developed and marketed by us, or that are commercially accepted before or perceived as preferred relative to any of our products, or that obtain preferential formulary and reimbursement status. Factors affecting competition in the pharmaceutical and therapeutic industries vary, depending on the extent to which a competitor is able to achieve a competitive advantage based on its proprietary technology and ability to market and sell therapeutics. The industry in which we compete is characterized by extensive research and development efforts and rapid technological progress. In particular, the competitive landscape for IgAN is rapidly evolving and is expected to continue to evolve as multiple new modalities advance in development and potentially gain approval. 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. More detailed information is available under the heading "Competition" in Item 1 of Part I of this Annual Report on Form 10-K.

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 December 31, 2024, several generic options for the 100mg and 300mg versions of Thiola EC have been approved by the FDA and become available.

Our competitive position also depends on our ability to enter into strategic alliances with one or more large pharmaceutical and contract manufacturing companies, attract and retain qualified personnel, develop effective proprietary products, implement development and marketing plans, obtain patent protection, secure adequate capital resources and successfully sell and market our approved products. There can be no assurance that we will be able to successfully achieve all of the foregoing objectives.

Use of third parties to manufacture our products and product candidates may increase the risk that we will not have sufficient quantities of our product and product candidates or such quantities at an acceptable cost, and clinical development and commercialization of our product and product candidates could be delayed, prevented or impaired.

We do not own or operate manufacturing facilities for clinical or commercial production of our products or product candidates. We have limited personnel with experience in drug manufacturing and we lack the resources and the capabilities to manufacture any of our product candidates on a clinical or commercial scale. We outsource all manufacturing and packaging of our nonclinical, clinical, and commercial products to third parties. The manufacture of pharmaceutical products in general, and biologics in particular, requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up initial production and in maintaining required quality control. These problems include difficulties with production costs and yields and quality control, including stability of the product candidate.

In September 2024, we announced a voluntary pause of enrollment in the Phase 3 HARMONY Study evaluating pegtibatinase for the treatment of classical homocystinuria (HCU). The voluntary enrollment pause enables us to work to address necessary process improvements in manufacturing scale-up to support commercial scale manufacturing as well as full enrollment in the HARMONY Study. The voluntary enrollment pause was enacted following our determination that the desired drug substance profile was not achieved in the recent scale-up process. We are making progress on necessary process improvements in manufacturing scale-up and currently anticipate that we should be in position to restart enrollment in the Phase 3 HARMONY Study 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. House of Representatives recently 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. Additionally, in September 2024, we announced a voluntary pause of enrollment in the Phase 3 HARMONY Study to enable us to work to address necessary process improvements in manufacturing scale-up to support commercial scale manufacturing as well as full enrollment in the HARMONY Study. The voluntary enrollment pause was enacted following our determination that the desired drug substance profile was not achieved in the 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.

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

If any of our products or any similar products distributed by other companies prove to be, or are asserted to be, harmful to consumers and/or subject to FDA or comparable foreign regulatory authority enforcement action, our ability to successfully market and sell our products could be impaired. Because of our dependence on patient and physician perceptions, any adverse publicity associated with illness or other adverse effects resulting from the use or misuse of our products or any similar products distributed by other companies could limit the commercial potential of our products and expose us to potential liabilities.

We may not have sufficient insurance to cover our liability in any current or future litigation claims either due to coverage limits or as a result of insurance carriers seeking to deny coverage of such claims.

We face a variety of litigation-related liability risks. Our certificate of incorporation, bylaws, other applicable agreements, and/or Delaware law require us to indemnify (and advance expenses to) our current and past directors and officers and employees from reasonable expenses related to the defense of any action arising from their service to us, including circumstances under which indemnification is otherwise discretionary. While our directors and officers are included in a director and officer liability insurance policy ("D&O insurance"), which covers all our directors and officers in some circumstances, our insurance coverage does not cover all of our indemnification obligations and may not be adequate to cover any indemnification or other claims against us. In addition, the underwriters of our present coverage may seek to avoid coverage in certain circumstances based upon the terms of the respective policies. If we incur liabilities that exceed our coverage under our D&O insurance or incur liabilities not covered by our insurance, we would have to self-fund any indemnification amounts owed to our directors and officers and employees in which case our results of operations and financial condition could be materially adversely affected. Further, if D&O insurance becomes prohibitively expensive to maintain in the future, we may be unable to renew such insurance on economic terms or unable to renew such insurance at all. The potential lack of D&O insurance may make it difficult for us to retain and attract talented and skilled directors and officers to serve our company, which could adversely affect our business.

We may need substantial funding and may be unable to raise capital when needed.

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

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

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

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

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

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

- results of clinical trials of our product candidates or those of our competitors;
- our entry into or the loss of a significant collaboration;
- regulatory or legal developments in the United States and other countries, including changes in the health care payment systems;
- our ability to obtain and maintain marketing approvals from the FDA or similar regulatory authorities outside the United States;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors and issuance of new or changed securities analysts' reports or recommendations;
- general economic, industry and market conditions, including the impacts thereon of inflation and high interest rates, actual or anticipated bank failures, new or increased tariffs, wars, armed conflicts and global geopolitical tensions;
- results of clinical trials conducted by others on therapies that would compete with our product candidates;
- developments or disputes concerning patents or other proprietary rights;
- public concern over our product candidates or any products approved in the future;
- litigation;
- communications from government officials regarding health care costs or pharmaceutical pricing;
- future sales or anticipated sales of our common stock by us or our stockholders; and
- the other factors described in this "Risk Factors" section.

In addition, the stock markets, and in particular, the Nasdaq Stock Market, have experienced extreme price and volume fluctuations that have affected and continue to affect the market prices of equity securities of many pharmaceutical companies. The realization of any of the above risks or any of a broad range of other risks, including those described in these "Risk Factors" could have a dramatic and material adverse impact on the market price of our common stock.

We may not receive some or all of the potential milestone and/or royalty payments from our corporate and licensing transactions.

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

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

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

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

Furthermore, these acquisitions and other arrangements, even if successfully integrated, may fail to further our business strategy as anticipated, expose us to increased competition or challenges with respect to our products or geographic markets, and expose us to additional liabilities associated with an acquired business, product, technology or other asset or arrangement. Any one of these challenges or risks could impair our ability to realize any benefit from our acquisitions or arrangements after we have expended resources on them.

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

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

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

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

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

From time to time we may become involved in certain litigation matters, including those described in Note 11 of the Consolidated Financial Statements included in this report. Although we intend to vigorously defend our interests in each matter, there is no guarantee that we will be successful and we may have to pay damages awards or otherwise may enter into settlement arrangements in connection with such matters. Any such payments or settlement arrangements could have material adverse effects on our business, operating results or financial condition. Even if we are successful in defending our interests in each matter, litigation with respect to such matters could result in substantial costs and significant adverse impact on our reputation and divert management's attention and resources, which could have a material adverse effect on our business, operating results or financial condition.

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

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

We are also subject to regulation by supranational, national, regional, state and local agencies and regulatory authorities, including but not limited to the FDA, the CMS, Department of Justice, the Federal Trade Commission, the HHS Office of Inspector General and other regulatory bodies. The FDC Act, Social Security Act, Public Health Service Act and other federal and state statutes and regulations, and comparable foreign regulatory acts, govern to varying degrees the research, development, manufacturing and commercial activities relating to prescription pharmaceutical products, including nonclinical testing, clinical research, approval, production, labeling, sale, distribution, post-market surveillance, advertising, dissemination of information, promotion, marketing, and pricing to government purchasers and government health care programs. Our manufacturing partners are subject to many of the same requirements.

Companies may not promote drugs for “off-label” uses—that is, uses that are not described in the product’s labeling and that differ from those approved by the FDA or other applicable regulatory authorities. However, a company may share truthful and not misleading information that is otherwise consistent with the product’s labeling. A company that is found to have improperly promoted off-label uses may be subject to significant liability, including civil and administrative remedies as well as criminal sanctions. In addition, management’s attention could be diverted from our business operations and our reputation could be damaged.

The federal health care program Anti-Kickback Statute prohibits, among other things, knowingly and willfully offering, paying, soliciting, or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any health care item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted broadly to apply to arrangements that pharmaceutical companies have with prescribers, purchasers and formulary managers, among others. Further, the PPACA, among other things, amends the intent requirement of the federal Anti-Kickback Statute so that a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. In addition, the PPACA provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act. Although there are a number of statutory exceptions and regulatory safe harbors under the federal Anti-Kickback Statute protecting certain common manufacturer business arrangements and activities from prosecution, the exceptions and safe harbors are drawn narrowly and an arrangement must meet all of the conditions specified in order to be fully protected from scrutiny under the federal Anti-Kickback Statute. We seek to comply with the exceptions and safe harbors whenever possible, but our practices, such as our patient assistance programs and discounts with certain customers, may not in all cases meet all of the criteria for protection from Anti-Kickback liability and may be subject to scrutiny.

The federal false claims laws, including the federal False Claims Act, prohibit any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to get a false claim paid. Additionally, the civil monetary penalties statute imposes penalties against any person or entity that, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent. Many pharmaceutical and other health care companies have been investigated and have reached substantial financial settlements with the federal government under the federal False Claims Act for a variety of alleged marketing activities, including providing free product to customers with the expectation that the customers would bill federal programs for the product; providing consulting fees, grants, free travel, and other benefits to physicians to induce them to prescribe the company’s products; and inflating prices reported to private price

publication services, which may be used by states to set drug payment rates under government health care programs. Companies have been prosecuted for causing false claims to be submitted because of the marketing of their products for unapproved uses. Pharmaceutical and other health care companies have also been prosecuted on other legal theories of Medicare and Medicaid fraud.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. It is not clear whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of any Travere products, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

We also could become subject to government investigations and related subpoenas. Such subpoenas are often associated with previously filed qui tam actions, or lawsuits filed under seal under the federal False Claims Act. Qui tam actions are brought by private plaintiffs suing on behalf of the federal government for alleged violations of the federal False Claims Act. The time and expense associated with responding to such subpoenas, and any related qui tam or other actions, may be extensive, and we cannot predict the results of our review of the responsive documents and underlying facts or the results of such actions. Responding to government investigations, defending any claims raised, and any resulting fines, restitution, damages and penalties, settlement payments or administrative actions, as well as any related actions brought by stockholders or other third parties, could have a material impact on our reputation, business and financial condition and divert the attention of our management from operating our business.

The number and complexity of both federal and state laws continues to increase, and additional governmental resources are being added to enforce these laws and to prosecute companies and individuals who are believed to be violating them. In particular, the PPACA includes a number of provisions aimed at strengthening the government's ability to pursue Anti-Kickback and False Claims Act cases against pharmaceutical manufacturers and other healthcare entities, including substantially increased funding for healthcare fraud enforcement activities, enhanced investigative powers, amendments to the federal False Claims Act that make it easier for the government and whistleblowers to pursue cases for alleged kickback and false claim violations and public reporting of certain payments and transfers of value by certain pharmaceutical manufacturers to physicians and teaching hospitals nationwide. We anticipate that government scrutiny of pharmaceutical sales and marketing practices will continue for the foreseeable future and subject us to the risk of further government investigations and enforcement actions. Responding to a government investigation or enforcement action would be expensive and time-consuming, and could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

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

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

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

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

requiring the reporting of certain information related to drug pricing; and requiring drug manufacturers to track and report information related to payments, gifts, compensation, and other items of value to physicians and other healthcare providers.

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

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

If we are not able to obtain and maintain required regulatory approvals, we will not be able to commercialize our products, and our ability to generate revenue will be materially impaired.

Our product candidates, once approved, and the activities associated with their manufacture, marketing, distribution, and sales are subject to extensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries. Failure to adhere to regulations set out by these bodies for one or more of our commercial products could prevent us from commercializing the product candidate in the jurisdiction of the regulatory authority. We have only limited experience in meeting the regulatory requirements incumbent on the sale of drugs in the United States and elsewhere, and expect to rely on third parties to assist us in these processes. If these third parties fail to adequately adhere to the regulations governing drug distribution and promotion, we may be unable to sell our products, which could have a material effect on our ability to generate revenue.

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

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

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

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

- a change in the approval policies or regulations of the FDA or comparable regulatory authorities or a change in the laws governing the approval process.

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

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

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

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

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

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

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

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

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

In the ordinary course of business, we transfer personal data from Europe and other jurisdictions to the United States or other countries. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal data to other countries. In particular, the European Economic Area ("EEA") and the United Kingdom ("UK") have significantly restricted the transfer of personal data to the United States and other countries whose privacy laws it generally believes are inadequate. Other jurisdictions may adopt or have already adopted similarly stringent localization and cross-border transfer laws, which could make it more difficult to transfer information across jurisdictions or prevent us from conducting business in certain countries. Although there are currently various mechanisms that are used to transfer personal data from the EEA and UK to the United States in compliance with these laws, such as the EU Standard Contractual Clauses ("EU SCCs"), the UK's International Data Transfer Agreement / International Data Transfer Addendum to the EU SCCs, and the EU-U.S. Data Privacy Framework and the UK extension thereto (which allows for transfers to relevant U.S.-based organizations who self-certify compliance and participate in the applicable frameworks), these mechanisms may be subject to legal challenges, and there is no assurance that we can satisfy or rely on the Data Privacy Framework to lawfully transfer personal data to the United States.

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

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

Our obligations related to data privacy and security (and consumers' data privacy expectations) are quickly changing in an increasingly stringent fashion, creating uncertainty. Additionally, these obligations are subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions. Preparing for and complying with these obligations requires significant resources and may necessitate changes to our information technologies, systems, and practices and to those of any third parties with whom we work. Although we endeavor to comply with all applicable data privacy and security obligations, we may at times fail (or be perceived to have failed) to do so. Moreover, despite our efforts, our personnel or third parties with whom we work may fail to comply with such obligations, which could negatively impact our business operations and compliance posture. For example, any failure by a third party with whom we work to comply with applicable law, regulations, or contractual obligations could result in adverse effects, including proceedings against us by governmental entities or others. If we or any of the third parties with whom we work fail to comply or are perceived to have failed to comply with applicable obligations, we or they could be subject to a range of regulatory actions, litigation (including class actions), or mass arbitration demands that could affect our or our partners' ability to commercialize our products and conduct necessary research and development, and could harm or prevent sales of the affected products, or could substantially increase the costs and expenses of commercializing and marketing our products. In particular, plaintiffs have become increasingly more active in bringing privacy-related claims against companies, including class claims and mass arbitration demands. Some of these claims allow for the

recovery of statutory damages on a per violation basis, and, if viable, carry the potential for monumental statutory damages, depending on the volume of data and the number of violations. Any threatened or actual government enforcement action or litigation could also generate adverse publicity and require that we devote substantial resources that could otherwise be used in other aspects of our business. Compliance with applicable federal, state, and foreign laws is difficult and time consuming, and companies that violate them may face substantial penalties. The potential sanctions include significant criminal fines, civil monetary penalties, administrative penalties, disgorgement, exclusion from participation in federal health care programs, individual imprisonment, injunctions, recall or seizure of products, total or partial suspension of production, reputational harm, administrative burdens, interruption or cessation of clinical trials, additional oversight and reporting obligations if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, diminished profits and future earnings, and the curtailment or restructuring of our operations, and other sanctions. Because of the breadth of these laws, it is possible that some of our business activities could be subject to challenge under one or more of these laws. Such a challenge, irrespective of the underlying merits of the challenge or the ultimate outcome of the matter, could have a material adverse effect on our business, financial condition, results of operations and growth prospects. Moreover, clinical trial subjects and other individuals about whom we or the third parties with whom we work obtain personal data, as well as the third parties with whom we work who share this information with us, may limit our ability to collect, use and disclose the information. Claims that we have violated individuals' privacy rights, failed to comply with data protection laws, or breached our contractual obligations, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business.

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

In the ordinary course of our business, we and the third parties with whom we work process proprietary, confidential, and sensitive data, including personal data (such as health-related data and data related to our clinical trials), intellectual property, and trade secrets (collectively, sensitive information).

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

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

While we have implemented security measures designed to protect against security incidents, there can be no assurance that these measures will be effective. We take steps designed to detect, mitigate and remediate vulnerabilities in our information security systems (such as our hardware and/or software, including that of third parties with whom we work), but we have not in the past and may not in the future be able to detect, mitigate, and remediate all such vulnerabilities including on a timely basis. It may also be difficult and/or costly to detect, investigate, mitigate, contain, and remediate a security incident. Further, we have, and may in the future, experienced 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.

Certain of the previously identified or similar threats have in the past, and may in the future, cause a security incident or other interruption that could result in unauthorized, unlawful, or accidental acquisition, modification, destruction, loss, alteration, encryption, disclosure of, or access to our sensitive information or our information technology systems, or those of the third parties with whom we work. For example, we have in the past become aware of a security incident involving one of our CROs where limited business contact data was accessed by an unauthorized third party. A security incident or other interruption could disrupt our ability (and that of third parties with whom we work) to provide our products. We expend resources and may have to modify our business activities (including our clinical trial activities) to try to protect against security incidents. Certain data privacy and security obligations require us to implement and maintain specific security measures, industry-standard or reasonable security measures to protect our information technology systems and sensitive information.

Applicable data security and public company disclosure obligations require us, or we may voluntarily choose, to notify relevant stakeholders of certain security incidents, including affected individuals, customers, regulators and investors, or to take other actions in certain circumstances, such as providing credit monitoring and identity theft protection services. Whether a cybersecurity incident is reportable to our investors may not be straightforward, may take considerable time to determine, and may be subject to change as the investigation of the incident progresses, including changes that may significantly alter any initial disclosure that we provide. Such disclosures and related actions can be costly, and the disclosures or the failure to comply with such applicable requirements, could lead to adverse consequences. 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, such as government enforcement actions (for example, investigations, fines, penalties, audits, and inspections); additional reporting requirements and/or oversight; restrictions on processing sensitive information (including personal data); litigation (including class claims); indemnification obligations; negative publicity; reputational harm; loss of customer, investor or partner confidence in the effectiveness of our cybersecurity measures; monetary fund diversions; the expenditure of significant capital and other resources; diversion of management attention; interruptions in our operations (including availability of data); financial loss and other similar harms. For example, the loss of clinical trial data from completed or ongoing clinical trials for any of our product candidates could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data.

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

In addition to experiencing a security incident, third parties may gather, collect, or infer sensitive information about us from public sources, data brokers, or other means that reveals competitively sensitive details about our organization and could be used to undermine our competitive advantage or market position. Sensitive information of us or our customers could also be leaked, disclosed, or revealed as a result of or in connection with our employee's, personnel's, or third parties with whom we work use of generative AI technologies.

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

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

Several jurisdictions around the globe, including Europe and certain U.S. states, have proposed, enacted, or are considering laws governing AI/ML, including the EU's AI Act and the Colorado AI Act. We expect other jurisdictions will adopt similar laws. Additionally, certain privacy laws extend rights to consumers (such as the right to delete certain personal data) and regulate automated decision making, which may be incompatible with our use of AI/ML. These obligations may make it harder for us and our employees and personnel to use AI/ML, lead to regulatory fines or penalties, require us to change our business practices or make changes to the AI/ML that we use, or prevent or limit our use of AI/ML. For example, the FTC has required other companies to turn over (or disgorge) valuable insights or trainings generated through the use of AI/ML where they allege the company has violated privacy and consumer protection laws. If we cannot use AI/ML or that use is restricted, our business may be less efficient, or we may be at a competitive disadvantage.

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

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

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

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

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 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.

U.S. 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, 2024, we had federal NOL carryforwards of $223.8 million. In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation's ability to use its pre-change NOL carryforwards and other pre-change U.S. tax attributes (such as research tax credits) to offset its post-change income or taxes may be limited. We continue to evaluate potential historical ownership changes and we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control.

As a result, our federal NOL carryforwards may be subject to a percentage limitation if used to offset income in tax years following an ownership change. In addition, it is possible that we have in the past undergone, and in the future may undergo, additional ownership changes that could limit our ability to use all of our pre-change NOL carryforwards and other pre-change tax attributes (such as research tax credits) to offset our post-change income or taxes. Similar provisions of state tax law may also apply to limit our use of accumulated state tax attributes. In addition, at the state level, there may be periods during which the use of NOL carryforwards is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. For example, California imposed limits on the usability of California state net operating losses to offset taxable income in tax years beginning after 2023 and before 2027. As a result, we may be unable to use all or a material portion of our NOL carryforwards and other tax attributes, which would harm our future operating results by effectively increasing our future tax obligations.

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

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

Disruptions at the FDA and other agencies may also slow the time necessary for new therapies to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the U.S. government has shut down several times 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 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 man-made disasters or business interruptions, for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses.

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

In addition, we rely on third-party manufacturers, some of whom are located in China, to manufacture API for FILSPARI and certain of our product candidates. Any disruption in production or inability of our manufacturers in China to produce or ship adequate quantities to meet our needs, whether as a result of a natural disaster or other causes (such as staffing shortages, or a health epidemic or pandemic), could impair our ability to meet commercial demand for FILSPARI, to operate our business on a day-to-day basis and to continue our research and development of our product candidates. In addition, we are exposed to the possibility of product supply disruption and increased costs in the event of changes in the policies of the United States or Chinese governments (such as tariffs on products or materials that we use that are manufactured in China), political unrest or unstable economic conditions in China. Any recall of the manufacturing lots or similar action regarding our API used in clinical trials could delay the trials or detract from the integrity of the trial data and its potential use in future regulatory filings. In addition, manufacturing interruptions or failure to comply with regulatory requirements by any of these manufacturers could significantly delay clinical development of potential products and reduce third-party or clinical researcher interest and support of proposed trials. These interruptions or failures could also impede commercialization of our product candidates and impair our competitive position.

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

A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting such that there is a reasonable possibility that a material misstatement of our annual or interim consolidated financial statements will not be prevented or detected on a timely basis. If material weaknesses in our internal control over financial reporting are discovered or occur in the future, or if we are unable to maintain effective internal control over financial reporting or disclosure controls and procedures for any reason, our ability to record, process and report financial information accurately, and to prepare financial statements within required time periods, could be adversely affected, which could subject us to litigation or investigations requiring management resources and payment of legal and other expenses and negatively impact the price of our common stock. In addition, we could be subject to sanctions or investigations by the SEC or other regulatory authorities, which would require additional financial and management resources.

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

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

Adverse developments that affect financial institutions, such as events involving liquidity that are rumored or actual, have in the past and may in the future lead to bank failures and market-wide liquidity problems. For example, on March 10, 2023, Silicon Valley Bank (SVB) was closed by the California Department of Financial Protection and Innovation, which appointed the Federal Deposit Insurance Corporation (FDIC) as receiver. Similarly, on March 12, 2023, Signature Bank and Silvergate Capital Corp. were each swept into receivership. In addition, on May 1, 2023, the FDIC seized First Republic Bank and sold its assets to JPMorgan Chase & Co. It is uncertain whether the U.S. Department of Treasury, FDIC and Federal Reserve Board will provide access to uninsured funds in the future in the event of the closure of other banks or financial institutions, or that they would do so in a timely fashion.

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

In addition, widespread investor concerns regarding the U.S. or international financial systems could result in less favorable commercial financing terms, including 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.

Risks Related to our Indebtedness and Investments

Our indebtedness could adversely affect our financial condition.

As of December 31, 2024, we had approximately $385 million of total debt outstanding, of which $69 million is classified as current and $316 million is classified as long term. As a result of our indebtedness, a portion of our cash flow will be required to pay interest and principal on the 2025 Notes and 2029 Notes if the notes are not converted to shares of common stock prior to maturity. We may not generate sufficient cash flow from operations or have future borrowings available to enable us to repay our indebtedness or to fund other liquidity needs.

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

- make it more difficult for us to satisfy our obligations with respect to any other debt we may incur in the future;
- increase our vulnerability to general adverse economic and industry conditions;
- require us to dedicate a substantial portion of our cash flow from operations to payments on our indebtedness and related interest, thereby reducing the availability of our cash flow to fund working capital, capital expenditures and other general corporate purposes;

- limit our flexibility in planning for, or reacting to, changes in our business and the industry in which we operate;
- increase our cost of borrowing;
- place us at a competitive disadvantage compared to our competitors that may have less debt; and
- limit our ability to obtain additional financing for working capital, capital expenditures, acquisitions, debt service requirements or general corporate purposes.

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

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

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

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

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

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

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

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

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

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

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

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

General Risk Factors

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

The global credit and financial markets have experienced extreme volatility and disruptions, including as a result of inflation and high interest rates, bank failures, new or increased tariffs, 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 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 1C. CYBERSECURITY

Risk Management and Strategy

We have implemented and maintain various information security processes designed to identify, assess and manage material risks from cybersecurity threats to our critical computer networks, third party hosted services, communications systems, hardware and software, and our critical data, including intellectual property, confidential information that is proprietary, strategic or competitive in nature, and data related to patients and clinical trials (“Information Systems and Data”).

Various members of our management team, IT department and other employees, including but not limited to the individuals on our cybersecurity incident management team, help identify, assess and manage our cybersecurity threats and risks, with the assistance of a third-party IT managed

service provider. We manage, identify and assess risks from cybersecurity threats by monitoring and evaluating our threat environment and risk profile using various methods including, for example: through the use of automated tools in certain environments, including but not limited to tools for monitoring, geolocation, remote wiping, threat detection, intrusion detection and prevention (including through the use of machine learning, a form of artificial intelligence), patch management, distributed denial of service (DDoS) protection and forensics; conducting (directly or through third parties) regular audits and threat assessments for internal and external threats; subscribing to reports and services that identify cybersecurity threats; analyzing reports of certain threats and actors; conducting vulnerability assessments in certain environments to identify vulnerabilities; evaluating our and our industry's risk profile; conducting tabletop incident response exercises; and evaluating certain threats reported to us.

Depending on the environment, we implement and maintain various technical, physical, and organizational measures, processes, standards and policies designed to manage and mitigate material risks from cybersecurity threats to our Information Systems and Data, including, for example: incident response plans and procedures, disaster recovery/business continuity plans, risk assessments, implementation of security standards and certifications, encryption of certain data, network security controls on certain networks, data segregation, Wi-Fi segregation, access controls for certain environments, physical security, asset management, tracking and disposal, systems monitoring, vendor risk management program, employee training and penetration testing.

Our assessment and management of material risks from cybersecurity threats are integrated into our overall risk management processes. For example, cybersecurity risk is addressed as a component of our enterprise risk management program, and members of our management team, IT department and other relevant team members work together to prioritize our risk management processes, mitigate cybersecurity threats that are more likely to lead to a material impact to our business, and report regularly to our board of directors on cybersecurity matters.

We use third-party service providers to assist us from time to time to identify, assess, and manage material risks from cybersecurity threats, including for example managed cybersecurity service providers, threat intelligence service providers, dark web monitoring services, and other cybersecurity software providers.

We use third-party service providers to perform a variety of functions throughout our business, including but not limited to application providers, hosting companies, contract manufacturing organizations and contract research organizations. We have a vendor management program to oversee, identify and manage cybersecurity risks associated with our use of certain of these providers. The program includes a risk assessment for vendors that may include, depending on the vendor and nature of services being performed, security questionnaires, review of the vendor's written security program, review of security assessments, audits and reports, vulnerability scans related to the vendor, security assessment calls with the vendor's security personnel, and the imposition of certain contractual obligations on the vendor, among other elements, in accordance with the processes outlined in our internal vendor selection, management, and oversight process policy and other internal guidelines. More specifically, the level of assessment may depend on the nature of the services provided, the sensitivity of the Information Systems and Data at issue, and the identity of the provider.

For a description of the risks from cybersecurity threats that may materially affect us and how they may do so, see our risk factors under Part 1. Item 1A. Risk Factors in this Annual Report on Form 10-K, including the risk factor captioned "If our information technology systems or data, or those of third parties upon which we rely, are or were compromised, we could experience adverse impacts resulting from such compromise, including, but not limited to, regulatory investigations or actions; litigation; fines and penalties; interruptions to our commercial operations, clinical trials or other operations; harm to our reputation; loss of revenue or profits; loss of sales and other adverse consequences."

Governance

Our Nominating / Corporate Governance Committee has oversight of our cybersecurity risk management program and reports to our board of directors on cybersecurity matters.

Our cybersecurity risk assessment and management processes are implemented and maintained by various members of our management team, IT department and other employees, including but not limited to the individuals on our cybersecurity incident management team, which includes individuals who have a diverse combination of relevant expertise, experience, education and training, with representation from our IT, legal, human resources, compliance, risk and privacy functions, among others. Our team includes individuals with relevant experience in enterprise risk management and disclosure controls and procedures. Additionally, certain members of our IT department have experience managing cybersecurity programs and are specifically assigned cybersecurity oversight.

Certain members of our management team and IT department are responsible for hiring appropriate personnel, helping to integrate cybersecurity risk considerations into our overall risk management strategy, communicating key priorities to relevant personnel, approving budgets, helping prepare for cybersecurity incidents, approving cybersecurity processes, and reviewing security assessments and other security-related reports.

Our cybersecurity incident response processes are designed to escalate certain cybersecurity incidents to members of management depending on the circumstances, including in some cases to our executive team. Our cybersecurity incident management team, and other individuals as needed, work to help us mitigate and remediate cybersecurity incidents of which we are notified. In addition, our incident response processes include a procedure for reporting certain cybersecurity incidents to the board of directors and/or the Nominating / Corporate Governance Committee.

The Nominating / Corporate Governance Committee receives regular reports from management concerning our cybersecurity risk management program, and also receives various summaries and/or presentations related to cybersecurity threats, risks and mitigation.

ITEM 2. PROPERTIES

As of December 31, 2024, we leased the following locations to conduct our business:

Location	Address	Lease Expiration	Square Feet
San Diego, California	3611 Valley Centre Drive, Suite 300	August 31, 2028	103,677
Dublin, Ireland	3 Mount Street Crescent, 2nd Floor	September 30, 2027	1,960

In November 2024, we entered into an arrangement to sublet 26,455 square feet of our San Diego office space beginning in January 2025 and ending in August 2028. Effective January 31, 2025, we have assigned our lease for the Dublin office to a third party.

We believe our current facilities are adequate to conduct our business.

For additional information regarding our lease agreements, see Note 18 of the Consolidated Financial Statements included in this report.

ITEM 3. LEGAL PROCEEDINGS

The information required by this Item is incorporated herein by reference to the Notes to the Consolidated Financial Statements--Note 11 Commitments and Contingencies: Legal Proceedings in Item 15 of this Annual Report on Form 10-K.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES

Market Information

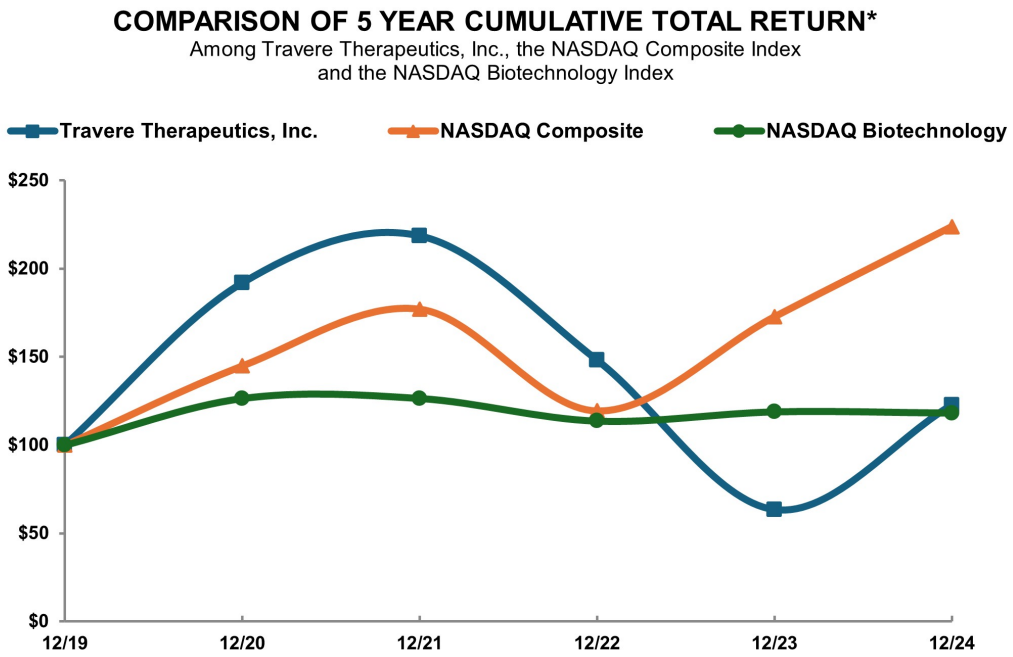
Our common stock is listed for quotation on the Nasdaq Global Market under the trading symbol “TVTX” and is part of the Nasdaq Biotechnology Index (Nasdaq: NBI).

As of February 18, 2025, we had approximately 173 holders of record of our common stock.

Performance Graph

The following is not deemed “filed” with the Securities and Exchange Commission and is not to be incorporated by reference into any filing we make under the Securities Act of 1933, as amended, whether made before or after the date hereof and irrespective of any general incorporation by reference language in such filing.

Our common stock is traded on the Nasdaq Global Market and is a component of both the Nasdaq Composite Index and the Nasdaq Biotechnology Index. The total return for our common stock and for each index assumes the reinvestment of dividends, although dividends have never been declared on our common stock, and is based on the returns of the component companies weighted according to their capitalizations as of the end of each monthly period. The Nasdaq-Composite tracks the aggregate price performance of equity securities of companies traded on the Nasdaq National Market. The Nasdaq Biotechnology Index contains securities and tracks the aggregate price performance of equity securities of Nasdaq-listed companies classified according to the Industry Classification Benchmark as either Biotechnology or Pharmaceuticals which also meet other eligibility criteria. The comparisons shown in the graph are based upon historical data and we caution that the stock price performance shown in the graph is not indicative of, nor intended to forecast, the potential future performance of our stock.



*$100 invested on 12/31/19 in stock or index, including reinvestment of dividends.
Fiscal year ending December 31.

Dividends

Since inception we have not paid any dividends on our common stock. We currently do not anticipate paying any cash dividends in the foreseeable future on our common stock. Although we intend to retain our earnings, if any, to finance the exploration and growth of our business, our Board of Directors will have the discretion to declare and pay dividends in the future. Payment of dividends in the future will depend upon our earnings, capital requirements and other factors which our Board of Directors may deem relevant.

ITEM 6. [RESERVED]

ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

Our discussion and analysis of our financial condition and results of operations for 2024 as compared to 2023 are discussed below and should be read in conjunction with our audited Consolidated Financial Statements, including the notes thereto. For a discussion of our financial condition and results of operations for 2023 as compared to 2022, except as set forth below, please refer to Item 7, “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in our 2023 Annual Report on Form 10-K, which discussion is incorporated by reference herein.

Overview

We are a biopharmaceutical company headquartered in San Diego, California, focused on identifying, developing and delivering life-changing therapies to people living with rare kidney and metabolic diseases. Our approach centers on advancing our innovative pipeline with multiple late-stage clinical programs targeting rare diseases with significant unmet medical needs. Upon approval of any of our late-stage programs, we intend to leverage the skills of our talented commercial organization which has successfully identified, supported and treated patients prescribed our approved products for over ten years.

FILSPARI® (sparsentan)

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

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

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

Results from the PROTECT Study showed that FILSPARI was well tolerated with a clearly defined safety profile that has been consistent across all clinical trials conducted to date.

FILSPARI is a dual endothelin angiotensin receptor antagonist (DEARA). Pre-clinical data have shown that blockade of both endothelin type A and angiotensin II type 1 pathways in forms of rare chronic kidney disease, reduces proteinuria, protects podocytes and prevents glomerulosclerosis and mesangial cell proliferation. FILSPARI has been granted seven years of Orphan Drug Exclusivity in the U.S. (running from the date of accelerated approval) for the reduction of proteinuria in adults with primary IgAN at risk of rapid disease progression, and has been granted a separate seven years of Orphan Drug Exclusivity in the U.S. (running from the date of full approval) to slow kidney function decline in adults with primary IgAN who are at risk for disease progression, excluding the use provided for in the aforementioned Orphan Drug Exclusivity granted in connection with the accelerated approval.

IgAN is characterized by hematuria, proteinuria, and variable rates of progressive renal failure. With an estimated prevalence of up to 150,000 people in the United States and greater numbers in Europe and Asia, IgAN is the most common primary glomerular disease. Most patients are diagnosed between the ages of 16 and 35, with up to 40% progressing to kidney failure within 15 years. FILSPARI is the first non-immunosuppressive therapy approved for IgAN and is the only oral, once-daily, non-immunosuppressive therapy approved for this condition that directly targets glomerular injury in the kidney by blocking two critical pathways of IgAN disease progression (endothelin-1 and angiotensin II). We estimate more than 70,000 patients in the United States to be addressable under FILSPARI's full approval indication.

Data to support the approval of FILSPARI was generated from the Phase 3 PROTECT Study, the largest head-to-head interventional study to date in IgAN. It is a global, randomized, multicenter, double-blind, parallel-arm, active-controlled clinical trial that evaluated the safety and efficacy of 400mg of sparsentan, compared to 300mg of irbesartan, in 404 patients ages 18 years and up with IgAN and persistent proteinuria despite available angiotensin converting enzyme (ACE) inhibitor or angiotensin receptor blockers (ARB) therapy, and is currently ongoing in the open label extension phase of the study.

FILSPARI is available only through a risk evaluation and mitigation strategy (REMS) approved by the FDA, 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. The Company submitted an sNDA for a potential modification to the frequency of liver monitoring for FILSPARI; the sNDA has been accepted for review by the FDA and assigned a PDUFA target action date of August 28, 2025.

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 countries. CSL Vifor submitted an application for full regulatory approval in the second 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 November 2024, the Medicines and Healthcare products Regulatory Agency (MHRA) approved FILSPARI in the United Kingdom.

In January 2024, we announced our entry into an exclusive licensing agreement with Renalys Pharma, Inc. ("Renalys"), to bring sparsentan for the treatment of IgAN to patients in Japan and other countries in Asia. Renalys 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 registration study of sparsentan in Japan to support potential approval of sparsentan in Japan. In July 2024, Renalys announced that the first patient was dosed in the study, and in January 2025, Renalys announced achievement of full enrollment in the study. Results from the urine protein/creatinine ratio (UP/C) endpoint in the study are expected in the second half of 2025 to support a submission for approval to PMDA. In December 2024, Renalys announced that sparsentan received Orphan Drug Designation from the Japanese Ministry of Health, Labour and Welfare for the indication of primary IgA nephropathy as of November 27, 2024. 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 pre-specified secondary endpoints on proteinuria and exploratory endpoints, including renal outcomes, which trended favorably for sparsentan. In addition, a review of the safety results through 108 weeks of treatment indicate sparsentan was generally well-tolerated and the overall safety profile in the study to date was generally consistent between treatment groups.

In December 2023, we announced that we completed a planned Type C meeting with the FDA to discuss results from the Phase 3 DUPLEX Study of sparsentan in FSGS. The FDA acknowledged the high unmet need for approved therapies as well as the challenges in studying FSGS but indicated that the two-year results from the Phase 3 DUPLEX Study alone were not sufficient to support an sNDA submission. The FDA acknowledged the work being done by the larger nephrology community to better understand proteinuria and eGFR as endpoints in clinical trials of FSGS and indicated a willingness to continue to engage with us on a potential path forward for sparsentan in FSGS following our consideration of additional evidence. Subsequently, a collaborative international effort referred to as the PARASOL project was initiated with a goal to define the quantitative relationships between short-term changes in biomarkers (proteinuria and GFR) and long-term outcomes in order to support the use of alternative proteinuria-based endpoints as a basis for accelerated and traditional approval. The PARASOL project is led by several patient advocacy organizations focused on glomerular diseases, with participation from regulators and industry representatives. The principal finding from PARASOL was that in FSGS, reduction in proteinuria over 24 months is strongly associated with a reduction in the risk of kidney failure, and responder definitions based on thresholds of proteinuria are both biologically plausible and strongly supported by epidemiological data. Following the 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 scheduled a Type C meeting with the FDA to discuss a potential regulatory pathway for a sparsentan FSGS indication. In February 2025, we announced that we had completed a Type C meeting with the FDA and that we plan to submit an sNDA around the end of the first quarter of 2025 seeking traditional approval of FILSPARI for FSGS. The sNDA will be based on existing data from the Phase 3 DUPLEX and Phase 2 DUET studies of FILSPARI.

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.

Under the terms of our exclusive license to CSL Vifor, CSL Vifor is responsible for all commercialization activities in its licensed territories. We remain responsible for the clinical development of sparsentan in the applicable territories. If sparsentan receives marketing authorization in any of the territories covered by the exclusive license to Renalys, Renalys will be responsible for all development, regulatory matters, and commercialization activities in such licensed territories. We will retain all rights to sparsentan in the United States and rest of world outside of the territories licensed to CSL Vifor and Renalys, provided that CSL Vifor has a right of negotiation to expand the licensed territories into Canada, 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. We estimate that there are approximately 7,000 to 10,000 addressable HCU patients globally. Pegtibatinase has been granted Rare Pediatric Disease, Fast Track and Breakthrough Therapy designations by the FDA, as well as orphan drug designation in the United States and European Union.

In December 2021, we announced positive topline results from the Phase 1/2 COMPOSE Study, a double blind, randomized, placebo-controlled dose escalation study to assess its safety, tolerability, pharmacokinetics, pharmacodynamics and clinical effects in patients with classical HCU. Pegtibatinase demonstrated dose-dependent reductions in total homocysteine (tHcy) during the 12 weeks of treatment, and in the highest dose cohort to date evaluating 1.5 mg/kg of pegtibatinase twice weekly (BIW), treatment with pegtibatinase resulted in rapid and sustained reductions in total homocysteine (tHcy) through 12 weeks of treatment, including a 55.1% mean relative reduction in tHcy from baseline as well as maintenance of tHcy below a clinically meaningful threshold of 100 µmol. Additionally, in a dose-dependent manner in the study to date, methionine levels were substantially reduced and cystathionine levels were substantially elevated following treatment with pegtibatinase, suggesting that pegtibatinase acts in a manner similar to the native CBS enzyme.

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

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

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

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

Preclinical Program:

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 U.S. is estimated to be 10,000 to 12,000, indicating that there may be as many as 4,000 to 5,000 affected individuals with cystinuria in the U.S. that would be candidates for Thiola or Thiola EC.

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

In May 2021, a generic option for the 100mg version of the original formulation of Thiola (tiopronin tablets) became available and in June 2022, a second option for the 100mg version of the original formulation of Thiola (tiopronin tablets) was approved. These generic versions of the original formulation of Thiola have impacted our sales, and these or additional generic versions of either formulation could have a material adverse impact on sales. As of December 31, 2024, several generic options for the 100mg and 300mg versions of Thiola EC have been approved by the FDA and become available. Accordingly, Thiola EC is subject to generic competition.

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 19 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. As of December 31, 2024, we have incurred total non-recurring charges of $13.8 million in connection with the restructuring, and are no longer incurring restructuring expenses.

Critical Accounting Estimates

Management makes certain judgments and uses certain estimates and assumptions when applying accounting principles generally accepted in the United States (“GAAP”) in the preparation of our Consolidated Financial Statements. We evaluate our estimates and judgments on an ongoing basis and base our estimates on historical experience and on assumptions that we believe to be reasonable under the circumstances. Our experience and assumptions form the basis for our judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may vary from what we anticipate and different assumptions or estimates about the future could change our reported results. We believe the following accounting policies are the most critical to us, in that they require our most difficult, subjective or complex judgments in the preparation of our Consolidated financial statements. For further information, see Note 2, Summary of Significant Accounting Policies, to our Consolidated Financial Statements, which outlines our application of significant accounting policies.

Revenue Recognition

We recognize revenue when the 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. We only apply the five-step model to contracts when it is probable that the entity will collect substantially all the consideration it is entitled to in exchange for the goods or services it transfers to the customer.

We recognize revenues from product sales when the customer obtains control of the product, which occurs upon delivery to our customer. We receive payments from our product sales based on terms that generally are within 30 days of delivery of product to the patient.

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 its customers, health care providers, payers and other indirect customers relating to the sale of our products. In order to determine the transaction price, we estimate, utilizing the expected value method, the amount of variable consideration to which we will be entitled. These provisions are based on the amounts earned or to be claimed on the related sales and are classified as a reduction of accounts receivable (if the amount is payable to the customer) or as a current liability (if the amount is payable to a party other than a customer). Calculating these provisions involves estimates and judgements. Where appropriate, these reserves take into consideration our historical experience, current contractual and statutory requirements and specific known market events and trends. Overall, these reserves reflect our 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 provisions, we will adjust the provision, which would affect net product revenue and earnings in the period such variances become known. For the years ended December 31, 2024 and 2023, the Company recorded adjustments to net product revenue of $0.5 million and $0.4 million, respectively, related to performance obligations satisfied in previous periods.

Government Rebates: We calculate the rebates that we will be obligated to provide to government programs and deduct these estimated amounts from our gross product sales at the time the revenues are recognized. Allowances for government rebates and discounts are established based on an estimated allocation of payers and the government-mandated discounts applicable to government-funded programs. Rebate discounts are included in accrued expenses in the accompanying consolidated balance sheets.

Commercial Rebates: We calculate the rebates we incur according to any contracts with certain commercial payers and deduct these amounts from our gross product sales at the time the revenues are recognized. Allowances for commercial rebates are established based on actual payer information, which is reasonably estimated at the time of delivery for applicable products. Rebate discounts are included in accrued expenses in the accompanying consolidated balance sheets.

Prompt Pay Discounts: We offer discounts to certain customers for prompt payments. We accrue for the calculated prompt pay discount based on the gross amount of each invoice for those customers at the time of sale.

Other Fees: We pay service fees to certain customers based on a contractually fixed percentage of the wholesale acquisition cost 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, we offer our customers a limited right to return product purchased directly from us, which is principally based upon the product's expiration date. Historically, returns have been immaterial.

Co-pay Assistance: We offer a co-pay assistance program, which is intended to provide financial assistance to qualified commercially insured patients with prescription drug co-payments required by 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.

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, we use judgement to evaluate whether the milestones are probable of being achieved and estimates the amount to include in the transaction price utilizing the most likely amount method. If it is probable that a significant revenue reversal will not occur, the estimated amount is included in the transaction price. Milestone payments that are not within our 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, we re-evaluate the probability of achievement of development milestones and any related constraint and adjust the estimate of the overall transaction price, if necessary. As of December 31, 2024, our evaluation concluded that all such milestones associated with our collaboration and licensing agreements remained constrained and therefore no adjustment to the respective transaction price was necessary. We recognize 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.

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

If the licenses to intellectual property are determined to be distinct from the other performance obligations identified in the arrangement, we recognize 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, we apply 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. We evaluate the measure of progress each reporting period and, if necessary, adjust 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. We generally utilize the cost-to-cost method of progress because it best measures the transfer of control to the customer which occurs as we incur 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. We use 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. We evaluate these cost estimates and the progress each reporting period and adjust the measure of progress, if necessary.

Changes in assumptions where management utilizes significant judgement could have a material impact on the revenue we recognize.

Clinical Trial Expenses

We record expenses in connection with our 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. We currently have four Phase 3 clinical trials in

process that are in varying stages of activity, with ongoing non-clinical support trials that are significant and changes in estimates could have a material impact on expenses we recognize.

Impairment of Intangible Assets subject to amortization

Intangible assets subject to amortization include certain license agreements and purchased technologies. Intangible assets subject to amortization are reviewed for impairment whenever events or circumstances indicate that the carrying amount of these assets may not be recoverable and are also reviewed annually to determine whether any impairment is necessary.

We are subject to generic competition, and if additional generic versions of Thiola and Thiola EC, any generic versions of FILSPARI following the expiration of patent or regulatory exclusivity for the product, or any of our current or future products, are approved, sales of that product likely would be negatively impacted, which could have a material adverse impact on the recoverability of certain related intangible assets. Generic versions of Thiola and Thiola EC have been approved, and these or additional generic versions of either formulation could have a material adverse impact on sales and the recoverability of the intangible assets depending on the timing of the market entry and the related impact on net sales.

Recently Issued Accounting Pronouncements

See Note 2 to the Consolidated Financial Statements for discussion.

Results of Operations

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

Revenue

For further background on our net product sales and license and collaboration revenue, see Revenue Recognition under our Critical Accounting Estimates.

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

	Year Ended December 31,		
	2024	2023	Change
FILSPARI	$ 132,222	$ 29,208	$ 103,014
Tiopronin products	94,485	98,329	(3,844)
Total net product sales	226,707	127,537	99,170
License and collaboration revenue	6,468	17,701	(11,233)
Total revenue	$ 233,175	$ 145,238	$ 87,937

Net product sales

The $99.2 million increase in total net product revenues for the year ended December 31, 2024 compared to the year ended December 31, 2023 was primarily due to growth in sales of FILSPARI, including a full twelve months of sales in 2024, following the February 2023 launch. The decrease in net sales of our tiopronin products was a driven by increased competition.

License and collaboration revenue

The decrease in license and collaboration revenue for the year ended December 31, 2024 compared to the year ended December 31, 2023 was primarily due to a $8.5 million decrease in collaboration revenue associated with the CSL Vifor License Agreement due to a decrease in amortization of deferred revenue, and the $3.3 million sale of active pharmaceutical ingredients to CSL Vifor in March 2023. We estimate that the remainder of the deferred revenue balance associated with these clinical development activities, $2.8 million, will be fully realized by mid-2025. We recognize costs for clinical development activities in research and development; costs related to sale of active pharmaceutical ingredients are recognized in cost of goods sold.

Operating Expenses

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

	Year Ended December 31,		
	2024	2023	Change
Cost of goods sold - product sales	$ 7,446	$ 8,406	$ (960)
Cost of goods sold - license and collaboration	298	3,044	(2,746)
Total cost of goods sold	7,744	11,450	(3,706)
Research and development	217,496	244,990	(27,494)
Selling, general and administrative	264,119	265,542	(1,423)
In-process research and development	65,205	—	65,205
Restructuring	2,438	11,394	(8,956)
Total operating expenses	$ 557,002	$ 533,376	$ 23,626

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 year ended December 31, 2024, sales of FILSPARI primarily consisted of zero-cost inventories, and therefore cost of goods sold did not increase proportionally to the increase in product sales. As of December 31, 2024, we had $2.3 million of zero-cost inventory remaining, the majority of which we expect will be consumed in 2025.

We began capitalizing inventory costs associated with FILSPARI (sparsentan) following the February 2023 approval for treatment in IgAN. At December 31, 2023, our evaluation of excess inventory and obsolescence considered certain minimum purchase obligations, which in combination with lower forecasted sales of FILSPARI resulted in a $3.2 million charge to cost of goods sold. The charge to cost of goods sold included a $2.1 million write-down of inventory balances and $1.1 million accrued for firm purchase commitments.

For the year ended December 31, 2024 compared to the year ended December 31, 2023, our cost of goods sold - license and collaboration decreased by $2.7 million, primarily due to the sale of active pharmaceutical ingredients to CSL Vifor in the first quarter of 2023.

Research and development expenses

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

We currently have four Phase 3 clinical trials in process that are in various stages of activity, with ongoing non-clinical support trials. As such, clinical trial expenses will vary depending on 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*):

	For the Year Ended December 31,		
	2024	2023	Change
External service provider costs:			
Sparsentan	$ 58,023	$ 91,702	$ (33,679)
Pegtibatinase	68,280	50,780	17,500
General and other product candidates	17,350	17,850	(500)
Total external service provider costs	143,653	160,332	(16,679)
Internal personnel costs	73,843	84,658	(10,815)
Total research and development	$ 217,496	$ 244,990	$ (27,494)

For the year ended December 31, 2024 compared to the year ended December 31, 2023, our research and development expenses decreased by $27.5 million. Internal personnel costs to support all programs decreased by $10.8 million, primarily as a result of restructuring initiatives. External service provider costs decreased by $16.7 million, which was largely driven by a decrease in costs associated with the development of sparsentan as our Phase 3 programs advance towards completion, offset by an increase in costs associated with the development of pegtibatinase following the December 2023 initiation of the Phase 3 HARMONY Study.

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.

In-process research and development expense

In March 2024, we recognized a non-recurring $65.2 million charge in in-process research and development (IPR&D) expense upon the achievement of a development milestone associated with our treatment candidate pegtibatinase, which was paid during the second quarter of 2024 and recorded within investing activities in the Consolidated Statements of Cash Flows. We acquired pegtibatinase 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 initiatives 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 $13.8 million recognized to date, $2.4 million was recognized during the year ended December 31, 2024, including $1.2 million related to impairment and disposal costs and initial direct costs to obtain a sublease. Restructuring costs were primarily comprised of one-time termination benefits, including severance, continuation of health insurance coverage, and other benefits for a specified period of time. In 2024, we recognized charges for impairment of operating lease right-of-use assets and related leasehold improvements, as well as disposal costs on furniture and fixtures associated with available office space that we decided to sublease as a result of the reduction in occupancy. As of December 31, 2024, we are no longer incurring restructuring expenses.

Other Income/Expenses

Other income/expenses consists of interest income and expense, finance expense and miscellaneous other income/expenses.

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

	Year Ended December 31,		
	2024	2023	Change
Interest income	$ 17,817	$ 21,768	$ (3,951)
Interest expense	(11,182)	(11,334)	152
Other (expense) income, net	(3,318)	1,594	(4,912)
Total other income (expense), net	$ 3,317	$ 12,028	$ (8,711)

The $8.7 million change in our total other income (expense), net for the year ended December 31, 2024 compared to the year ended December 31, 2023, is primarily attributable to a $4.0 million decrease in interest income in 2024. Additionally, for the year ended December 31, 2024, we recognized $3.4 million in other expense in connection with our equity investment in Renalys, related to its characterization as IPR&D as measured at inception.

Discontinued Operations

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

	Year Ended December 31,		
	2024	2023	Change
(Loss) income from discontinued operations, net of tax	$ (915)	$ 264,934	$ (265,849)

The $265.8 million change in (loss) income from discontinued operations, net of tax for the year ended December 31, 2024 compared to the year ended December 31, 2023 is primarily due to the August 31, 2023 sale of our bile acid business, which resulted in a gain, net of tax, of $226.0 million. The gain consists of net consideration, including the upfront payment and the deduction of investment banker fees owed upon the Closing, plus the derecognition of the carrying value of the net liabilities included in the transaction and the immaterial tax due on the sale.

See Note 19 to our 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.

We believe that our available cash and short-term investments as of the date of this filing, together with anticipated cash generated from operations, will be sufficient to fund our anticipated level of operations beyond the next 12 months from the date of this filing. We expect that our operating results will vary from quarter-to-quarter and year-to-year depending upon various factors including revenues, selling, general and administrative expenses, and research and development expenses, particularly with respect to our clinical and preclinical development activities. Our ability to fund our operations in subsequent years will depend upon certain factors which are beyond our control and may require us to obtain additional debt or equity capital or refinance all or a portion of our debt, including the 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.

For the years ended December 31, 2024 and 2023, we had the following balances and financial performance (*in thousands*):

	December 31, 2024	December 31, 2023
Cash and cash equivalents	$ 58,535	$ 58,176
Marketable debt securities, at fair value	$ 312,166	$ 508,675
Convertible debt	$ 378,988	$ 377,263
Accumulated deficit	$ (1,447,167)	$ (1,125,622)
Stockholders' equity	$ 59,077	$ 200,810
Net working capital*	$ 215,951	$ 438,867
Net working capital ratio**	2.08	3.47

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

As of December 31, 2024, we had cash and cash equivalents of $58.5 million and available-for-sale marketable debt securities of $312.2 million. Substantial sources of funds since the beginning of 2024, as summarized further below, include net proceeds of $134.7 million from an underwritten public offering of 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 and the anticipated repayment of the outstanding principal of approximately $68.9 million on the 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, with the potential for additional milestone payments depending on timing and outcomes that are currently uncertain.

Beyond the next 12 months and over the foreseeable future, our known commitments and potential financial obligations will likely include ongoing operations funding, operating lease payments, interest payments on our outstanding debt, royalties on sales of our existing commercialized products, research and development expenses pertaining to clinical and preclinical development activities across our pipeline, milestone and royalty payments associated with FILSPARI, pegtibatinase, and other developmental programs based upon the achievement of certain agreement-specific criteria, along with sales-based royalties and the repayment of principal on the outstanding 2029 Notes, which mature on September 1, 2029. Potential sources of cash over this time horizon may include net revenues from sales of our existing products and, if commercialized, our pipeline products, licensing revenue, the sale or maturity of marketable debt securities in our investment portfolio, the refinancing of all or a portion of our debt, on or before maturity, or the issuance of additional debt or equity. In addition, depending on prevailing market conditions, our liquidity requirements, contractual restrictions, and other factors, we may also from time to time seek to retire or purchase our outstanding debt through cash purchases and/or exchanges for equity securities, in open market purchases, privately negotiated transactions or otherwise. We may not be able to successfully conduct financing or refinancing activity on favorable terms or at all.

Purchase Agreement Proceeds

Sale of Bile Acid Product Portfolio

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

Collaboration and License Proceeds

License and Collaboration Agreement with CSL Vifor

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

See Note 4 to Consolidated Financial Statements for further discussion.

Licensing Agreement with Renalys

In January 2024, our license agreement with Renalys Pharma, Inc. came into effect. Under the terms of the agreement, we granted an exclusive license to Renalys for the 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. In addition, we received an option to purchase shares of common stock of Renalys (“Option Agreement”), which we exercised in January 2024. We also have 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”).

Equity Offerings

2024 Underwritten Public Offering of Common Stock

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

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 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 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. All of the pre-funded warrants were exercised in the third quarter of 2024, resulting in the issuance of 1.25 million shares of our common stock.

At-the-Market Equity Offering

In October 2024, we filed a prospectus supplement to the prospectus included in our registration statement on Form S-3 (File No. 333-281194), pursuant to which we may offer and sell, from time to time through Jefferies LLC, as agent (“Jefferies”), up to $100.0 million of our common stock pursuant to an Amended and Restated Open Market Sale Agreement ("ATM Agreement") with Jefferies dated October 2024. We did not sell any shares under the ATM Agreement during the year ended December 31, 2024.

Operating Leases

Future Minimum Rental Commitments

As of December 31, 2024, we have future minimum rental commitments totaling $25.4 million arising from our operating leases. These commitments represent the aggregate base rent through August 2028.

See Note 18 to 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 December 31, 2024, we have capitalized $47.2 million for contractual milestones achieved under the Ligand License Agreement, which includes a $5.8 million regulatory milestone payment to Ligand (and Bristol-Myers Squibb Company ("BMS")) in the second quarter of 2024. Following commercialization of sparsentan or any products containing related compounds, we are obligated to pay to Ligand an escalating royalty between 15% and 17% of net sales 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 year ended December 31, 2024, we capitalized $20.3 million 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.

Mission License Agreement

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

See Note 9 to Consolidated Financial Statements for further discussion.

Acquisition of Orphan Technologies Limited

In November 2020, we completed the acquisition of Orphan Technologies Limited (“Orphan”), including Orphan’s rare metabolic disorder drug pegtibatinase. We acquired Orphan by purchasing all of its outstanding shares. Under the Stock Purchase Agreement ("the Agreement"), we agreed to make contingent cash payments up to an aggregate of $427.0 million based on the achievement of certain development, regulatory and commercialization events as set forth in the Agreement, as well as additional tiered mid-single digit royalty payments based upon future net sales of any pegtibatinase products in the U.S. and Europe, subject to certain reductions as set forth in the Agreement, and a contingent payment in the event a pediatric rare disease voucher for any pegtibatinase product is granted. We made a $65.0 million payment to Orphan in the second quarter of 2024 following the achievement of a development milestone.

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 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 expected 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 sell the shares back for a nominal payment at any time upon 60 days' notice, subject to survival of contingent obligations, if any.

See Note 5 to Consolidated Financial Statements for further discussion.

French Rebate Accrual

In October 2021, our distributor in France for our previously marketed product Kolbam informed us that they had received a notice that the price previously paid for Kolbam during its period on the market in France had been recalculated by the agency responsible for pharmaceutical pricing in France. As of December 31, 2023, $5.4 million for estimated amounts to be repaid was recorded in Accrued Expenses in the Consolidated Balance Sheets. In October 2024, we received an invoice from the government authority in the amount of $6.2 million (€5.6 million) for reimbursement of amounts previously paid for Kolbam, which we paid in November 2024. We have appealed the pricing decision and will pursue an appeal of the amount paid with the Competent Administrative Court.

Borrowings

Convertible Senior Notes Due 2029

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

Convertible Senior Notes Due 2025

On September 10, 2018, we completed a registered underwritten public offering of $276.0 million aggregate principal amount of 2.50% Convertible Senior Notes due 2025 ("2025 Notes"), and entered into a base indenture and supplemental indenture agreement ("2025 Indenture") with respect to the 2025 Notes. The 2025 Notes will mature on September 15, 2025, 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 December 31, 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.

See Note 7 to Consolidated Financial Statements for further discussion.

Funding Requirements

We believe that our available cash and short-term investments as of the date of this filing will be sufficient to fund our anticipated level of operations beyond the next 12 months from the date of this filing. We expect to use cash flows from operations and, when necessary, outside financings, to meet our current and future financial obligations, including funding our operations, debt service and capital expenditures. Our ability to make these payments depends on our future performance, which will be affected by financial, business, economic, regulatory and other factors, many of which we cannot control. Factors that may affect financing requirements include, but are not limited to:

- the timing, progress, cost and results of our clinical trials, preclinical studies and other discovery and research and development activities;
- the timing and outcome of, and costs involved in, seeking and obtaining marketing approvals for our products, and in maintaining quality systems standards for our products;
- the timing of, and costs involved in, commercial activities, including product marketing, sales and distribution;
- our ability to successfully commercialize FILSPARI for the treatment of IgAN, and to obtain regulatory approval for, and successfully commercialize, sparsentan for FSGS and our other or future product candidates;
- increases or decreases in revenue from our marketed products, including decreases in revenue resulting from generic entrants or health epidemics or pandemics;
- 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

The following table summarizes our cash flows for the periods set forth below (*in thousands*):

	Year Ended December 31,		
	2024	2023	2022
Net cash used in operating activities - continuing operations	$ (230,024)	$ (325,357)	$ (260,846)
Net cash provided by (used in) investing activities - continuing operations	99,325	(151,626)	(32,553)
Net cash provided by financing activities - continuing operations	139,422	220,134	120,052
Cash flows from continuing operations	8,723	(256,849)	(173,347)
Cash flows from discontinued operations	(7,451)	251,356	72,076
Effect of exchange rate changes on cash	(913)	1,981	(2,794)
Net increase (decrease) in cash and cash equivalents	359	(3,512)	(104,065)
Cash and cash equivalents, beginning of year	58,176	61,688	165,753
Cash and cash equivalents, end of year	58,535	58,176	61,688
Marketable debt securities, at fair value	312,166	508,675	388,557
Total cash and cash equivalents and marketable debt securities	**$ 370,701**	**$ 566,851**	**$ 450,245**

Management considers marketable debt securities to be available to fund current operations, and they are classified as available for sale and included within current assets in our Consolidated Balance Sheets. Therefore, cash and short-term investments available to fund operations is $370.7 million as of December 31, 2024.

Cash Flows from Operating Activities

Cash used in operating activities from continuing operations for the year ended December 31, 2024 was $230.0 million compared to cash used of $325.4 million for the year ended December 31, 2023. The decrease in cash used was due to a $99.2 million increase in total net product sales, along with 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 year ended December 31, 2024 was $99.3 million compared to cash provided of $151.6 million for the year ended December 31, 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 year ended December 31, 2024 was $139.4 million compared to cash provided of $220.1 million for the year ended December 31, 2023. The change was due to the November 2024 issuance of common stock through an underwritten public offering that provided $134.7 million in net proceeds, compared to $215.8 million in net proceeds from the March 2023 issuance of common stock and pre-funded warrants.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Our primary exposure to market risk is related to changes in interest rates. As of December 31, 2024, we had cash equivalents and marketable debt securities of approximately $370.7 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 $1.7 million impact on our investments.

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

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

Inflation generally affects us by increasing our salaries and fees paid to third-party contract service providers. 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 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

The Consolidated Financial Statements and supplementary data of Travere Therapeutics, Inc. required by this Item are described in Item 15 of this Annual Report on Form 10-K and are presented beginning on page F-1.

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE

None.

ITEM 9A. CONTROLS AND PROCEDURES

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in our Exchange Act reports 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 year 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.

Management's Annual Report on Internal Control Over Financial Reporting

Internal control over financial reporting refers to the process designed by, or under the supervision of, our Chief Executive Officer and Chief Financial Officer, and effected by our board of directors, management and other personnel, 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, and includes those policies and procedures that:

(1) Pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of our assets;

(2) Provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that our receipts and expenditures are being made only in accordance with authorization of our management and directors; and

(3) Provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of our assets that could have a material effect on the financial statements.

Internal control over financial reporting cannot provide absolute assurance of achieving financial reporting objectives because of its inherent limitations. Internal control over financial reporting is a process that involves human diligence and compliance and is subject to lapses in judgment

and breakdowns resulting from human failures. Internal control over financial reporting also can be circumvented by collusion or improper management override. Because of such limitations, there is a risk that material misstatements may not be prevented or detected on a timely basis by internal control over financial reporting. However, these inherent limitations are known features of the financial reporting process. Therefore, it is possible to design into the process safeguards to reduce, though not eliminate, this risk. Management is responsible for establishing and maintaining adequate internal control over financial reporting for the company.

Management has used the framework set forth in the report entitled *Internal Control-Integrated Framework (2013 framework)* published by the Committee of Sponsoring Organizations of the Treadway Commission, known as COSO, to evaluate the effectiveness of our internal control over financial reporting. Based on this assessment, our Chief Executive Officer and Chief Financial Officer concluded that our internal control over financial reporting was effective as of December 31, 2024. Ernst & Young LLP ("EY"), our independent registered public accounting firm, has issued an attestation report on our internal control over financial reporting as of December 31, 2024, which is included herein.

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 fourth quarter of 2024 and that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting. There have not been any changes in our internal control over financial reporting during the quarter ended December 31, 2024 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Travere Therapeutics, Inc.

Opinion on Internal Control over Financial Reporting

We have audited Travere Therapeutics, Inc. and subsidiaries' internal control over financial reporting as of December 31, 2024, based on criteria established in Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework) (the COSO criteria). In our opinion, Travere Therapeutics, Inc. and subsidiaries (the Company) maintained, in all material respects, effective internal control over financial reporting as of December 31, 2024, based on the COSO criteria.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the consolidated balance sheets of the Company as of December 31, 2024 and 2023, the related consolidated statements of operations and comprehensive loss, stockholders' equity and cash flows for each of the two years in the period ended December 31, 2024 and the related notes and our report dated February 20, 2025 expressed an unqualified opinion thereon.

Basis for Opinion

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting included in the accompanying Management's Annual Report on Internal Control Over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects.

Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

Definition and Limitations of Internal Control over Financial Reporting

A company's internal control over financial reporting is a process designed 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. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

/s/ Ernst & Young LLP

San Diego, California

February 20, 2025

ITEM 9B. OTHER INFORMATION

Trading Arrangements

During the fiscal quarter ended December 31, 2024, our directors and/or officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted, modified or terminated the "Rule 10b5-1 trading arrangements" or "non-Rule 10b5-1 trading arrangements," as those terms are 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)
Timothy Coughlin, member of our Board of Directors	November 15, 2024	Rule 10b5-1 Trading Arrangement	Up to 40,000 shares (3)	March 31, 2025
Gary Lyons, chair of our Board of Directors	December 17, 2024	Rule 10b5-1 Trading Arrangement	Up to 8,000 shares (4)	June 6, 2025
Jeffrey Meckler, member of our Board of Directors	December 17, 2024	Rule 10b5-1 Trading Arrangement	Up to 8,000 shares (4)	June 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 March 2025.

4 Consists of shares of underlying stock options expiring in June 2025.

Trading Arrangements Modified or Terminated:

None.

ITEM 9C. DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS

Not applicable.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

Information required by this item and not included below will be contained under the captions "Election of Directors" and "Information Regarding the Board of Directors and Corporate Governance", in our Definitive Proxy Statement for our 2025 Annual Meeting of Stockholders, to be filed pursuant to Regulation 14A with the Securities and Exchange Commission within 120 days of December 31, 2024 (the "Proxy Statement"). Such information is incorporated herein by reference.

Our Board of Directors consists of the following members:

Roy D. Baynes, M.D., Ph.D. has served as a director of the Company since June 2016. Since July 2022, Dr. Baynes has served as Executive Vice President and Chief Medical Officer of Eikon Therapeutics, Inc., a privately-held biotechnology company. Prior to Eikon Therapeutics, Inc., until April 2022 he served as Senior Vice President and Head of Global Clinical Development at Merck Research Laboratories, the research division of Merck and Co., Inc., commencing in December 2013 and as Chief Medical Officer of Merck and Co, Inc., a global healthcare company, commencing in July 2016. Prior to his roles at Merck, Dr. Baynes served as Senior Vice President of Oncology, Inflammation and Respiratory Therapeutics at Gilead Sciences, Inc., a biopharmaceutical company, from January 2012 to December 2013. Prior to Gilead, Dr. Baynes held positions of increasing responsibility at Amgen Inc., a biotechnology company, including Vice President of Global Clinical Development and Therapeutic Area Head for Hematology/Oncology. Before joining Amgen, Dr. Baynes was the Charles Martin Professor of Cancer Research at the Barbara Ann Karmanos Cancer Institute, a National Cancer Institute-designated Comprehensive Cancer Center, at Wayne State University. Dr. Baynes has authored more than 150 publications and is a member or fellow of several international medical societies. Dr. Baynes currently serves on the Board of Directors of Natera, Inc., a genetic testing and diagnostics company, and on the Board of Directors of Aardvark Therapeutics, Inc., a biopharmaceutical company. Dr. Baynes also currently serves on the Board of Directors of CatalYm GmbH, a privately-held Germany based biotechnology company, and on the Board of Directors of Adcendo, a privately held Denmark based biotechnology company. Previously he served on the Board of Directors of Atara Biotherapeutics, Inc., a T-cell immunotherapy company. Dr. Baynes received his medical degree and doctorate in philosophy from the University of the Witwatersrand in South Africa, and completed his medical training in the Department of Hematology and Oncology at Johannesburg Hospital.

Suzanne Bruhn, Ph.D. has served as a director of the Company since April 2020. Since November 2023, Dr. Bruhn has served as Chief Executive Officer of The Charcot-Marie-Tooth Association (CMTA), a nonprofit patient advocacy organization dedicated to finding a cure for CMT, a rare, debilitating peripheral neuropathy. Previously, from May 2019 to December 2023, Dr. Bruhn served as the President and Chief Executive Officer of Tiaki Therapeutics Inc., a biotechnology company. Dr. Bruhn served as President and Chief Executive Officer of Proclara Biosciences, Inc., a biotechnology company, from April 2017 to September 2018, and as President and Chief Executive Officer of Promedior, Inc., a biotechnology company, from May 2012 to November 2015. Currently, Dr. Bruhn serves on the Boards of Directors of Pliant Therapeutics, Inc., Vigil Neuroscience, Inc., and Mind Medicine Inc. (MindMed). Previously, Dr. Bruhn served as a member of the Board of Directors of Raptor Pharmaceuticals Corp., a pharmaceutical company, from April 2011 until it was acquired by Horizon Pharma plc in October 2016, as a member of the Board of Directors of Novelion Therapeutics, Inc., a biopharmaceutical company, from October 2017 to January 2020, as a member of the Board of Directors of Aeglea BioTherapeutics, Inc., a clinical stage biotechnology company, from February 2017 to August 2020, and on the Board of Directors of Avalo Therapeutics, Inc. (formerly Cerecor, Inc.), a biopharmaceutical company from April 2020 to November 2021. Earlier in her career Dr. Bruhn served in roles of increasing responsibility at Shire Human Genetic Therapies (formerly Transkaryotic Therapies), including Senior Vice President, Strategic Planning and Program Management. Dr. Bruhn received her B.S. degree in Chemistry from Iowa State University and her Ph.D. in Chemistry from Massachusetts Institute of Technology.

Timothy Coughlin has served as a director of the Company since March 2015. Mr. Coughlin is the former Chief Financial Officer of Neurocrine Biosciences, Inc., a biopharmaceutical company that received FDA approval for INGREZZA® (valbenazine) and ORILISSA® (elagolix), both of which were discovered, developed and commercially launched during his tenure at Neurocrine from 2002 to 2018. Mr. Coughlin currently serves on the board of directors of Fate Therapeutics, Inc., and as the Chair of the Board of Directors of aTyr Pharma, Inc., both biotechnology companies, and previously served on the Board of Directors of Peloton Therapeutics, Inc. prior to its sale to Merck in 2019. Prior to joining Neurocrine, he was with Catholic Health Initiatives, a nationwide integrated healthcare delivery system, where he served as Vice President, Financial Services. Earlier in his career Mr. Coughlin served as a Senior Manager in the Health Sciences practice of Ernst & Young LLP and its predecessors. Mr. Coughlin holds a master's degree in international business from San Diego State University and a bachelor’s degree in accounting from Temple University. Mr. Coughlin is a certified public accountant in both California and Pennsylvania.

Eric Dube, Ph.D. has served as President and Chief Executive Officer of the Company and as a member of our board of directors since January 2019. Previously, Dr. Dube served as the Head of North America of ViiV Healthcare Limited, a pharmaceuticals company, since January 2018. From June 2015 to December 2017, Dr. Dube served as Sr. Vice President and Head, Global Respiratory Franchise, of GlaxoSmithKline Pharmaceuticals plc (“GSK”), a pharmaceutical company. From February 2013 to May 2015, Dr. Dube served as Senior Vice President and Business Unit Head, Respiratory Japan of GSK. Earlier in his career, Dr. Dube held positions of increasing responsibility at GSK including senior

leadership roles in Strategy, Planning & Operations, Oncology, Managed Markets and Marketing. Dr. Dube currently serves on the Board of Directors for the Biotechnology Innovation Organization (BIO). Previously, Dr. Dube served on the Board of Trustees for AIDS United, on the Board of Directors for Biocom California, and on the Board of Directors for Reneo Pharmaceuticals, Inc. Dr. Dube holds a B.S. from Santa Clara University and a M.A. and Ph.D. from Cornell University.

Gary Lyons has served as a director of the Company since October 2014 and Chair of the Company since May 2016. Mr. Lyons was the founding President and Chief Executive Officer of Neurocrine Biosciences, Inc. and he remains a member of its Board of Directors. Prior to joining Neurocrine, Mr. Lyons held a number of senior management positions at Genentech, Inc., including Vice President of Business Development and Vice President of Sales. Previously, Mr. Lyons served on the Board of Directors of Eledon Pharmaceuticals, Inc. (formerly Novus Therapeutics, Inc.), from May 2017 to June 2023, on the Board of Directors of Fresh Tracks Therapeutics, Inc. (formerly known as Brickell Biotech, Inc.) from August 2019 to September 2023 and on the Board or Directors of Rigel Pharmaceuticals, Inc. from October 2005 to May 2024. Mr. Lyons is also a Senior Advisor for HealthCare Royalty Partners. Mr. Lyons holds a B.A. in Marine Biology from the University of New Hampshire and an M.B.A. from Northwestern University's J.L. Kellogg Graduate School of Management.

Jeffrey Meckler has served as a director of the Company since October 2014. Since August 2021, Mr. Meckler has served as Chief Executive Officer of Indaptus Therapeutics, Inc. (formerly Intec Inc.), a biopharmaceutical company. From April 2017 to August 2021, Mr. Meckler served as Chief Executive Officer and Vice Chair of the Board of Intec Pharma, Ltd. He served as Chief Executive Officer and a director of Cocrystal Pharma, Inc., a pharmaceutical company, from April 2015 to July 2016, as a director of QLT, Inc., an ultra-orphan ophthalmic biotechnology company based in Canada, from June 2012 to November 2016, as well as the Managing Director of The Andra Group, a life sciences consulting firm. Previously, Mr. Meckler acted as a director of several biopharmaceutical companies and medical device companies. Earlier in his career, Mr. Meckler held a series of positions at Pfizer Inc. in Manufacturing Systems, Market Research, Business Development, Strategic Planning and Corporate Finance, which included playing a significant role in acquisitions and divestitures. Mr. Meckler is the past President and continues to serve on the board of Children of Bellevue, a non-profit organization focused on advocating and developing pediatric programs at Bellevue Hospital Center. Mr. Meckler holds a B.S. in Industrial Management and M.S. in Industrial Administration from Carnegie Mellon University. In addition, Mr. Meckler received a J.D. from Fordham University School of Law.

John A. Orwin has served as a director of the Company since March 2017. From April 2018 to June 2024, Mr. Orwin served as the President and Chief Executive Officer of Atreca, Inc., a biopharmaceutical company. From June 2013 through June 2017 he served as Chief Executive Officer of Relypsa, Inc., and from June 2013 through March 2017 also served as its President and on its board of directors from June 2013 until Relypsa's acquisition by the Galenica Group in September 2016. Prior to Relypsa, Mr. Orwin served as President and Chief Operating Officer of Affymax, Inc., a biotechnology company, from April 2010 to January 2011, and as Affymax's Chief Executive Officer and a member of the board of directors from February 2011 to May 2013. Earlier in his career he served as Vice President and then Senior Vice President of the BioOncology Business Unit at Genentech, Inc. (now a member of the Roche Group), and served in various executive-level positions at Johnson & Johnson. Prior to such roles, Mr. Orwin held senior marketing and sales positions at various life sciences and pharmaceutical companies, including Alza Corporation (acquired by Johnson & Johnson), SangStat Medical Corporation (acquired by Genzyme), Rhone-Poulenc Rorer Pharmaceuticals, Inc. (merged with Sanofi-Aventis) and Schering-Plough Corporation (merged with Merck). Mr. Orwin currently serves as the Chair of the Board of Directors of CARGO Therapeutics, Inc., a biotechnology company, and as the Chair of the Board of Directors for AnaptysBio, Inc., a clinical-stage biotechnology company. Additionally, Mr. Orwin currently serves as the Chair of the Board of Directors of the privately held company Nested Therapeutics, on the Board of Directors of the privately held company Ambrosia Biosciences, Inc., and as the Executive Chair of the Board of Directors of the privately held company Agni Bio, Inc., and is a Venture Partner in Samsara BioCapital. Previously, Mr. Orwin served as a member of the Board of Directors of Seagen Inc. from January 2014 until its acquisition by Pfizer in December of 2023, the board of directors of NeurogesX, Inc., and on the board of directors of Array BioPharma Inc. from November 2012 until its acquisition by Pfizer in July of 2019. Mr. Orwin received a B.A. in Economics from Rutgers University and an M.B.A. from New York University.

Sandra Poole has served as a director of the Company since May 2019. Since July 2020, Ms. Poole has served as the Chief Operating Officer of Mythic Therapeutics, a clinical stage biotechnology company advancing antibody-drug conjugates (ADCs) for cancer therapy. Ms. Poole has deep expertise in product development, technical operations, company building, operations and strategy from a greater than 25-year career in the biopharmaceutical industry. She is the former Chief Operating Officer at Candel Therapeutics, a biotechnology company focused on developing viral immunotherapies, where she served from January 2020 to March 2020. Prior to Candel Therapeutics she served as Chief Operating Officer at LogicBio Therapeutics Inc. (acquired by Alexion), a company focused on developing genetic medicines for rare diseases. Prior to LogicBio, Ms. Poole served in executive leadership roles of increasing responsibility at ImmunoGen, Inc. (acquired by AbbVie Inc.), a company focused on developing ADC therapies, where she most recently served as executive Vice President of Technical Operations and Commercial Development. Earlier in her career, Ms. Poole spent more than 15 years in CMC product development and manufacturing leadership positions at Genzyme (now Sanofi), most recently serving as SVP of Biologics Manufacturing, managing global biomanufacturing of six commercial therapies for rare disease across five manufacturing sites in the US and EU including Cerezyme®, Fabrazyme®, and Myozyme®/Lumizyme®. Previously, Ms. Poole served on the Supervisory Board for Valneva, SE a France based biotechnology company developing vaccines for infectious diseases, and on the Board of Directors of ViaCyte, a privately held biotechnology company developing novel stem cell-derived cell replacement therapies until its acquisition by Vertex Pharmaceuticals Incorporated in July of 2022. Ms. Poole holds an M.A.Sc. and a B.A.Sc. in chemical engineering from the University of Waterloo (Ontario, Canada).

Ron Squarer has served as a director of the Company since April 2017. Since March 2020, Mr. Squarer has served as Chair of the Board of Directors of ADC Therapeutics SA, a commercial-stage biopharmaceutical company. Previously, from June 2023 to June 2024, Mr. Squarer served as Chair of the Board of Directors of Deciphera Pharmaceuticals, Inc., a commercial-stage biopharmaceutical company, where he had also served as a Director since December 2019, through its acquisition by Ono Pharmaceuticals Co., Ltd. for approximately $2.5 billion. Mr. Squarer has extensive commercial, development and executive leadership expertise from a greater than 25-year career in the pharmaceutical industry. Previously, Mr. Squarer served as the Chief Executive Officer and a member of the Board of Directors of Array BioPharma, Inc., an oncology focused biopharmaceutical company from April 2012 to July 2019, when Array BioPharma, Inc. was acquired by Pfizer, Inc. at an enterprise value of approximately $11.4 billion. Prior to this, Mr. Squarer held positions of increasing responsibility with Hospira Inc., a global pharmaceutical and medical device company, including serving as Senior Vice President, Chief Commercial Officer, where he was responsible for delivering $4 billion in annual revenue and leading more than 2,000 employees worldwide. Mr. Squarer joined Hospira from Mayne Pharma, an oncology-focused, global pharmaceutical company, where he served as Senior Vice President, Global Corporate and Business Development when Mayne was sold to Hospira for $2 billion in 2007. Prior to Mayne Pharma, Mr. Squarer held senior management roles at both Pfizer, Inc., focused on global oncology commercial development, and at SmithKline Beecham Pharmaceuticals (now GlaxoSmithKline) in the U.S. and Europe. Mr. Squarer holds an MBA from the Kellogg School of Management, Northwestern University and a bachelor's degree in biochemistry from the University of California, Berkeley.

Ruth Williams-Brinkley has served as a director of the Company since September 2021. In January 2024, Ms. Williams-Brinkley retired as President of the Kaiser Foundation Health Plan of the Mid-Atlantic States, where she led all of Kaiser Permanente's care delivery and health plan operations in Washington, D.C., suburban Maryland, Baltimore, and Northern Virginia since June 2020. She joined Kaiser Permanente in 2017 as President of Kaiser Foundation Health Plan and Hospitals of the Northwest, in Portland, Oregon. Prior to joining Kaiser, Ms. Williams-Brinkley served as CEO of KentuckyOne Health, an affiliate of CommonSpirit Health, from 2011 to 2017, as President and CEO of Carondelet Health Network in Tucson, Arizona, an affiliate of Ascension Health, from 2008 to 2011, and as President and CEO of Memorial Health Care System of Chattanooga, Tennessee, an affiliate of CommonSpirit Health, from 2002 to 2008. Currently Ms. Williams-Brinkley serves on the Board of Directors of Natera, Inc., on the Board of Trustees of the University of Phoenix, and on the Board of Directors of the privately held companies OOTify, Inc. and Swan AI Studios. Additionally, Ms. Williams-Brinkley currently serves on the not for profit Boards of DePaul University in Chicago, Illinois, Allina Health and The Leverage Network. Previously, she served as a member of the Board of Directors of Results Physiotherapy, a private care delivery company until it was acquired by Upstream Rehabilitation in 2021, and Chattem, Inc. until it was acquired by Sanofi in 2009. Earlier in her career, Ms. Williams-Brinkley held various nursing staff and management roles of increasing responsibility. Ms. Williams-Brinkley received her B.S. and Master of Science in Nursing from DePaul University and is a Life Fellow of the American College of Healthcare Executives.

In addition to Dr. Dube, our executive officers are as follows:

Christopher Cline has served as the Chief Financial Officer of the Company since August 2022. Mr. Cline brings more than 15 years of industry experience in investor relations, corporate communications, and financial strategy, planning and analysis to the chief financial officer position. Previously, Mr. Cline served as senior vice president, investor relations and corporate communications at the Company. Since joining the Company in 2014, Mr. Cline has been responsible for leading engagement with the investment community, as well as building a developed corporate communications infrastructure and strategy. Prior to the Company, Mr. Cline was a member of the global investor relations group at Elan Corporation, plc, and the financial planning and analysis group at Phase Forward. Mr. Cline is a CFA charter holder and holds a degree in finance from the Williams College of Business at Xavier University.

Peter Heerma has served as Chief Commercial Officer of the Company since October 2019. Previously, Mr. Heerma served as Global Product General Manager for oncology and cardiovascular products at Amgen Inc., a biotechnology company, from December 2015 to September 2019. From December 2003 until November 2015, Mr. Heerma held roles of increasing responsibility at Abbott Laboratories ("Abbott"), and following Abbott's spin-off of AbbVie, Inc., a biopharmaceutical company, at AbbVie. These roles included Senior Director of Portfolio Strategy for hepatology and nephrology, Senior Director and Asset Team Lead for HCV, diabetic nephropathy, and neuroscience development projects (all AbbVie), Director of Commercial Strategy Renal Care, International Marketing Director, Business Unit Manager of hospital products, and Product Manager for obesity and cardiovascular products (all Abbott). Mr. Heerma holds a Master of Science in European business administration and business law from the Lund University in Sweden and a Bachelor of Science in retail management and marketing from Stenden University in the Netherlands.

Jula Inrig has served as Chief Medical Officer of the Company since January 2022. Previously, Dr. Inrig served as Global Head of the Renal Center of Excellence at IQVIA, a global provider of analytics, technology solutions, and clinical research services to the life sciences industry, from September 2017 to December 2021, where she helped develop the design, execution and strategy of clinical trials leading to FDA and European Commission approvals in autosomal dominant polycystic kidney disease (ADPKD), and diabetic kidney disease. From August 2012 to March 2015 Dr. Inrig served as Medical Director, and then as Senior Medical Director from April 2015 to December 2021 and was responsible for the execution of numerous global clinical trials, including pivotal phase 3 trials in FSGS, IgAN and lupus nephritis. From March 2013 to January 2018 Dr. Inrig served on the board of directors for the Kidney Health Initiative, a public-private partnership with the FDA, working to improve the development of therapies for patients with kidney disease. Dr. Inrig has authored or co-authored over 50 peer-reviewed publications and editorials and is currently a member of several professional medical societies. Dr. Inrig is board certified in nephrology and internal medicine and has served on the faculty at the University of California, Irvine, and as an adjunct in the Department of Medicine at the Duke University School of Medicine. Dr. Inrig holds a B.A. from California State University, Sacramento, and received her M.D. from Loma Linda University and completed her internal medicine residency, her nephrology fellowship and Masters of Health Science at Duke University.

Elizabeth E. Reed has served as Senior Vice President, General Counsel and Corporate Secretary of the Company since January 2017. Previously, Ms. Reed served as Vice President, General Counsel and Secretary of Celladon Corporation, a publicly traded biotechnology company, from June 2014 to March 2016 and served as a legal consultant for companies in the life sciences industry from 2013 to June 2014 and again during 2016. From 2001 to 2012, Ms. Reed led the legal function at Anadys Pharmaceuticals, Inc., a publicly traded biopharmaceutical company, including serving as Senior Vice President, Legal Affairs, General Counsel and Corporate Secretary until Anadys' acquisition by Roche. Prior to Anadys, Ms. Reed was an attorney with the law firms Cooley LLP and Brobeck, Phleger & Harrison LLP. Ms. Reed is a member of the State Bar of California and received her B.S. in Business Administration from the Haas School of Business at the University of California, Berkeley and holds a J.D., cum laude, from Harvard Law School.

William E. Rote has served as Senior Vice President of Research & Development of the Company since February 2017. Previously, Dr. Rote led clinical development at Ardea Biosciences, a wholly owned subsidiary of AstraZeneca, serving as Vice President, Clinical Development from September 2014 to July 2016. From 2003 to 2014, Dr. Rote held numerous positions of increasing responsibility at Amylin Pharmaceuticals, Inc., a biopharmaceutical company, including Vice President, Site Head for Research & Development from September 2012 to July 2014, Vice President, Research & Product Development from January 2010 to September 2012, and Vice President, Corporate Development, New Ventures, from 2007 to 2010, among others. Prior to Amylin, Dr. Rote served as Executive Director, Development of Corvas International, a biopharmaceutical company. He earned both his Ph.D. in Pharmacology and B.S. in Pre-Medicine from Pennsylvania State University and received postdoctoral training from the University of Michigan.

We have adopted a Code of Business Conduct that applies to our directors and employees (including our principal executive officer, principal financial officer, principal accounting officer and controller), and have posted the text of the policy on our website (https://ir.travere.com/governance-documents). In addition, we intend to promptly disclose on our website in the future (i) the date and nature of any amendment (other than technical, administrative or other non-substantive amendments) to the policy that applies to our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions and relates to any element of the code of ethics definition enumerated in Item 406(b) of Regulation S-K, and (ii) the nature of any waiver, including an implicit waiver, from a provision of the policy that is granted to one of these specified individuals that relates to one or more of the elements of the code of ethics definition enumerated in Item 406(b) of Regulation S-K, the name of such person who is granted the waiver and the date of the waiver.

We have adopted insider trading policies and procedures governing the purchase, sale, and other dispositions of our securities by our directors, officers and employees, as well as Travere itself, that are reasonably designed to promote compliance with insider trading laws, rules and regulations, and listing standards applicable to Travere.

ITEM 11. EXECUTIVE COMPENSATION

Information required by this item will be contained under the captions "Compensation Discussion and Analysis," "Executive Compensation," "Director Compensation Summary" and "Compensation Committee Interlocks and Insider Participation" in the Proxy Statement. Such information is incorporated herein by reference.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

Information required by this item will be contained under the captions "Security Ownership of Certain Beneficial Owners and Management" and "Securities Authorized for Issuance Under Equity Compensation Plans" in the Proxy Statement. Such information is incorporated herein by reference.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE

Information required by this item will be contained under the caption "Transaction With Related Persons" and "Information Regarding the Board of Directors and Corporate Governance" in the Proxy Statement. Such information is incorporated herein by reference.

ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES

Information required by this item will be contained under the caption "Principal Accountant Fees and Services" in the Proxy Statement. Such information is incorporated herein by reference.

PART IV

ITEM 15. EXHIBITS, FINANCIAL STATEMENT SCHEDULES

(a) The financial statements at page F-1 are incorporated by reference to a part of this Annual Report on Form 10-K.

Financial statement schedules are omitted because they are not applicable or the required information is shown in the financial statements or notes thereto.

(b) Exhibits: The exhibits to this report are listed in the exhibit index below.

Exhibit No.	Description
2.1*	Asset Purchase Agreement, dated January 10, 2015, by and between the Company and Asklepion Pharmaceuticals, LLC (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on August 3, 2023).
2.2*	Stock Purchase Agreement, dated October 21, 2020, by and among the Company, Orphan Technologies Limited and Citco Trustees (Cayman) Limited acting solely in its capacity as the sole trustee of The Fuhrer Family Trust (incorporated by reference to Exhibit 2.1 to the Company's Current Report on Form 8-K, filed with the SEC on November 18, 2020).
2.3ǂ*	Asset Purchase Agreement, dated July 16, 2023, by and between Mirum Therapeutics, Inc. and the Company (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on July 17, 2023).
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 to the 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).
4.1	Reference is made to Exhibits to 3.1, 3.2, 3.3, 3.4, 3.5 and 3.6.
4.2	Description of Common Stock (incorporated by reference to Exhibit 4.2 to the Company's Annual Report on Form 10-K for the year ended December 31, 2021, filed with the SEC on February 24, 2022).
4.3	Base Indenture, dated September 10, 2018, between the Company and U.S. Bank National Association, as Trustee (incorporated by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K, filed with the SEC on September 10, 2018).
4.4	First Supplemental Indenture, dated September 10, 2018, between the Company and U.S. Bank National Association, as Trustee (including the form of 2.50% Convertible Senior Note due 2025) (incorporated by reference to Exhibit 4.2 to the Company's Current Report on Form 8-K, filed with the SEC on September 10, 2018).
4.5	Second Supplemental Indenture, dated March 11, 2022, between the Company and U.S. Bank Trust Company, National Association, as Trustee (including the form of 2.25% Convertible Senior Note due 2029) (incorporated by reference to Exhibit 4.2 to the Company's Current Report on Form 8-K, filed with the SEC on March 11, 2022).
10.1	Form of Indemnity Agreement (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 1, 2018).
10.2†	Employment Agreement, dated January 4, 2019, by and between the Company and Eric M. Dube (incorporated by reference to Exhibit 10.34 to the Company's Annual Report on Form 10-K, filed with the SEC on February 26, 2019).
10.3†	Employment Agreement, effective September 1, 2022, between the Company and Christopher Cline (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K/A, filed with the SEC on August 17, 2022).
10.4†	Employment Agreement, dated February 13, 2017, and Amendment to Employment Agreement, dated April 11, 2017, by and between the Company and William Rote (incorporated by reference to Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 1, 2018)
10.5†	Employment Agreement, dated February 6, 2017, and Amendment to Employment Agreement, dated April 11, 2017, by and between the Company and Elizabeth E. Reed (incorporated by reference to Exhibit 10.4 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 1, 2018)
10.6†	Employment Agreement, dated October 1, 2019, by and between the Company and Peter Heerma (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 5, 2020).
10.7†	Employment Agreement, effective January 1, 2022, between the Company and Jula Inrig, M.D. (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 4, 2023).
10.8†	Non-Employee Director Compensation Program, as amended (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on May 6, 2024).

10.9†	The Company's 2025 Executive Officer Annual Bonus Plan.
10.10†	The Company's 2014 Incentive Compensation Plan as amended (incorporated by reference to Exhibit 99.1 to the Company's Current Report on Form 8-K, filed with the SEC on February 9, 2015).
10.11†	The Company's 2015 Equity Incentive Plan, as amended (incorporated by reference to Exhibit 99.1 to the Company's Current Report on Form 8-K, filed with the SEC on May 18, 2017).
10.12†	Form of Stock Option Grant Notice, Option Agreement and Notice of Exercise for Inducement Grant Outside of 2015 Equity Incentive Plan (incorporated by reference to Exhibit 99.3 to the Company's current report on Form S-8, filed with the SEC on June 8, 2017).
10.13†	Form of Restricted Stock Unit Grant Notice and Restricted Stock Unit Award Agreement for Inducement Grant Outside of 2015 Equity Incentive Plan (incorporated by reference to Exhibit 99.4 to the Company's current report on Form S-8, filed with the SEC on June 8, 2017).
10.14†	The Company's 2017 Employee Stock Purchase Plan (incorporated by reference to Exhibit 99.2 to the Company's Current report on Form 8-K, filed with the SEC on May 18, 2017).
10.15†	The Company's 2018 Equity Incentive Plan, as amended (incorporated by reference to Exhibit 99.1 to the Company's Current Report on Form 8-K, filed with the SEC on May 13, 2024).
10.16†	Form of Stock Option Grant Notice, Option Agreement and Exercise Notice for use under the Company's 2018 Equity Incentive Plan, as amended (incorporated by reference to Exhibit 10.21 to the Company's Annual Report on Form 10-K for the year ended December 31, 2021, filed with the SEC on February 24, 2022).
10.17†	Form of Restricted Stock Unit Grant Notice and Restricted Stock Unit Award Agreement for use under the Company's 2018 Equity Incentive Plan, as amended (incorporated by reference to Exhibit 10.22 to the Company's Annual Report on Form 10-K for the year ended December 31, 2021, filed with the SEC on February 24, 2022).
10.18†	Form of Stock Option Grant Notice, Option Agreement and Notice of Exercise for Inducement Grant Outside of 2018 Equity Incentive Plan, as amended (incorporated by reference to Exhibit 99.2 to the Company's Registration Statement on Form S-8 (File No. 333-232857), filed with the SEC on July 26, 2019).
10.19†	Form of Restricted Stock Unit Grant Notice and Restricted Stock Unit Award Agreement for Inducement Grant Outside of 2018 Equity Incentive Plan, as amended (incorporated by reference to Exhibit 99.3 to the Company's Registration Statement on Form S-8 (File No. 333-232857), filed with the SEC on July 26, 2019).
10.20+↓	Sublicense Agreement, dated February 16, 2012, by and among Ligand Pharmaceuticals Incorporated, a Delaware corporation, Pharmacopeia, Inc., a Delaware limited liability company, and the Company, a Delaware limited liability company (incorporated by reference to Exhibit 10.25 to the Company's Annual Report on Form 10-K for the year ended December 31, 2021, filed with the SEC on February 24, 2022).
10.21*	Amendment No. 3 to Sublicense Agreement dated as of February 27, 2015, between the Company and Ligand Pharmaceuticals Incorporated (incorporated by reference to Exhibit 10.26 to the Company's Annual Report on Form 10-K for the year ended December 31, 2021, filed with the SEC on February 24, 2022).
10.22*	Amendment No. 4 to Sublicense Agreement dated as of September 17, 2015, between the Company and Ligand Pharmaceuticals Incorporated (incorporated by reference to Exhibit 10.27 to the Company's Annual Report on Form 10-K for the year ended December 31, 2021, filed with the SEC on February 24, 2022).
10.23*	Amendment No. 5 to Sublicense Agreement dated as of March 20, 2018, between the Company and Ligand Pharmaceuticals Incorporated (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on October 31, 2024).
10.24*	Trademark License and Supply Agreement, dated May 29, 2014, by and between the Company and Mission Pharmacal Company (incorporated by reference to Exhibit 10.25 to the Company's Annual Report on Form 10-K, filed with the SEC on February 20, 2024).
10.25	First Amendment to Trademark License and Supply Agreement, effective as of July 28, 2014, by and between Mission Pharmacal Company and the Company (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K, filed with the SEC on July 29, 2014).
10.26	Addendum to Trademark License and Supply Agreement, dated October 19, 2015, by and between to Company and Mission Pharmacal (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on November 6, 2015).
10.27*	Third Amendment to Trademark License and Supply Agreement dated as of March 17, 2016, between the Company and Mission Pharmacal Company (incorporated by reference to Exhibit 10.28 to the Company's Annual Report on Form 10-K, filed with the SEC on February 20, 2024).
10.28*	Amendment One to the Third Amendment to Trademark License and Supply Agreement, dated September 12, 2016, by and between the Company and Mission Pharmacal Company (incorporated by reference to Exhibit 10.29 to the Company's Annual Report on Form 10-K, filed with the SEC on February 20, 2024).
10.29*	Amendment Two to the Third Amendment to Trademark License and Supply Agreement, dated November 3, 2017, by and between the Company and Mission Pharmacal Company (incorporated by reference to Exhibit 10.30 to the Company's Annual Report on Form 10-K, filed with the SEC on February 20, 2024).
10.30	Fourth Amendment to Trademark License and Supply Agreement dated as of November 28, 2018, between the Company and Mission Pharmacal (incorporated by reference to Exhibit 10.19 to the Company's Annual Report on Form 10-K, filed with the SEC on February 26, 2019).
10.31*	Fifth Amendment to Trademark License and Supply Agreement dated as of September 30, 2020, between the Company and Mission Pharmacal Company (incorporated by reference to Exhibit 10.32 to the Company's Annual Report on Form 10-K, filed with the SEC on February 20, 2024).
10.32↓*	Master Manufacturing Supply Agreement, dated September 30, 2020, between the Company and STA Pharmaceutical Hong Kong Limited, a subsidiary of WuXi AppTec (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on November 5, 2020).
10.33↓*	First Amendment to Master Manufacturing Supply Agreement, effective as of November 14, 2022, between the Company and STA Pharmaceutical Hong Kong Limited, a subsidiary of WuXi AppTec (incorporated by reference to Exhibit 10.37 to the Company's Annual Report on Form 10-K, filed with the SEC on February 23, 2023).

10.34↓*	License and Collaboration Agreement, dated September 15, 2021, by and among Orphan Technologies and Vifor (International) Ltd., and, solely with respect to Article 15, the Company (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on October 29, 2021).
10.35↓*	Amendment No. 1 to the License and Collaboration Agreement, effective as of October 5, 2022, by and between Travere Therapeutics Switzerland GmbH (formerly known as Orphan Technologies Limited) and Vifor (International) Ltd. (incorporated by reference to Exhibit 10.39 to the Company's Annual Report on Form 10-K, filed with the SEC on February 23, 2023).
10.36↓*	Commercial Supply Agreement, dated December 21, 2021, between the Company and Catalent Pharma Solutions, LLC (incorporated by reference to Exhibit 10.40 to the Company's Annual Report on Form 10-K for the year ended December 31, 2021, filed with the SEC on February 24, 2022).
10.37	Office Lease, effective April 12, 2019, between the Company and Kilroy Realty, L.P. (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q, filed with the SEC on August 6, 2019).
10.38*	First Amendment to Office Lease, dated November 7, 2019, between the Company and Kilroy Realty, L.P. (incorporated by reference to Exhibit 99.1 to the Company's Current Report on Form 8-K, filed with the SEC on June 8, 2020).
10.39	Third Amendment to Existing Office Lease and Second Amendment to Long Term Lease, dated May 29, 2020, between the Company and Kilroy Realty, L.P. (incorporated by reference to Exhibit 99.2 to the Company's Current Report on Form 8-K, filed with the SEC on June 8, 2020).
19.1	Insider Trading Policy.
21.1	List of subsidiaries of the Company.
23.1	Consent of Ernst & Young.
23.2	Consent of BDO USA, P.C.
24.1	Power of Attorney (see signature page hereto).
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 Sarbanes Oxley Act of 2002.
32.2	Chief Financial Officer's Certification pursuant to Section 906 of Sarbanes Oxley Act of 2002.
97	Incentive Compensation Recoupment Policy (incorporated by reference to Exhibit 97 to the Company's Annual Report on Form 10-K, filed with the SEC on February 20, 2024).
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 Annual Report on Form 10-K has been formatted in Inline XBRL.

† Indicates management contract or compensatory plan.

↓ Schedules have been omitted pursuant to Item 601(a)(5) of Regulation S-K. The Company undertakes to furnish supplemental copies of any of the omitted schedules upon request by the SEC.

* Certain portions of this exhibit are omitted pursuant to Item 601(b)(10)(iv).

ITEM 16. FORM 10-K SUMMARY

None.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Date: February 20, 2025

Travere Therapeutics, Inc.

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

POWER OF ATTORNEY

Know all persons by these presents, that each person whose signature appears below constitutes and appoints Eric Dube and Christopher Cline, and each of them, as his attorneys-in-fact and agents, each with power of substitution in any and all capacities, to sign any amendments to this annual report on Form 10-K, and to file the same with exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, hereby ratifying and confirming all that the attorney-in-fact or his substitute or substitutes may do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated:

Signature	Title	Date
/s/ Eric Dube Eric Dube	Chief Executive Officer and Director (Principal Executive Officer)	February 20, 2025
/s/ Christopher Cline Christopher Cline	Chief Financial Officer (Principal Financial Officer)	February 20, 2025
/s/ Sandra Calvin Sandra Calvin	Senior Vice President and Chief Accounting Officer (Principal Accounting Officer)	February 20, 2025
/s/ Roy D. Baynes Roy D. Baynes	Director	February 20, 2025
/s/ Suzanne Bruhn Suzanne Bruhn	Director	February 20, 2025
/s/ Timothy Coughlin Timothy Coughlin	Director	February 20, 2025
/s/ Gary Lyons Gary Lyons	Chair of the Board	February 20, 2025
/s/ Jeffrey A. Meckler Jeffrey A. Meckler	Director	February 20, 2025
/s/ John A. Orwin John A. Orwin	Director	February 20, 2025
/s/ Sandra E. Poole Sandra E. Poole	Director	February 20, 2025
/s/ Ron Squarer Ron Squarer	Director	February 20, 2025
/s/ Ruth Williams-Brinkley Ruth Williams-Brinkley	Director	February 20, 2025

TRAVERE THERAPEUTICS, INC. AND SUBSIDIARIES

INDEX TO FINANCIAL STATEMENTS

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Travere Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Travere Therapeutics, Inc. and subsidiaries (the Company) as of December 31, 2024 and 2023, the related consolidated statements of operations and comprehensive loss, stockholders' equity and cash flows for each of the two years in the period ended December 31, 2024 and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2024 and 2023, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2024, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2024, based on criteria established in Internal Control-Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework), and our report dated February 20, 2025 expressed an unqualified opinion thereon.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current period audit of the financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective or complex judgments. The communication of the critical audit matter does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Government rebate deductions from revenue

Description of the Matter	As described in Note 3 to the consolidated financial statements under the caption “Deductions from Revenue”, the Company calculates the rebates for Filspari that it will be obligated to provide to government programs and deducts these estimated amounts from its gross product sales at the time the revenues are recognized. Allowances for government rebates and discounts are established based on an estimated allocation of payers and the government-mandated discounts applicable to government-funded programs. Where appropriate, these allowances take into consideration the Company’s historical experience, current statutory requirements and specific known market events and trends. Estimated government rebates are included in accrued expenses on the consolidated balance sheet. Auditing the Filspari government rebate deductions from revenue was complex and required significant auditor judgment because the related accruals were dependent on certain subjective assumptions. Such subjective assumptions included estimated allocations of payers and estimated government-mandated discounts applicable to government-funded programs, each of which are adjusted for the Company’s historical experience, current statutory requirements and specific known market events and trends, where appropriate.
How We Addressed the Matter in Our Audit	We obtained an understanding, evaluated the design, and tested the operating effectiveness of controls over the Company’s Filspari government rebate deductions from revenue process. This included testing controls over management’s review of the significant assumptions described above and other inputs into the estimation of government rebates including the accuracy of data used in the calculation. To test the Filspari government rebate deductions from revenue, our audit procedures included, among others, understanding and evaluating the significant assumptions and underlying data used in management’s calculations. Our testing of significant assumptions included a lookback analysis to evaluate the historical accuracy of management’s estimates by comparing actual activity to previous estimates and performing sensitivity analyses over the subjective assumptions to evaluate the completeness of the reserves. As a part of our procedures, we evaluated the reasonableness of the Company’s assumptions considering recent sales trends and regulatory factors related to Filspari government rebates.

/s/ Ernst & Young LLP

We have served as the Company’s auditor since 2023.

San Diego, California

February 20, 2025

Report of Independent Registered Public Accounting Firm

Shareholders and Board of Directors

Travere Therapeutics, Inc.

San Diego, California

Opinion on the Consolidated Financial Statements

We have audited the accompanying consolidated statements of operations and comprehensive loss, stockholders' equity, and cash flows of Travere Therapeutics, Inc. and subsidiaries (the "Company") for the year ended December 31, 2022, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the results of its operations and its cash flows for the year ended December 31, 2022, in conformity with accounting principles generally accepted in the United States of America.

Change in Accounting Principle

As discussed in Note 2 to the consolidated financial statements, effective January 1, 2022, the Company adopted Accounting Standards Update ("ASU") No. 2020-06, Debt — Debt with Conversion and Other Options *("Subtopic 470-20") and* Derivatives and Hedging — Contracts in Entity's Own Equity *("Subtopic 815-40"):* Accounting for Convertible Instruments and Contracts in an Entity's Own Equity ("ASU 2020-06").

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's consolidated financial statements based on our audit. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) ("PCAOB") and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud.

Our audit included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audit also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audit provides a reasonable basis for our opinion.

/s/ BDO USA, P.C.

We served as the Company's auditor from 2014 to 2023.

San Diego, California

February 23, 2023, except for the effects of discontinued operations discussed in Notes 1 and 19, as to which the date is February 20, 2024 and Note 20, as to which the date is February 20, 2025.

TRAVERE THERAPEUTICS, INC. AND SUBSIDIARIES
CONSOLIDATED BALANCE SHEETS
(In thousands, except share and per share amounts)

	December 31, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 58,535	$ 58,176
Marketable debt securities, at fair value	312,166	508,675
Accounts receivable, net	27,116	21,179
Inventory	6,200	9,410
Prepaid expenses and other current assets	12,685	19,335
Total current assets	416,702	616,775
Long-term inventory	35,656	31,494
Property and equipment, net	5,336	7,479
Operating lease right-of-use assets	14,295	18,061
Intangible assets, net	103,974	104,443
Other assets	18,162	10,661
Total assets	$ 594,125	$ 788,913
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 23,534	$ 41,675
Accrued expenses	86,028	118,991
Convertible debt, current portion	68,678	—
Deferred revenue, current portion	2,815	7,096
Operating lease liabilities, current portion	5,405	4,909
Other current liabilities	14,291	5,237
Total current liabilities	200,751	177,908
Convertible debt, less current portion	310,310	377,263
Operating lease liabilities, less current portion	17,191	22,612
Other non-current liabilities	6,796	10,320
Total liabilities	535,048	588,103
Commitments and Contingencies (See Note 11)		
Stockholders' Equity:		
Preferred stock $0.0001 par value; 20,000,000 shares authorized; no shares issued and outstanding as of December 31, 2024 and 2023	—	—
Common stock $0.0001 par value; 200,000,000 shares authorized; 87,452,835 and 75,367,117 issued and outstanding as of December 31, 2024 and 2023, respectively	9	7
Additional paid-in capital	1,506,315	1,327,881
Accumulated deficit	(1,447,167)	(1,125,622)
Accumulated other comprehensive loss	(80)	(1,456)
Total stockholders' equity	59,077	200,810
Total liabilities and stockholders' equity	$ 594,125	$ 788,913

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

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

	Year Ended December 31,		
	2024	2023	2022
Net product sales	$ 226,707	$ 127,537	$ 97,970
License and collaboration revenue	6,468	17,701	11,490
Total revenue	233,175	145,238	109,460
Operating expenses:			
Cost of goods sold	7,744	11,450	4,420
Research and development	217,496	244,990	227,333
Selling, general and administrative	264,119	265,542	197,520
In-process research and development	65,205	—	—
Restructuring	2,438	11,394	—
Total operating expenses	557,002	533,376	429,273
Operating loss	(323,827)	(388,138)	(319,813)
Other income (expense), net:			
Interest income	17,817	21,768	6,276
Interest expense	(11,182)	(11,334)	(11,014)
Other (expense) income, net	(3,318)	1,594	974
Loss on extinguishment of debt	—	—	(7,578)
Total other income (expense), net	3,317	12,028	(11,342)
Loss from continuing operations before income tax provision	(320,510)	(376,110)	(331,155)
Income tax provision on continuing operations	(120)	(223)	(313)
Loss from continuing operations, net of tax	(320,630)	(376,333)	(331,468)
(Loss) income from discontinued operations, net of tax	(915)	264,934	52,986
Net loss	$ (321,545)	$ (111,399)	$ (278,482)
Per share data			
Basic and diluted:			
Net loss from continuing operations	$ (4.07)	$ (5.07)	$ (5.20)
Net (loss) income from discontinued operations	(0.01)	3.57	0.83
Net loss per common share	$ (4.08)	$ (1.50)	$ (4.37)
Weighted average common shares outstanding	78,888,861	74,267,418	63,758,515
Comprehensive loss:			
Net loss	$ (321,545)	$ (111,399)	$ (278,482)
Unrealized gain (loss) on defined benefit pension plan	328	(374)	(368)
Foreign currency translation gain (loss)	1,422	(1,871)	507
Unrealized (loss) gain on marketable debt securities	(374)	3,696	(2,484)
Comprehensive loss	$ (320,169)	$ (109,948)	$ (280,827)

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

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

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
BALANCE - DECEMBER 31, 2021	62,491,498	$ 6	$ 1,068,634	$ (562)	$ (765,966)	$ 302,112
Cumulative-effect adjustment from adoption of ASU 2020-06	—	—	(74,945)	—	30,225	(44,720)
Share based compensation	—	—	37,897	—	—	37,897
Issuance of common stock under the equity incentive plan and proceeds from exercise	935,598	—	4,585	—	—	4,585
Employee stock purchase program purchase and expense	161,874	—	4,259	—	—	4,259
Issuance of common stock under At-The-Market offering, net of issuance costs of $0.6 million.	701,600	—	19,545	—	—	19,545
Unrealized loss on defined benefit pension plan	—	—	—	(368)	—	(368)
Foreign currency translation adjustments	—	—	—	507	—	507
Unrealized loss on marketable debt securities	—	—	—	(2,484)	—	(2,484)
Net loss	—	—	—	—	(278,482)	(278,482)
BALANCE - DECEMBER 31, 2022	64,290,570	$ 6	$ 1,059,975	$ (2,907)	$ (1,014,223)	$ 42,851
Share based compensation	—	—	43,985	—	—	43,985
Issuance of common stock under the equity incentive plan and proceeds from exercise	1,046,276	—	3,240	—	—	3,240
Employee stock purchase program purchase and expense	326,521	—	4,853	—	—	4,853
Equity offering, net of issuance costs of $12.6 million	9,703,750	1	191,198	—	—	191,199
Issuance of pre-funded common stock warrants, net of issuance costs of $1.6 million	—	—	24,630	—	—	24,630
Unrealized loss on defined benefit pension plan	—	—	—	(374)	—	(374)
Foreign currency translation adjustments	—	—	—	(1,871)	—	(1,871)
Unrealized gain on marketable debt securities	—	—	—	3,696	—	3,696
Net loss	—	—	—	—	(111,399)	(111,399)
BALANCE - DECEMBER 31, 2023	75,367,117	$ 7	$ 1,327,881	$ (1,456)	$ (1,125,622)	$ 200,810
Share based compensation	—	—	35,679	—	—	35,679
Issuance of common stock under the equity incentive plan and proceeds from exercise	1,455,575	1	4,452	—	—	4,453
Employee stock purchase program purchase and expense	395,768	—	3,566	—	—	3,566
Equity offering, net of issuance costs of $9.0 million	8,984,375	1	134,737	—	—	134,738
Exercise of pre-funded common stock warrants	1,250,000	—	—	—	—	—
Unrealized gain on defined benefit pension plan	—	—	—	328	—	328
Foreign currency translation adjustments	—	—	—	1,422	—	1,422
Unrealized loss on marketable debt securities	—	—	—	(374)	—	(374)
Net loss	—	—	—	—	(321,545)	(321,545)
BALANCE - DECEMBER 31, 2024	87,452,835	$ 9	$ 1,506,315	$ (80)	$ (1,447,167)	$ 59,077

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

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

	For the year ended December 31,		
	2024	2023	2022
Cash Flows From Operating Activities:			
Net loss	$ (321,545)	$ (111,399)	$ (278,482)
Net (loss) income from discontinued operations	(915)	264,934	52,986
Net loss from continuing operations	(320,630)	(376,333)	(331,468)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	43,555	38,530	20,719
Share based compensation	36,913	44,246	38,143
In-process research and development	65,205	—	—
Loss on extinguishment of debt	—	—	7,578
Loss on allowance for inventory	2,819	3,039	1,039
Amortization of debt discount and issuance costs	1,725	1,718	1,622
Amortization of discounts on investments	(4,535)	(7,456)	(700)
Other	7,944	(3,626)	1,346
Changes in operating assets and liabilities:			
Accounts receivable	(6,001)	(11,264)	(1,060)
Inventory	(3,771)	(39,420)	(1,209)
Tax receivable	(41)	(35)	(374)
Prepaid expenses and other current and non-current assets	(3,164)	(4,657)	(5,196)
Change in lease assets and liabilities, net	(949)	(986)	(984)
Accounts payable	(17,563)	24,763	2,055
Accrued expenses	(31,061)	22,321	22,181
Deferred revenue, current and non-current	(6,116)	(15,779)	(12,929)
Other current and non-current liabilities	5,646	(418)	(1,609)
Net cash used in operating activities - continuing operations	(230,024)	(325,357)	(260,846)
Net cash (used in) provided by operating activities - discontinued operations	(7,451)	45,336	74,555
Net cash used in operating activities	(237,475)	(280,021)	(186,291)
Cash Flows From Investing Activities:			
Proceeds from the sale and maturity of marketable debt securities	326,334	334,575	381,993
Purchase of marketable debt securities	(125,757)	(443,942)	(385,389)
Purchase of intangible assets	(36,212)	(41,591)	(28,366)
Payment of milestone	(65,000)	—	—
Other	(40)	(668)	(791)
Net cash provided by (used in) investing activities - continuing operations	99,325	(151,626)	(32,553)
Net cash provided by investing activities - discontinued operations	—	207,402	—
Net cash provided by (used in) investing activities	99,325	55,776	(32,553)
Cash Flows From Financing Activities:			
Payment of guaranteed minimum royalty	(2,100)	(2,100)	(2,100)
Proceeds from issuance of 2029 convertible senior notes	—	—	316,250
Payment of debt issuance costs	—	—	(9,882)
Repurchase of 2025 convertible senior notes including premium	—	—	(211,324)
Proceeds from the issuance of common stock, net of issuance costs	134,738	191,198	—

Proceeds from the issuance of pre-funded warrants, net of issuance costs	—	24,630	—
Proceeds from exercise of stock options	4,452	3,240	4,585
Proceeds from the issuances under the employee stock purchase plan	2,332	3,166	2,978
Proceeds from the issuance of common stock in At-the-Market equity offering	—	—	19,545
Net cash provided by financing activities - continuing operations	139,422	220,134	120,052
Net cash used in financing activities - discontinued operations	—	(1,382)	(2,479)
Net cash provided by financing activities	139,422	218,752	117,573
Effect of exchange rate changes on cash	(913)	1,981	(2,794)
Net increase (decrease) in cash and cash equivalents	359	(3,512)	(104,065)
Cash and cash equivalents, beginning of year	58,176	61,688	165,753
Cash and cash equivalents, end of year	$ 58,535	$ 58,176	$ 61,688
Supplemental Disclosure of Cash Flow Information:			
Operating cash flows used for operating leases	$ 6,392	$ 6,315	$ 6,020
Cash paid for interest	$ 8,838	$ 8,838	$ 10,159
Cash paid for income taxes	$ (453)	$ (578)	$ (996)
Non-cash investing and financing activities:			
Accrued royalty in excess of minimum payable to the sellers of Thiola	$ 15,436	$ 16,206	$ 16,067

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

TRAVERE THERAPEUTICS, INC. AND SUBSIDIARIES

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

NOTE 1. DESCRIPTION OF BUSINESS

Organization and Description of Business

Travere Therapeutics, Inc. (“we”, “our”, “us”, “Travere” and the “Company”) refers to Travere Therapeutics, Inc., a Delaware corporation, as well as its subsidiaries. Travere is a fully integrated biopharmaceutical company headquartered in San Diego, California focused on identifying, developing and delivering life-changing therapies to people living with rare kidney and metabolic diseases. The Company regularly evaluates and, where appropriate, acts on opportunities to expand its product pipeline and approved products through licenses and acquisitions of products in areas that will serve rare disease patients with serious unmet medical need and that the Company believes offer attractive growth characteristics.

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, 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 Consolidated Financial Statements for all periods presented. See Note 19 for further discussion.

Unless otherwise noted, amounts and disclosures throughout the Notes to the Consolidated Financial Statements relate to the Company's continuing operations.

Approved Products:

FILSPARI® (sparsentan)

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

FILSPARI had previously been granted accelerated approval in February 2023 based on the surrogate marker of proteinuria. Full approval 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.

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). In November 2024, the Medicines and Healthcare products Regulatory Agency (MHRA) approved FILSPARI in the United Kingdom.

In January 2024, the Company entered into an exclusive licensing agreement with Renalys Pharma, Inc. ("Renalys"), to bring sparsentan for the treatment of IgAN to patients in Japan and other countries in Asia, 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 registration study of sparsentan in Japan to support potential approval of sparsentan in Japan. In July 2024, Renalys announced that the first patient was dosed in the study, and in January 2025, Renalys announced achievement of full enrollment in the study. In December 2024, Renalys announced that sparsentan received Orphan Drug Designation from the Japanese Ministry of Health, Labour and Welfare for the indication of primary IgA nephropathy as of November 27, 2024. Under the terms of the licensing agreement, Renalys will be responsible for development, regulatory matters, and commercialization in the licensed territories.

Thiola® and Thiola EC® (tiopronin tablets)

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

Clinical-Stage Programs:

Sparsentan for the treatment of FSGS

Sparsentan remains a novel investigational product candidate which has been granted Orphan Drug Designation for the treatment of 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 a planned Type C meeting with the FDA to discuss previously reported results from the Phase 3 DUPLEX Study of sparsentan in FSGS. The FDA acknowledged the high unmet need for approved therapies as well as the challenges in studying FSGS but indicated that the two-year results from the Phase 3 DUPLEX Study alone were not sufficient to support an sNDA submission. In February 2025, the Company announced that it had completed a Type C meeting with the FDA and that the Company plans to submit an sNDA around the end of the first quarter of 2025. The sNDA will be based on existing data from the Phase 3 DUPLEX and Phase 2 DUET studies of FILSPARI.

Pegtibatinase

Pegtibatinase is a novel investigational human enzyme replacement candidate being evaluated for the treatment of classical homocystinuria (HCU). Pegtibatinase has been granted Rare Pediatric Disease, Fast Track and Breakthrough Therapy designations by the FDA, as well as orphan drug designation in the United States and European Union. In May 2023, the Company announced positive topline results from cohort 6 in the Phase 1/2 COMPOSE Study. In December 2023, the Company initiated the pivotal Phase 3 HARMONY Study to support the potential approval of pegtibatinase for the treatment of classical HCU. The HARMONY Study is a global, randomized, multi-center, double-blind, placebo-controlled Phase 3 clinical trial designed to evaluate the efficacy and safety of pegtibatinase as a novel treatment to reduce total homocysteine (tHcy) levels. In the beginning of 2024, the first patients were dosed in the HARMONY Study.

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

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

Principles of Consolidation

The 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 5 for further discussion of variable interest entities (“VIE”) that the Company consolidates.

Use of Estimates

In preparing financial statements in conformity with U.S. GAAP, management is required to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported amounts of expenses during the reporting period. Due to inherent uncertainty involved in making estimates, actual results reported in future periods may be affected by changes in these estimates. On an ongoing basis, the Company evaluates its estimates and assumptions. These estimates and assumptions include revenue recognition, forecasting probability-weighted cash flows based upon estimates of forecasted revenues, clinical and regulatory timelines and discount rates, valuing equity securities in share-based payments, estimating expenses of contracted research organizations, estimating reserves for inventory, estimating the useful lives of depreciable and amortizable assets, estimating of valuation allowances and uncertain tax positions, and estimates associated with the assessment of impairment for long-lived assets.

Revenue Recognition

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

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

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

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

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

Inventory, Related Reserves and Cost of Goods Sold

Inventory, which is recorded at the lower of cost or net realizable value, includes materials and other direct and indirect costs and is valued using the first-in, first-out method. The Company periodically analyzes its inventory levels to identify inventory that may expire prior to expected sale or has a cost basis in excess of its estimated realizable value, and writes down such inventory as appropriate. In addition, the Company's products are

subject to strict quality control and monitoring which the Company's manufacturers perform throughout their manufacturing process. The Company does not directly manufacture any product. The Company has a single supplier for its product Thiola, and utilizes contract service providers for the manufacture of the active pharmaceutical ingredient for FILSPARI and the manufacture of primary packaging, secondary packaging and serialization for its product FILSPARI. The inventory reserve was $3.7 million and $2.4 million at December 31, 2024 and 2023, respectively.

Inventory, net of reserves, consisted of the following at December 31, 2024 and 2023 (*in thousands*):

	December 31, 2024	December 31, 2023
Raw materials	$ 30,552	$ 33,790
Work in process	7,625	4,727
Finished goods	3,679	2,387
Total inventory	$ 41,856	$ 40,904
Classified as:		
Inventory	$ 6,200	$ 9,410
Long-term inventory	35,656	31,494
Total inventory	$ 41,856	$ 40,904

The balance classified as long-term inventory consists of raw materials, work in process and finished goods for both Thiola and FILSPARI as of December 31, 2024. 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.

Cost of goods sold includes the cost of inventory sold, third party manufacturing and supply chain costs, product shipping, tracking 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 generally consists of the sale of active pharmaceutical ingredients to the Company's collaboration partners, generally at cost plus a margin.

The following table summarizes cost of goods sold for the years ended December 31, 2024, 2023 and 2022 (*in thousands*):

	Year Ended December 31,		
	2024	2023	2022
Cost of goods sold - product sales	$ 7,446	$ 8,406	$ 4,420
Cost of goods sold - license and collaboration	298	3,044	—
Total cost of goods sold	$ 7,744	$ 11,450	$ 4,420

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 year ended December 31, 2024, sales of FILSPARI primarily consisted of zero-cost inventories. As of December 31, 2024, the Company had approximately $2.3 million of zero-cost inventory remaining, the majority of which the Company expects will be consumed in 2025. The Company began capitalizing inventory costs associated with FILSPARI following the February 2023 accelerated approval.

At December 31, 2024, the Company's evaluation of excess inventory and obsolescence resulted in a $1.0 million charge to cost of goods sold and an equal write-down of inventory balances due to lower forecasted sales of the Company's tiopronin products. For the year ended December 31, 2024, there were no charges recorded on FILSPARI inventory.

At December 31, 2023, the Company's evaluation of excess inventory and obsolescence considered certain minimum purchase obligations, which in combination with lower forecasted sales of FILSPARI resulted in a $3.2 million charge to cost of goods sold. The charge to cost of goods of sold included a $2.1 million write-down of inventory balances and $1.1 million accrued for firm purchase commitments.

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.

During the three months ended December 31, 2022, the Company recorded an out of period adjustment that decreased research and development expenses and related accrued expenses by $7.7 million, $1.5 million of which was previously recorded in fiscal year 2022 and $6.2 million that was originally recorded in fiscal year 2021. The adjustment was the result of a certain pre-launch inventory contract that was not properly evaluated for accounting implications at inception. The Company evaluated the impact of the adjustment and concluded it is not material, individually and in the aggregate, to 2022 or any prior period financial statements.

Clinical Trial Expenses

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

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

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

Share-Based Compensation

The Company recognizes all employee share-based compensation as a cost within research and development expenses and selling, general, and administrative expenses. Equity-classified awards principally related to stock options, restricted stock units (“RSUs”) and performance stock units ("PSUs"), are measured at the grant date fair value of the award. The Company determines grant date fair value of stock option awards using the Black-Scholes option-pricing model. The fair value of RSUs and PSUs are determined using the closing price of the Company’s common stock on the grant date. For service based vesting grants, expense is recognized over the requisite service period. For PSUs, expense is recognized over the implicit service period, once vesting is probable. No expense is recognized for PSUs if it is not probable the vesting criteria will be satisfied. Forfeitures are accounted for as they occur.

	Expiration Term	Vesting Term
Stock Options	10 years	3 to 4 years
Restricted Stock Units	----	1 to 4 years

Earnings (Loss) Per Share

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

The Company's diluted earnings/(loss) per share computation includes the effect, if any, of shares that would be issuable upon the exercise of outstanding stock options, convertible debt and RSUs, reduced by the number of shares which are assumed to be purchased by the Company from the resulting proceeds at the average market price during the year, when such amounts are dilutive to the earnings per share calculation. 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.

Cash and Cash Equivalents

The Company considers all highly liquid marketable securities with an original maturity of three months or less to be cash equivalents. Due to the short-term maturity of such investments, the carrying amounts are a reasonable estimate of fair value.

Concentration of Credit Risk

The Company maintains its cash and cash equivalents at insured financial institutions, the balances of which may, at times, exceed federally insured limits. Generally, these deposits may be redeemed upon demand, and the Company believes there is minimal risk of losses on such balances.

The Company monitors its investments with counterparties with the objective of minimizing concentrations of credit risk. The Company's investment policy is to invest only in institutions that meet high credit quality standards and established limits on the amount and time to maturity of investments with any individual counterparty. The policy also requires that investments are only entered into with corporate and financial institutions that meet high quality standards.

Marketable Debt Securities

The Company classified marketable debt securities held as “available-for-sale” and carries them at fair value. The Company classifies these investments as current assets, even if the maturity when acquired by the Company is greater than one year due to the ability to liquidate within the next 12 months. The amortized cost of marketable debt securities is adjusted for amortization of premiums and accretion of discounts to maturity. Such amortization and accretion, as well as interest and dividends, is included in interest income. Unrealized gains and losses on marketable debt securities are recorded as a separate component of stockholders’ equity as accumulated other comprehensive loss, unless an impairment is determined to be the result of credit-related factors or the Company intends to sell the security or it is more likely than not that the Company will be required to sell the security before recovery. Unrealized losses that are determined to be credit-related are recorded as an allowance against the amortized cost basis. Realized gains or losses on debt security transactions and declines in value that are determined to be the result of credit losses, if any, are reported in other income or expense in the Consolidated Statements of Operations and Comprehensive Loss. The cost of securities sold is based on the specific identification method. Marketable debt securities are maintained at one financial institution and are governed by the Company’s investment policy. See Note 6 for further discussion.

Accounts Receivables, Net

Trade accounts receivable are recorded net of reserves for prompt pay discounts and expected credit losses. Estimates for allowances for credit losses are determined based on existing contractual obligations, historical payment patterns and individual customer circumstances. The allowance for credit losses was zero at both December 31, 2024 and 2023, respectively. For the years ended December 31, 2024, 2023 and 2022, bad debt expense recorded in the Consolidated Statements of Operations was immaterial. The Company's evaluation of 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.

Supplier Concentration Risk

The Company has no manufacturing capabilities and relies on third party manufacturers who are sole source suppliers for manufacturing of its products. The Company intends to rely on third-party manufacturers for the long-term commercial supply of FILSPARI and for its development stage product candidates, including sparsentan for the treatment of FSGS and pegtibatinase. The Company expects 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 the Company.

Property and Equipment, net

Property and equipment are stated at cost, net of accumulated depreciation. Depreciation is computed using the straight-line method over the related estimated useful lives as presented in the table below. Significant additions and improvements are capitalized, while repairs and maintenance are charged to expense as incurred. Property and equipment purchased for specific research and development projects with no alternative use is expensed as incurred.

The major classifications of property and equipment, including their respective expected useful lives, consist of the following:

Computers and equipment	3 years
Furniture and fixtures	7 years
Leasehold improvements	Shorter of length of lease or life of the asset

Leases

The Company determines whether a contract is, or contains, a lease at inception. The Company classifies each of its leases as operating or financing considering factors such as the length of the lease term, the present value of the lease payments, the nature of the asset being leased, and the potential for ownership of the asset to transfer during the lease term. Leases with terms greater than one year are recognized on the Consolidated Balance Sheets as Right-of-use assets and Lease liabilities and are measured at the present value of the fixed payments due over the expected lease term minus the present value of any incentives, rebates or abatement expected to be received from the lessor. Options to extend a lease are typically excluded from the expected lease term as the exercise of the option is typically not reasonably certain. The interest rate implicit in lease contracts is typically not readily determinable. As such, the Company utilizes the appropriate incremental borrowing rate, which is the rate incurred to borrow on a collateralized basis an amount equal to the lease payments over a similar term and in a similar economic environment.

In addition to rent, the leases may require the Company to pay additional amounts for taxes, insurance, maintenance, and other expenses, which are generally referred to as non-lease components. The Company has elected to not separate lease and non-lease components. Only the fixed costs for lease components and their associated non-lease components are accounted for as a single lease component and recognized as part of the Right-of-use assets and Lease liabilities. The Company records expense to recognize fixed lease payments, including payment escalation, on a straight-line basis over the expected lease term. Costs determined to be variable and not based on an index or rate are not included in the measurement of the lease liability and are expensed as incurred.

The Company has made an accounting policy election to not recognize short-term leases, or leases that have a lease term of 12 months or less at commencement date, within its Consolidated Balance Sheets and to recognize those lease payments in the Consolidated Statements of Operations on a straight-line basis over the lease term.

The Company recognizes income from sublet office space on a straight-line basis over the term of the sublease, recorded in other income in the Consolidated Statements of Operations.

Intangible Assets, Net

The Company's intangible assets consist of licenses and purchased technology. Intangible assets with definite lives are amortized on a straight-line basis over their estimated useful lives and are reviewed periodically for impairment.

Intangible Assets with Cost Accumulation Model

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

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, resulting in incremental expense of $3.7 million recorded in selling, general, and administrative. The change in estimated useful life was accounted for as a change in accounting estimate and the remaining carrying amounts of the Thiola Intangible are 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 December 31, 2024 as such royalties are not yet probable and estimable.

In 2012, the Company entered into an agreement with Ligand Pharmaceuticals, Inc. (“Ligand”) for a worldwide sublicense to develop, manufacture and commercialize sparsentan (the “Ligand License Agreement”). The acquisition of the Ligand License Agreement qualified as an asset acquisition under the principles of ASC 805 in effect at the time of acquisition. The license agreement requires the Company to make royalty payments based on net sales of FILSPARI (sparsentan) and milestone payments. The liabilities for royalties and milestone payments are recognized in the period in which they become probable and estimable, which is typically in the period corresponding with the respective sales or achievement of the milestone.

The Company records an offsetting increase to the cost basis of the intangible asset under the cost accumulation model following the approval of FILSPARI. The additional cost basis is subsequently amortized over the remaining estimated useful life.

Variable Interest Entity

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

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

Equity Securities

The Company applies the equity method of accounting for investments when it has significant influence, but no controlling interest in the investee. Judgment regarding the level of influence over each equity method investment includes key factors such as ownership interest, representation on the board of directors, participation in joint steering committees and material intercompany transactions. Upon investment, the Company evaluates any basis difference between the carrying value and fair value of the Company's proportionate share of the investee's net assets. Basis differences relating to in-process research and development (IPR&D) are expensed when the investee is not considered a business as defined in ASC 805, *Business Combinations,* due to substantially all of the estimated fair value of the gross assets being concentrated in a group of similar IPR&D assets with no alternative future use. For the year ended December 31, 2024, the Company recognized $3.4 million in other (expense) income, net 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 5 for further discussion. Investments accounted for using the equity method are 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.

Goodwill

Goodwill represents the excess of purchase price over fair value of net assets acquired in a business combination and is not amortized. Goodwill is subject to impairment testing at least annually or when a triggering event occurs that could indicate a potential impairment. The Company has one segment and one reporting unit and as such reviews goodwill for impairment at the consolidated level.

Impairment of Long-Lived Assets

The Company's long-lived assets are primarily comprised of intangible assets, right-of-use assets, and property and equipment. The Company evaluates its finite-lived intangible assets, right-of-use assets, and property and equipment for impairment whenever events or changes in circumstances indicate the carrying value of an asset or group of assets may not be recoverable. If these circumstances exist, recoverability of assets to be held and used is measured by a comparison of the carrying amount of an asset group to future undiscounted net cash flows expected to be generated by the use and eventual disposition of the asset group. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the carrying amount of the assets exceeds the fair value of the assets.

In addition, indefinite-lived intangible assets are reviewed for impairment annually and whenever events or changes in circumstances indicate that it is more likely than not that the asset is impaired by comparing the fair value to the carrying value of the asset. To determine the fair value of the asset, the Company used the multi-period excess earnings method of the income approach. The more significant assumptions inherent in the application of this method include: the amount and timing of projected future cash flows (including revenue, cost of sales, research and development costs, and sales and marketing expenses), and the discount rate selected to measure the risks inherent in the future cash flows. See Note 8 for further discussion of certain long-lived assets measured at fair value on a nonrecurring basis when there are indicators of impairment.

Income Taxes

The Company follows ASC 740, *Income Taxes* ("ASC 740"), which requires recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Under this method, deferred tax assets and liabilities are based on the differences between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Deferred tax assets are reduced by a valuation allowance to the extent management concludes it is more likely than not that the asset will not be realized.

The standard addresses the determination of whether tax benefits claimed or expected to be claimed on a tax return should be recorded in the financial statements. Under ASC 740, the Company may recognize the tax benefit from an uncertain tax position only if it is more likely than not that the tax position will be sustained on examination by the tax authorities, based on the technical merits of the position. The tax benefits recognized in the financial statements from such a position should be measured based on the largest benefit that has a greater than fifty percent likelihood of being realized upon ultimate settlement. ASC 740 also provides guidance on de-recognition, classification, interest and penalties on income taxes, accounting in interim periods and requires increased disclosures. The Company's policy is to record estimated interest and penalty related to the underpayment of income taxes or unrecognized tax benefits as a component of its income tax provision.

Foreign Currency Translation

Functional and presentation currency

Items included in the financial statements of each entity comprising the Company are measured using the currency of the primary economic environment in which the entity operates (the functional currency).

Transactions and balances

Foreign currency transactions in each entity comprising the Company are remeasured into the functional currency of the entity using the exchange rates prevailing at the respective transaction dates. Foreign exchange gains and losses resulting from the settlement of such transactions and from the remeasurement at year-end exchange rates of monetary assets and liabilities denominated in foreign currencies are recognized within Other income (expense), net in the Consolidated Statements of Operations and Comprehensive Loss.

An aggregate loss of $0.7 million arising from foreign exchange transactions is included in other (expense) income, net for the year ended December 31, 2024. An aggregate gain of $0.5 million and $1.4 million is included in other (expense) income, net for the years ended December 31, 2023 and 2022, respectively.

The results and financial position of the Company that have a functional currency different from the U.S. dollar are translated as follows:

a. assets and liabilities presented are translated at the closing exchange rate as of December 31, 2024 and 2023;

b. income and expenses for the statements of operations and comprehensive loss are translated at average exchange rates that are relevant for the respective periods for which the income and expenses occurred; and

c. significant transactions use the exchange rate on the date of the transaction.

All resulting exchange differences arising from such translations are recognized directly in comprehensive income and presented as a separate component of equity.

The following table summarizes the foreign currency translation adjustment included in accumulated other comprehensive loss for the year ended December 31, 2024, 2023 and 2022 (*in thousands*):

	Foreign Currency Translation Adjustments included in Accumulated Other Comprehensive (Loss) Gain		
	2024	2023	2022
Balance at January 1,	$ (1,456)	$ 415	$ (92)
Foreign currency translation adjustments	1,422	(1,871)	507
Balance at December 31,	$ (34)	$ (1,456)	$ 415

Reclassifications

Certain reclassifications have been made to the prior year financial statements in order to conform to the current year's presentation. These reclassifications did not have an impact on total assets or total liabilities and stockholders' equity in the Consolidated Balance Sheets or net loss in the Consolidated Statements of Operations.

Patents

The Company expenses external costs, such as filing fees and associated attorney fees, incurred to obtain issued patents and patent applications pending. The Company also expenses costs associated with maintaining and defending patents subsequent to their issuance in the period incurred.

Legal Contingencies

The Company may, from time to time, be involved in various claims and legal actions that arise in the ordinary course of business. The Company accrues for legal contingencies when it is determined probable that a liability has been incurred and the amount of the loss can be reasonably estimated. See Note 11 for further discussion.

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 19 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. Restructuring costs were primarily comprised of one-time termination benefits, including severance, continuation of health insurance coverage, and other benefits for a specified period of time. In 2024, we also recognized charges for impairment of operating lease right-of-use assets and related leasehold improvements, as well as disposal costs on furniture and fixtures associated with available office space that the Company has sublet as a result of the reduction in occupancy. As of December 31, 2024, we are no longer incurring restructuring expenses. Of the $13.8 million recognized to date, $2.4 million was recognized during the year ended December 31, 2024, including $1.2 million related to the impairment and disposal costs and initial direct costs to obtain the sublease.

The following table summarizes the cash payments and accruals, included in accrued expenses of the Consolidated Balance Sheets, related to the restructuring for the years ended December 31, 2024 and 2023 (*in thousands*):

	2024	2023
Liability balance at January 1,	$ 11,421	$ —
Restructuring expenses	2,438	11,394
Non-cash impairment and disposal charges	(856)	—
Payments	(12,929)	—
Foreign currency impact	(74)	27
Liability balance at December 31,	$ —	$ 11,421

Recently Adopted Accounting Pronouncements

In November 2023, the FASB issued ASU No. 2023-07, Improvements to Reportable Segment Disclosures. 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 this new standard effective December 31, 2024. See Note 20, Segment Information, for disclosures related to the adoption of ASU 2023-07.

In August 2020, the FASB issued ASU No. 2020-06, Accounting for Convertible Instruments and Contracts in an Entity's Own Equity. The ASU includes amendments to the guidance on convertible instruments and the derivative scope exception for contracts in an entity's own equity in Subtopic 815-40 and simplifies the accounting for convertible instruments which include beneficial conversion features or cash conversion features by removing certain separation models in Subtopic 470-20. Additionally, the ASU requires entities to use the "if-converted" method when calculating diluted earnings per share for convertible instruments. The adoption of the new standard impacted the Company's accounting for its Convertible Senior Notes Due 2025 (2025 Notes), discussed in Note 7, which were previously accounted for using the cash conversion model applied under ASC 470-20, Debt with Conversion and Other Options ("ASC 470-20"). The Company adopted ASU 2020-06 on January 1, 2022 using the modified retrospective method. The cumulative effect of the accounting change as of January 1, 2022 increased the carrying amount of the 2025 Notes by $44.7 million, reduced additional paid-in capital by $74.9 million, and reduced accumulated deficit by $30.2 million.

Recently Issued Accounting Pronouncements

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

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

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

In December 2023, the FASB issued ASU No. 2023-09, Improvements to Income Tax Disclosures. This ASU does not change accounting for income taxes but requires new disclosures focusing on two areas, the effective rate reconciliation and taxes paid. This new standard is effective for public business entities for annual periods beginning after December 15, 2024. Early adoption is permitted. The Company is currently evaluating the impact of the adoption of this standard on the related disclosures.

NOTE 3. REVENUE RECOGNITION

Product Sales, Net

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

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

Deductions from Revenue

Revenues from product sales are recorded at the net sales price, which includes provisions resulting from discounts, rebates and co-pay assistance that are offered to customers, payers and other indirect customers relating to the Company's sales of its products. These provisions are based on the estimates of the amounts earned or to be claimed on the related sales. These amounts are treated as variable consideration, estimated and recognized as a reduction of the transaction price at the time of the sale, using the most likely amount method, and are classified as a reduction of accounts receivable (if the amount is payable to a customer) or as a current liability (if the amount is payable to a party other than a customer). The Company includes these estimated amounts in the transaction price to the extent it is probable that a significant reversal of cumulative revenue recognized for such transactions will not occur. Where appropriate, these reserves take into consideration the Company's historical experience, current contractual and statutory requirements and specific known market events and trends. Overall, these reserves reflect the Company's best estimates of the amount of consideration to which it is entitled based on the terms of the contract. If actual results in the future vary from the Company's provisions, the Company will adjust the estimate, which would affect net product revenue and earnings in the period such variances become known. For the years ended December 31, 2024, 2023 and 2022, adjustments to net product revenue related to performance obligations satisfied in previous periods were $0.5 million, $0.4 million, and $0.2 million, respectively.

Government Rebates: The Company calculates the rebates that it will be obligated to provide to government programs and deducts these estimated amounts from its gross product sales at the time the revenues are recognized. Allowances for government rebates and discounts are established based on an estimated allocation of payers and the government-mandated discounts applicable to government-funded programs. Rebate discounts are included in accrued expenses in the accompanying consolidated balance sheets.

Commercial Rebates: The Company calculates the rebates it incurs according to any contracts with certain commercial payers and deducts these amounts from its gross product sales at the time the revenues are recognized. Allowances for commercial rebates are established based on actual payer information, which is reasonably estimated at the time of delivery for applicable products. Rebate discounts are included in accrued expenses in the accompanying consolidated balance sheets.

Prompt Pay Discounts: The Company offers discounts to certain customers for prompt payments. The Company accrues for the calculated prompt pay discount based on the gross amount of each invoice for those customers at the time of sale.

Other Fees: The Company pays service fees to certain customers based on a contractually fixed percentage of the wholesale acquisition cost 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 year ended December 31, 2024, 2023 and 2022 (*in thousands*):

	Year Ended December 31,		
	2024	2023	2022
FILSPARI	$ 132,222	$ 29,208	$ —
Tiopronin products	94,485	98,329	97,970
Total net product sales	$ 226,707	$ 127,537	$ 97,970

NOTE 4. COLLABORATION AND LICENSE AGREEMENTS

License Agreement with CSL Vifor

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

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

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

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

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

For the year ended December 31, 2024, the Company recognized $5.8 million in license and collaboration revenue for clinical development activities, based upon the ratio of costs incurred to total estimated costs, and $0.6 million for royalties earned in 2024 on net sales of FILSPARI in the CSL Vifor Licensed Territories following the August 2024 launch. For the year ended December 31, 2023, the Company recognized a total of $17.7 million in license and collaboration revenue, which consisted of $3.3 million from the sale of active pharmaceutical ingredients to CSL Vifor, and $14.4 million for clinical development activities, based upon the ratio of costs incurred to total estimated costs. A decrease in total costs for clinical development activities due to the restructuring announced in December 2023 resulted in a $2.9 million increase in amortization of deferred revenue under the cost-to-cost model for the year ended December 31, 2023.

Deferred revenue related to the clinical development activities as of December 31, 2024 was $2.8 million, classified as current as the Company estimates that the remainder of the deferred revenue balance will be fully realized by mid-2025. 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 $1.8 million classified as non-current as of December 31, 2023 is included in other non-current liabilities in the Consolidated Balance Sheets.

The following table sets forth a summary of changes in deferred revenue for the years ended December 31, 2024, 2023 and 2022 *(in thousands)*:

	Deferred Revenue		
	2024	2023	2022
Balance at January 1,	$ 8,931	$ 22,907	$ 36,647
License and collaboration revenue	(5,847)	(14,363)	(11,490)
Foreign currency impact	(269)	387	(2,250)
Balance at December 31,	$ 2,815	$ 8,931	$ 22,907

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 also 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 concluded that Renalys represents a customer and applied relevant guidance from ASC 606 to evaluate the accounting under the Renalys License Agreement.

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

In accordance with ASC 606, 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 current liabilities, and will be recognized in revenue upon termination of the Buyout Right.

See Note 5 for further discussion of VIE’s.

NOTE 5. VARIABLE INTEREST ENTITIES

Stock Purchase and Collaboration Agreement with PharmaKrysto

On March 8, 2022, the Company entered into a Collaboration Agreement with PharmaKrysto Limited (“PharmaKrysto”), a privately held pre-clinical stage company related to PharmaKrysto's early-stage cystinuria discovery program, and concurrently therewith entered into a Stock Purchase Agreement with PharmaKrysto (together, the "Agreements"). Pursuant to the terms of the Agreements, the Company paid PharmaKrysto's shareholders $0.6 million in cash to purchase 5% of the outstanding common shares of PharmaKrysto and $0.4 million to PharmaKrysto as a one-time signing fee. Under the Collaboration Agreement, the Company will fund all research and development expenses for the pre-clinical activities

associated with the cystinuria program, which are estimated to be approximately $5.0 million. The Agreements require the Company to purchase an additional 5% of the outstanding common shares for $1.0 million upon the occurrence of a specified pre-clinical milestone, and granted an option to the Company 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 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 December 31, 2024 and 2023 were immaterial. The results of operations were not significant for the years ended December 31, 2024, 2023 and 2022. The Company is not required to provide additional funding other than the contractually required amounts disclosed above. The creditors and beneficial holders of PharmaKrysto have no recourse to the general credit or assets of the Company.

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 December 31, 2024, the carrying amount of the liabilities related to the Company's variable interests was $8.9 million, recorded in other current liabilities in the Company's Consolidated Balance Sheets. The Company's maximum exposure to loss as of December 31, 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 6. MARKETABLE DEBT SECURITIES

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

	As of December 31,	
	2024	2023
Marketable debt securities:		
Commercial paper	$ 73,325	$ 34,458
Corporate debt securities	203,816	368,323
Securities of government-sponsored entities	35,025	105,894
Total available-for-sale marketable debt securities	$ 312,166	$ 508,675

The following is a summary of short-term marketable debt securities classified as available-for-sale as of December 31, 2024 (*in thousands*):

	Remaining Contractual Maturity (in years)	Amortized Cost	Unrealized Gains	Unrealized Losses	Aggregate Estimated Fair Value
Marketable debt securities:					
Commercial paper	Less than 1	$ 73,410	$ 1	$ (86)	$ 73,325
Corporate debt securities	Less than 1	203,395	483	(62)	203,816
Securities of government-sponsored entities	Less than 1	34,993	33	(1)	35,025
Total available-for-sale marketable debt securities		$ 311,798	$ 517	$ (149)	$ 312,166

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 marketable debt securities		$ 507,934	$ 1,640	$ (899)	$ 508,675

During 2024 and 2023, realized gains and losses on marketable debt securities were immaterial. As of December 31, 2024 and December 31, 2023, the accrued interest receivable related to the Company's marketable debt securities was $2.3 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 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 December 31, 2024 (*in thousands*):

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

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*):

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 December 31, 2024 and December 31, 2023, the amortized cost of the available-for-sale marketable debt securities in an unrealized loss position was $123.7 million and $269.7 million, respectively.

As of December 31, 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 year ended December 31, 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 7. CONVERTIBLE NOTES PAYABLE

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

	December 31, 2024	December 31, 2023
2.25% convertible senior notes due 2029	$ 316,250	$ 316,250
2.50% convertible senior notes due 2025	68,904	68,904
Unamortized debt issuance costs - 2.25% convertible senior notes due 2029	(5,940)	(7,348)
Unamortized debt issuance costs - 2.50% convertible senior notes due 2025	(226)	(543)
Total convertible senior notes, net of unamortized debt discount and debt issuance costs	$ 378,988	$ 377,263
Classified as:		
Convertible debt, current portion	$ 68,678	$ —
Convertible debt, less current portion	310,310	377,263
Total convertible debt	$ 378,988	$ 377,263

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 December 31, 2024, accrued interest on the 2029 Notes of $2.4 million is included in accrued expenses in the accompanying Consolidated Balance Sheets. The 2029 Notes comprise the Company's senior, unsecured obligations and are (i) equal in right of payment with the Company's existing and future senior, unsecured indebtedness; (ii) senior in right of payment to the Company's existing and future indebtedness that is expressly subordinated to the 2029 Notes; (iii) effectively subordinated to the Company's existing and future secured indebtedness, to the extent of the value of the collateral securing that indebtedness; and (iv) structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables.

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

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

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

Convertible Senior Notes Due 2025

On September 10, 2018, the Company completed a registered underwritten public offering of $276.0 million aggregate principal amount of 2.50% Convertible Senior Notes due 2025 ("2025 Notes"), and entered into a base indenture and supplemental indenture agreement ("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 December 31, 2024, accrued interest of $0.5 million is included in accrued expenses in the accompanying Consolidated Balance Sheets. The 2025 Notes comprise the Company's senior, unsecured obligations and are (i) equal in right of payment with the Company's existing and future senior, unsecured indebtedness; (ii) senior in right of payment to the Company's existing and future indebtedness that is expressly subordinated to the 2025 Notes; (iii) effectively subordinated to the Company's existing and future secured indebtedness, to the extent of the value of the collateral securing that indebtedness; and (iv) structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables.

Holders may convert their 2025 Notes at their option only in the following circumstances: (1) during any calendar quarter commencing after the calendar quarter ending on December 31, 2018 (and only during such calendar quarter), if the last reported sale price per share of the Company's common stock for each of at least 20 trading days, whether or not consecutive, during the period of 30 consecutive trading days ending on, and including, the last trading day of the immediately preceding calendar quarter exceeds 130% of the conversion price on the applicable trading day; (2) during the five consecutive business days immediately after any 10 consecutive trading day period ("measurement period") if the trading price per $1,000 principal amount of 2025 Notes for each trading day of the measurement period was less than 98% of the product of the last reported sale price per share of the Company's common stock on such trading day and the conversion rate on such trading day; (3) upon the occurrence of certain

corporate events or distributions on the Company's common stock; (4) if the Company calls the 2025 Notes for redemption; and (5) at any time from, and including, May 15, 2025 until the close of business on the scheduled trading day immediately before the maturity date. The Company will settle conversions by paying or delivering, as applicable, cash, shares of the Company's common stock, or a combination of cash and shares of the Company's common stock, at the Company's election, based on the applicable conversion rate.

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

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

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

On March 11, 2022, the Company completed its repurchase of $207.1 million aggregate principal amount of 2025 Notes for cash, including accrued and unpaid interest, for a total of $213.8 million. This transaction involved a contemporaneous exchange of cash between the Company and holders of the 2025 Notes participating in the issuance of the 2029 Notes. Accordingly, we evaluated the transaction for modification or extinguishment accounting in accordance with ASC 470-50, *Debt – Modifications and Extinguishments* on a creditor-by creditor basis depending on whether the exchange was determined to have substantially different terms. The repurchase of the 2025 Notes and issuance of the 2029 Notes were deemed to have substantially different terms based on the present value of the cash flows or significant difference between the value of the conversion option immediately prior to and after the exchange. Therefore, the repurchase of the 2025 Notes was accounted for as a debt extinguishment. The Company recorded a $7.6 million loss on extinguishment of debt on its Consolidated Statements of Operations for the year ended December 31, 2022, which included the write-off of related deferred financing costs of $3.4 million. After giving effect to the repurchase, and as of December 31, 2024, the total remaining principal amount outstanding under the 2025 Notes 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 December 31, 2024.

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

Interest Expense

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

	Year Ended December 31,		
	2024	2023	2022
Contractual interest expense	$ 8,838	$ 8,838	$ 8,433
Amortization of debt issuance costs	1,725	1,718	1,622
Total interest expense for the 2025 and 2029 Notes	$ 10,563	$ 10,556	$ 10,055

Total interest expense recognized for the years ended December 31, 2024, 2023 and 2022 was $11.2 million, $11.3 million and $11.0 million, respectively.

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 counterparties 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 December 31, 2024 and 2023, the fair value of the Company's 2.50% Convertible Senior Notes due 2025 was $68.2 million and $58.3 million, respectively. As of December 31, 2024 and 2023, the fair value of the Company's 2.25% Convertible Senior Notes due 2029, which were issued in 2022, was $302.1 million and $212.1 million, respectively. 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 December 31, 2024 *(in thousands):*

	As of December 31, 2024	Fair Value Hierarchy at December 31, 2024		
	Total carrying and estimated fair value	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Assets:				
Cash and Cash Equivalents	$ 58,535	$ 51,060	$ 7,475	$ —
Marketable debt securities, available-for-sale	312,166	—	312,166	—
Total	$ 370,701	$ 51,060	$ 319,641	$ —

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	Fair Value Hierarchy at 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	$ —

Assets Measured at Fair Value on a Nonrecurring Basis

The Company's long-lived assets are measured at fair value on a nonrecurring basis when there are indicators of impairment and a loss is recognized. During the year ended December 31, 2024, the Company recorded impairment charges of $1.2 million on right-of-use assets, property and equipment, and other capitalized assets as the fair value of the asset group was less than their carrying value. The fair value of the asset group was determined under the income approach based on projected future cash flows from the operating sublease discounted by a risk adjusted rate of 6.9%. The Company classified the fair value of the asset group as Level 3. See Note 18 for further discussion.

NOTE 9. INTANGIBLE ASSETS

Amortizable Intangible Assets

Ligand License Agreement

In 2012, the Company entered into the Ligand License Agreement with Ligand for a worldwide sublicense to develop, manufacture and commercialize sparsentan. 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 December 31, 2024, the Company has capitalized $47.2 million for contractual milestones achieved under the Ligand License Agreement, including $5.8 million for the year ended December 31, 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 years ended December 31, 2024 and 2023, the Company capitalized $20.3 million and $4.4 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

Mission License Agreement

In 2014, the Company entered into the Mission License Agreement with Mission in which the Company 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. The initial term of the license was 10 years.

The Company paid Mission an up-front license fee of $3.0 million and during the term of the agreement will pay the greater of $2.0 million, representing the guaranteed minimum royalty, or 20% of the Company's net sales of Thiola during each calendar year.

In November 2017, the Company amended its agreement with Mission to extend the term of the current exclusive U.S. and Canada licensing agreement by an additional five years, to 2029. The royalty rate and guaranteed minimum payment were also extended through the new agreement term. Upon execution of the amendment, the Company capitalized an additional $5.9 million in intangible assets and recorded a guaranteed minimum liability for the same amount.

In November 2018, the Company amended its agreement with Mission to remove all territorial restrictions on our license. As consideration for the expanded territory, the Company paid an up-front fee of $0.3 million and will pay the greater of $0.1 million, representing the guaranteed minimum, or 20% of the Company's net sales of Thiola generated outside of the United States during each calendar year. Upon execution of the amendment, the Company capitalized an additional $1.0 million in intangible assets and recorded a guaranteed minimum liability of $0.7 million related to this amendment.

The present value of guaranteed minimum royalties payable using a discount rate ranging from approximately 7% to 11% based on the Company's then borrowing rate is $8.2 million and $9.7 million as of December 31, 2024 and 2023, respectively. As of December 31, 2024, the guaranteed minimum royalty current and long-term liability was approximately $2.1 million and $6.1 million, respectively, and is recorded as Other Liability in the Consolidated Balance Sheets. As of December 31, 2023, the guaranteed minimum royalty current and long-term liability was approximately $2.1 million and $7.6 million, respectively, and is recorded as Other Liability in the Consolidated Balance Sheets.

The Company has capitalized $171.0 million related to the Thiola intangible asset which consists of the up-front license fee, professional fees, present value of the guaranteed minimum royalties and any additional payment obligations through December 31, 2024 in excess of minimum royalties. In 2024 the Company added $15.4 million to the intangible asset related to royalties in excess of the minimum. Consistent with all prior periods since Thiola was acquired, the Company has not accrued any liability for royalties in excess of the annual contractual minimum at December 31, 2024, as such royalties are not yet probable and estimable.

Amortizable intangible assets as of December 31, 2024 (*in thousands*):

	Useful Life	Gross Carrying Amount	Accumulated Amortization	Net Book Value
Mission license	12	$ 171,025	$ (115,303)	$ 55,722
Ligand license	11	53,268	(5,769)	47,499
Total amortizable intangible assets		$ 224,293	$ (121,072)	$ 103,221

Amortizable intangible assets as of December 31, 2023 (*in thousands*):

	Useful Life	Gross Carrying Amount	Accumulated Amortization	Net Book Value
Mission license	12	$ 155,589	$ (77,311)	$ 78,278
Ligand license	11	27,448	(2,036)	25,412
Total amortizable intangible assets		$ 183,037	$ (79,347)	$ 103,690

The following table summarizes amortization expense for the year ended December 31, 2024, 2023 and 2022 (*in thousands*):

	2024	2023	2022
Selling, general and administrative	$ 41,739	$ 29,021	$ 12,339
Research and development	—	7,261	6,264
Total amortization expense	$ 41,739	$ 36,282	$ 18,603

As of December 31, 2024, amortization expense for the next five years and thereafter is expected to be as follows (*in thousands*):

2025	$ 50,397
2026	16,722
2027	5,700
2028	5,714
2029	5,700
Thereafter	18,988
Total	$ 103,221

There were no impairments related to finite-lived intangible assets in the years ended December 31, 2024, 2023 and 2022.

Goodwill

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

For the years ended December 31, 2024, 2023 and 2022 there were no impairments to goodwill.

NOTE 10. ACCRUED EXPENSES

Accrued expenses consist of the following at December 31, 2024 and 2023 (*in thousands*):

	2024	2023
Compensation related costs	$ 35,166	$ 29,908
Research and development	16,090	26,006
Accrued royalties	12,309	6,991
Sales discounts, rebates, and allowances	10,585	13,730
Selling, general and administrative	6,154	7,190
Transition services accrual	285	12,282
Accrued restructuring costs	—	11,421
Miscellaneous accrued expenses	5,439	11,463
Total accrued expenses	$ 86,028	$ 118,991

NOTE 11. 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 U.S. and Europe, subject to certain reductions as set forth in the Agreement, and a contingent payment in the event a pediatric rare disease voucher for any pegtibatinase product is granted.

In accordance with ASC 450, *Contingencies*, contingent cash payments will be accrued for when it is probable that a liability has been incurred and the amount can be reasonably estimated. In March 2024, the Company recognized $65.2 million in IPR&D expense upon the achievement of a development milestone, which was paid during the second quarter of 2024 and recorded within investing activities in the Consolidated Statements of Cash Flows. As of December 31, 2024, no contingent cash payments have been accrued.

Legal Proceedings

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

NOTE 12. STOCKHOLDERS’ EQUITY

Common Stock

The Company is currently authorized to issue up to 200,000,000 shares of $0.0001 par value common stock. All issued shares of common stock are entitled to vote on a 1 share/1 vote basis.

Preferred Stock

The Company is currently authorized to issue up to 20,000,000 shares of $0.0001 par value preferred stock, of which 1,000 shares are designated Class "A" Preferred shares. Class A Preferred Shares are not entitled to interest, have certain liquidation preferences, special voting rights and other provisions. No preferred stock has been issued to date.

2018 Equity Incentive Plan

The Company's 2018 Equity Incentive Plan (the "2018 Plan") is the successor to and continuation of the Company's 2015 Equity Incentive Plan (the "2015 Plan") and the Company's 2014 Equity Incentive Plan (the "2014 Plan", and together with the 2015 Plan, the "Prior Plans"). Unallocated shares under the Prior Plans are no longer available for issuance under the Prior Plans, and have instead been added to the shares available for issuance under the 2018 Plan. The 2018 Plan, as amended, and including the unallocated shares of the Prior Plans, provides for a total of 18.4 million shares to be issued, plus the Prior Plans' returning shares, if any, which become available for grant under the 2018 Plan from time to time. Options issued under the 2018 Plan will generally expire ten years from the date of grant and vest over a four-year period. As of December 31, 2024, there were approximately 5.7 million shares reserved for future issuance under the 2018 Plan.

2017 Employee Stock Purchase Plan

The 2017 Employee Stock Purchase Plan ("2017 ESPP") originated with 380,000 shares of common stock available for issuance. Beginning on January 1, 2018, and ending on (and including) January 1, 2026, the number of shares of common stock available for issuance under the 2017 ESPP may increase by an amount equal to the lesser of (i) one percent (1%) of the total number of shares of common stock outstanding on December 31st of the preceding calendar year or (ii) 300,000 shares of common stock.

Substantially all employees are eligible to participate in the 2017 ESPP and, through payroll deductions, can purchase shares on established dates semi-annually. The purchase price per share sold pursuant to the 2017 ESPP will be the lower of (i) 85% of the fair market value of common stock on the first day of the offering period or (ii) 85% of the fair market value on the purchase date. Each offering period will span up to six months. Purchases may be up to 15% of qualified compensation, with an annual limit of $25,000. The 2017 ESPP is intended to qualify as an “employee stock purchase plan” under Section 423 of the Internal Revenue Code.

As of December 31, 2024, there were approximately 2,480,000 shares authorized and 1,004,889 shares reserved for future issuance under the 2017 ESPP.

Stock Options

The fair values of stock option grants during the years ended December 31, 2024, 2023 and 2022 were calculated on the date of grant using the Black-Scholes option pricing model. Compensation expense is recognized over the period of service, generally the vesting period. The following weighted average assumptions were used in the Black-Scholes options pricing model to estimate the fair value of stock options for the specified reporting periods:

	Year Ended December 31,		
	2024	**2023**	**2022**
Risk free rate	3.9 %	3.9 %	1.7 %
Expected volatility	59 %	50 %	50 %
Expected life (in years)	6.2	6.4	6.4
Expected dividend yield	—	—	—

The risk-free interest rate was based on rates established by the Federal Reserve. The Company’s expected volatility was based on analysis of the Company’s historical volatility. The expected life of the Company’s options was determined using the Company's historical exercise activity. The dividend yield is based upon the fact that the Company has not historically paid dividends and does not expect to pay dividends in the foreseeable future.

The following table summarizes our stock option activity and related information for the year ended December 31, 2024:

	Shares Underlying Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2023	10,211,353	$ 21.52	4.91	$ —
Granted	1,524,300	8.63	—	—
Forfeited and expired	(2,076,808)	20.02	—	—
Exercised	(375,514)	11.86	—	2,016
Outstanding at December 31, 2024	9,283,331	$ 20.13	5.51	$ 16,408
Vested and expected to vest at December 31, 2024	9,283,331	$ 20.13	5.51	$ 16,408

The aggregate intrinsic value of stock options exercised in the years ended December 31, 2024, 2023 and 2022 was $2.0 million, $1.4 million, and $5.1 million, respectively.

The following table summarizes our stock options exercisable at December 31, 2024, 2023 and 2022:

	Shares Underlying Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Exercisable at December 31, 2022	6,999,669	$ 20.22	4.62	$ 21,806
Exercisable at December 31, 2023	7,942,964	$ 20.85	3.95	$ —
Exercisable at December 31, 2024	6,892,156	$ 22.11	4.46	$ 3,249

The weighted average grant date fair value of options granted was $5.10, $11.66, and $13.45 during the years ended December 31, 2024, 2023 and 2022, respectively. The aggregate intrinsic value for outstanding options is calculated as the difference between the exercise price of the underlying awards and the closing price of the Company's common stock of $17.42, $8.99 and $21.03 as of December 31, 2024, 2023 and 2022, respectively. Unrecognized compensation cost associated with unvested stock options amounts to $16.2 million as of December 31, 2024, which will be expensed over a weighted average remaining vesting period of 2.4 years.

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

Restricted Stock Units

As of December 31, 2024, there was approximately $35.8 million of unrecognized compensation cost related to restricted stock units ("RSUs") granted. This amount is expected to be recognized over a weighted average period of 2.3 years.

The following table summarizes our restricted stock unit activity for the year ended December 31, 2024:

	Number of RSUs	Weighted Average Grant Date Fair Value
Unvested December 31, 2023	2,874,046	$ 22.97
Granted	2,077,025	9.07
Vested	(1,013,811)	22.52
Forfeited/cancelled	(419,997)	18.66
Unvested December 31, 2024	3,517,263	$ 15.41

The fair value of restricted stock units vested for the years ended December 31, 2024, 2023 and 2022 was $22.8 million, $18.7 million, and $11.5 million, respectively. The weighted average grant date fair value for stock awards granted during the years ended December 31, 2024, 2023 and 2022 was $9.07, $21.20 and $26.07, respectively.

Performance-based Stock Units

Performance-based stock units ("PSUs") are subject to vest only if certain specified criteria are achieved. As of December 31, 2024, there was approximately $1.2 million of unrecognized compensation cost related to PSUs granted and deemed probable of vesting. This amount is expected to be recognized over a weighted average period of 0.9 years.

The following table summarizes our performance-based stock unit activity for the year ended December 31, 2024:

	Number of PSUs	Weighted Average Grant Date Fair Value
Unvested December 31, 2023	175,458	$ 25.61
Granted	107,000	8.93
Vested	(66,250)	22.40
Forfeited/cancelled	—	—
Unvested December 31, 2024	216,208	$ 18.34

The fair value of PSUs vested for the years ended December 31, 2024, 2023 and 2022 was $1.5 million, $0.3 million, and $0.4 million, respectively. The weighted average grant date fair value for performance-based stock awards granted during the years ended December 31, 2024, 2023 and 2022 was $8.93, $22.40 and $27.54, respectively.

Share Based Compensation

Total non-cash stock-based compensation expense consisted of the following for the years ended December 31, 2024, 2023 and 2022 (*in thousands*):

	Year Ended December 31,		
	2024	2023	2022
Selling, general and administrative expenses	$ 22,735	$ 27,111	$ 24,368
Research and development expenses	14,178	17,135	13,775
Total stock-based compensation expense	$ 36,913	$ 44,246	$ 38,143

NOTE 13. 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 15, as part of its February 2023 underwritten public offering, the Company issued and sold pre-funded warrants to purchase 1.25 million shares of its common stock at a price to the public of $20.9999 per pre-funded warrant. The pre-funded warrants were immediately exercisable upon issuance, and were exercised in the third quarter of 2024, resulting in the issuance of 1.25 million shares of the Company's common stock. Due to the nominal exercise price of the pre-funded warrants and the lack of any contingencies to exercise, the shares underlying the pre-funded warrants have been included in the calculation of basic net loss per common share since the date the warrants were issued.

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

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

	For the year ended December 31,								
	2024			2023			2022		
	Shares	Net (loss) income	EPS	Shares	Net (loss) income	EPS	Shares	Net (loss) income	EPS
Continuing operations	78,888,861	$ (320,630)	$ (4.07)	74,267,418	$ (376,333)	$ (5.07)	63,758,515	$ (331,468)	$ (5.20)
Discontinued operations	78,888,861	(915)	(0.01)	74,267,418	264,934	3.57	63,758,515	52,986	0.83
Basic and diluted loss per share	78,888,861	$ (321,545)	$ (4.08)	74,267,418	$ (111,399)	$ (1.50)	63,758,515	$ (278,482)	$ (4.37)

For the years ended December 31, 2024, 2023 and 2022, the following weighted-average number of common stock equivalents were excluded because they were anti-dilutive:

	For the year ended December 31,		
	2024	2023	2022
Convertible debt	11,697,953	11,697,952	10,869,000
Options	10,447,541	10,555,550	10,041,249
Restricted stock units and performance-based stock units	3,780,441	3,417,245	2,175,100
Total anti-dilutive shares	25,925,935	25,670,747	23,085,349

NOTE 14. INCOME TAXES

For financial reporting purposes, net loss from continuing operations before income taxes includes the following components (*in thousands*):

	Year Ended December 31,		
	2024	2023	2022
United States	$ (152,356)	$ (293,283)	$ (268,887)
Foreign	(168,154)	(82,827)	(62,268)
Total	$ (320,510)	$ (376,110)	$ (331,155)

The components of the provision for income taxes, in the Consolidated Statements of Operations are as follows (*in thousands*):

	2024	2023	2022
Current			
Federal	$ —	$ —	$ —
State	120	223	314
Foreign	—	—	(1)
Total current	120	223	313
Deferred			
Federal	—	—	—
State	—	—	—
Total deferred	—	—	—
Total tax provision	$ 120	$ 223	$ 313

The following is a reconciliation of the statutory federal income tax rate to the Company's effective tax rate expressed as a percentage of loss before income taxes:

	2024	2023	2022
Statutory rate - federal	(21.00)%	(21.00)%	(21.00)%
State taxes, net of federal benefit	(1.55)%	(3.19)%	(3.62)%
Foreign rate differential	2.15 %	1.29 %	0.81 %
IPR&D	4.27 %	— %	— %
Nondeductible executive compensation	0.44 %	1.50 %	0.41 %
Excess tax benefits associated with share-based awards	2.92 %	0.68 %	(0.17)%
Other permanent differences	0.23 %	0.37 %	0.17 %
Tax credits	(4.24)%	(1.13)%	(5.29)%
Return to provision adjustments and other true-ups	(0.80)%	4.19 %	(0.17)%
Adoption of ASU 2020-06	— %	— %	(3.54)%
Other	0.93 %	0.57 %	— %
Change in valuation allowance	16.69 %	16.78 %	32.49 %
Income tax provision	0.04 %	0.06 %	0.09 %

The significant components of the Company's deferred tax assets and liabilities as of December 31, 2024 and 2023 are as follows (*in thousands*):

	2024	2023
Deferred Tax Assets:		
Net operating loss	$ 113,303	$ 81,531
Research and development and other tax credits	91,760	77,555
Intangible assets	53,919	47,731
Capitalized research and development	47,880	39,907
Stock based compensation	15,680	21,607
Other accrued expenses	14,266	13,187
Charitable contributions	5,797	6,110
Operating lease liabilities	5,421	6,812
Depreciation	435	292
Loan costs	153	62
Interest expense limitation	—	1,679
Total deferred tax assets	348,614	296,473
Deferred Tax Liabilities:		
Operating lease right of use assets	(4,259)	(5,595)
Prepaid assets	(178)	(198)
Total deferred tax liabilities	(4,437)	(5,793)
Net deferred tax assets before valuation allowance	344,177	290,680
Valuation allowance	(344,177)	(290,680)
Total deferred tax assets	$ —	$ —

The Company has established a full valuation allowance against its U.S. federal, state, and foreign deferred tax assets due to the uncertainty surrounding the realization of such assets in future periods. The ultimate realization of deferred tax assets is dependent upon the generation of future taxable income during the periods in which temporary differences become deductible. Management considers the scheduled reversal of deferred liabilities and tax planning strategies in making this assessment and evaluates the recoverability of the deferred tax assets as of each reporting date. At such time as it is determined that it is more likely than not that deferred assets are realizable, the valuation allowance will be reduced accordingly and recorded as a tax benefit.

The Company has recorded a valuation allowance of $344.2 million as of December 31, 2024 to reflect the estimated amount of deferred tax assets that may not be realized. The Company increased its valuation allowance by $53.5 million for the year ended December 31, 2024, compared to a $2.7 million decrease for the year ended December 31, 2023.

As of December 31, 2024, the Company had available unused U.S. federal and state net operating loss ("NOL") carryforwards of $223.8 million and $241.8 million, respectively, all of which are fully offset by a valuation allowance. The federal NOL has an indefinite life. The state NOL carryforwards will begin to expire in 2025 unless previously utilized, except for $45.4 million of the state net operating losses that have an indefinite carryforward period. In addition, at December 31, 2024, the Company had federal orphan drug tax credit carryforwards of $104.1 million that begin to expire in 2035 unless utilized, federal research and development tax credit carryforwards of $4.9 million that begin to expire in 2033 unless utilized, state research and development tax credit carryforwards of $1.4 million that begin to expire in 2030 unless utilized and $9.9 million that have an indefinite carryforward period, and California Competes tax credit carryforwards of $1.6 million that begin to expire in 2025. Pursuant to Internal Revenue Code Sections 382 and 383, use of the Company's federal net operating loss and credit carryforwards may be limited upon a cumulative change in ownership of more than 50% within a three-year period. The Company continues to monitor potential historical ownership changes.

As of December 31, 2024, the Company had Irish NOL carryforwards of $14.2 million which are fully offset by a valuation allowance and have an indefinite life. The Company also had Swiss NOL carryforwards of $359.1 million which are fully offset by a valuation allowance and begin to expire in 2025, as well as Federal Act on Tax Reform and AHV Financing ("TRAF") cantonal tax benefits of $526.2 million which expire in 2029.

The Company accounts for uncertain tax benefits in accordance with the provisions of ASC 740-10 of the *Accounting for Uncertainty in Income Taxes*. As of December 31, 2024, the Company had $27.4 million in unrecognized tax benefits, which were recorded as a reduction to the deferred tax assets with a corresponding reduction in the Company's valuation allowance of $27.4 million. To the extent unrecognized tax benefits are recognized at a time when a valuation allowance does not exist, the recognition of the $27.4 million tax benefit would reduce the effective tax rate. The Company does not anticipate that the amount of unrecognized tax benefits as of December 31, 2024 will change materially within the following 12 months.

A reconciliation of the Company's unrecognized tax benefits for the years 2024, 2023 and 2022 is provided in the following table (*in thousands*):

	2024	2023	2022
Balance as of January 1:	$ 22,906	$ 11,490	$ 7,825
Increase in current period positions	4,248	4,871	2,056
Increase in prior period positions	250	7,383	1,919
Decrease in prior period positions	—	(838)	—
Decrease due to settlements with tax authorities	—	—	(310)
Balance as of December 31:	$ 27,404	$ 22,906	$ 11,490

The Company files income tax returns in the U.S. federal jurisdiction, various state and local, and foreign jurisdictions. With few exceptions, the Company's income tax returns are open to examination by federal and state authorities for the years ended December 31, 2013 and forward, due to the carryforward of unutilized tax attributes. The Company's Swiss income tax returns are open to examination for the years ended December 31, 2019 and forward, and the Company's Irish tax returns are open to examination for the years ended December 31, 2020 and forward.

The Company recognizes interest and penalties as a component of income tax expense. The Company did not recognize any interest or penalties for the year ended December 31, 2024 and 2023. Interest and penalties for the year ended December 31, 2022 were less than $0.1 million.

NOTE 15. EQUITY OFFERINGS

Underwritten Public Offering of Common Stock

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

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 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 October 2024, the Company filed a prospectus supplement to the prospectus included in its registration statement on Form S-3 (File No. 333-281194), pursuant to which the Company may offer and sell, from time to time through Jefferies LLC, as agent (“Jefferies”), up to $100.0 million of common stock pursuant to an Amended and Restated Open Market Sale Agreement ("ATM Agreement") with Jefferies dated October 2024. The Company did not sell any shares under the ATM Agreement during the year ended December 31, 2024.

NOTE 16. RETIREMENT PLAN

401(k) Savings Plan

The Company has a 401(k) defined contribution savings plan for the benefit of all eligible employees. Employer matching contributions were $2.4 million, $2.7 million, and $2.1 million for the years ended December 31, 2024, 2023 and 2022, respectively.

Switzerland Defined Benefit Plan

The Company maintains a defined benefit pension plan covering employees of its Swiss subsidiary, Travere Therapeutics Switzerland GmbH (the "Swiss Plan"). The Swiss Plan is a government-mandated retirement fund that provides employees with a minimum benefit. Employer and employee contributions are made to the Swiss Plan based on various percentages of participants' salaries and wages that vary according to the participants' age and other factors. As of December 31, 2024, the projected benefit obligations under the Swiss Plan were approximately $3.0 million, and plan assets were approximately $2.5 million. The funded status of the Swiss Plan is included in other long-term liabilities on the Company's Consolidated Balance Sheets.

NOTE 17. PROPERTY AND EQUIPMENT

Property, plant and equipment, net consisted of the following *(in thousands)*:

	December 31,	
	2024	2023
Leasehold improvements	$ 9,370	$ 9,692
Furniture and fixtures	2,446	2,990
Computers and equipment	2,275	2,071
Construction-in-progress	—	169
	14,091	14,922
Less: Accumulated depreciation	(8,755)	(7,443)
Total property and equipment, net	$ 5,336	$ 7,479

Depreciation expense for the years ended December 31, 2024, 2023 and 2022 was $1.8 million, $2.2 million and $2.1 million, respectively.

NOTE 18. LEASES

As of December 31, 2024, the Company had 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 and utilizing a discount rate equal to the Company's estimated incremental borrowing rate, the Company established right-of-use assets totaling $34.6 million and lease liabilities totaling $34.5 million. The total right-of-use asset and lease liability at measurement were each offset by lease incentives associated with tenant improvement allowances totaling $7.9 million.

The initial term of the office lease ends in August 2028, and the Landlord has granted the Company an option to extend the term of the lease by a period of 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 right-of-use asset and lease liability measurements. The measurement of the lease term occurs from the February 2021 occupancy date of the office space.

As of December 31, 2024, the Company also had an operating lease with Esprit Investments Limited for office space located in Dublin, Ireland, which was entered into in October 2022. 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 will elect this option and therefore the renewal period has been excluded from the initial lease measurement. Utilizing a discount rate equal to the Company's borrowing rate, the Company established a right-of-use asset and corresponding lease liability of $0.4 million.

During the year ended December 31, 2024, due to the decreased occupancy from the 2023 restructuring along with efforts to optimize a hybrid work model, the Company decided to reduce its use of office space located in San Diego, California. In November 2024, the Company entered into a sublease of its 5th floor premises. The term of the sublease runs from January 2025 through August 2028.

The Company's sublease arrangement has been classified as an operating lease with sublease income recognized on a straight-line basis over the term of the sublease arrangement. To measure the Company's periodic sublease income, the Company elected to use a practical expedient under ASC 842 to aggregate non-lease components with the related lease components when (i) the timing and pattern of transfer for the non-lease components and the related lease components are the same and (ii) the lease components, if accounted for separately, would be classified as an operating lease.

Given the circumstances surrounding the 5th floor premises, the Company identified an indicator of impairment of the related operating lease right-of-use assets, property and equipment, and other capitalized assets and compared the carrying value of the asset group to an estimate of the future undiscounted cash flows expected to result from the sublease and eventual disposition of the asset group. The sum of the undiscounted cash flows of the asset group was below the carrying value. Consequently, the Company utilized the present value of the estimated future cash flows attributable to the assets to determine the fair value of the asset group. The resulting fair value of the operating lease right-of-use assets, property and equipment, and other capitalized assets resulted in an impairment of $1.2 million recorded in restructuring expense in the Consolidated Statements of Operations and categorized the aforementioned measurement of fair value as Level 3 within the ASC Topic 820, "Fair Value Measurements" fair value hierarchy. There were no impairments related to right-of-use assets or property and equipment in the years ended December 31, 2023 and 2022.

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 December 31, 2024 (*in thousands*):

	December 31, 2024
2025	$ 6,665
2026	6,889
2027	7,064
2028	4,781
Thereafter	—
Total undiscounted future minimum payments	25,399
Present value discount	(2,803)
Total lease liability	22,596
Unamortized lease incentives	(3,491)
Cash payments in excess of straight-line lease expense	(4,810)
Total ROU asset	$ 14,295

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

	December 31,	
	2024	2023
Weighted-average remaining lease term in years	3.7	4.7
Weighted-average discount rate	6.48 %	6.48 %

For the years ended December 31, 2024, 2023 and 2022 the Company recorded $4.8 million, $4.9 million, and $5.0 million, respectively, in expense related to operating leases, including amortized tenant improvement allowances.

NOTE 19. 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 the 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 years ended December 31, 2024 and 2023, the Company recognized $0.5 million and $1.0 million, respectively, under the TSA, included in continuing operations within other income (expense), net. As of December 31, 2024, the Company collected $1.5 million. 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 December 31, 2024 was $0.3 million, and is included in accrued expenses in the accompanying Consolidated Balance Sheets. TSA services provided by the Company are substantially complete as of December 31, 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*):

	Year Ended December 31,		
	2024	2023	2022
Net product sales	$ (550)	$ 66,164	$ 102,558
Total revenue	(550)	66,164	102,558
Operating expenses:			
Cost of goods sold	(9)	1,899	3,172
Research and development	247	6,118	8,447
Selling, general and administrative	127	19,500	22,686
Change in fair value of contingent consideration	—	(473)	15,006
Total operating expenses	365	27,044	49,311
Operating income	(915)	39,120	53,247
Other income (expenses), net:			
Interest expense	—	(191)	(261)
Gain on disposal of discontinued operations, net of tax	—	226,005	—
Total other income (expense), net	—	225,814	(261)
(Loss) income from discontinued operations before income tax	(915)	264,934	52,986
Income tax on discontinued operations	—	—	—
Net (loss) income from discontinued operations	$ (915)	$ 264,934	$ 52,986

NOTE 20. SEGMENT INFORMATION

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

The Company sells its products to specialty pharmacies and through direct-to-patient distributors worldwide. Net product sales outside of the United States ("U.S.") were not significant for the years ended December 31, 2024, 2023 and 2022. Long-lived assets located outside the U.S. were $4.5 million as of December 31, 2024. The Company had no long-lived assets located outside the U.S. as of December 31, 2023. The measure of segment assets is reported on the consolidated balance sheets as total assets. The accounting policies of the segment are the same as those described in Note 2, Summary of Significant Accounting Policies.

The following table presents reportable segment loss, including significant expenses regularly provided to the CODM, attributable to the Company's reportable segment for the years ended December 31, 2024, 2023 and 2022 (*in thousands*):

	Year Ended December 31,		
	2024	**2023**	**2022**
Revenue	$ 233,175	$ 145,238	$ 109,460
Less:			
Cost of goods sold	7,744	11,450	4,420
Research and development:			
External research and development	126,303	142,482	129,698
Internal personnel costs	73,843	84,658	75,664
Other Research and development	17,350	17,850	21,971
Total research and development	217,496	244,990	227,333
Selling, general and administrative	264,119	265,542	197,520
In-process research and development	65,205	—	—
Restructuring	2,438	11,394	—
Total other income (expense), net	3,317	12,028	(11,342)
Income tax provision on continuing operations	(120)	(223)	(313)
(Loss) income from discontinued operations, net of tax	(915)	264,934	52,986
Net loss	$ (321,545)	$ (111,399)	$ (278,482)

EXHIBIT 10.9

2025 Travere Therapeutics, Inc.
Executive Officer Annual Bonus Plan

Plan Objective

The purpose of the Travere Therapeutics, Inc. Executive Officer Bonus Plan (the "Plan") is to provide incentives to and reward executive officers of Travere Therapeutics, Inc. (the "Company") (each a "Participant," as defined below) to achieve corporate performance goals and to work together to achieve outstanding results in all aspects of the Company's business, thus benefiting themselves, Company shareholders and the people who benefit from the Company's services.

Eligibility

- All regular full-time executive officers of Travere Therapeutics are eligible to receive a bonus under this Plan ("Participant").
- Participants must be employed as a regular full-time employee by the Company prior to October 1 of the bonus plan year.
- Employees hired during the Bonus Plan Year prior to October 1 will be eligible to receive a prorated bonus based on the number of calendar days actively paid by Travere during the Bonus Plan Year.
- In order to be eligible to receive a bonus for a particular Bonus Plan Year (if any is earned), a Participant must be actively employed, and in good standing, as of the date the bonus checks are distributed for that year or as otherwise approved by the Board.
- Temporary executive officers and consultants (regardless of their roles or responsibilities) are not eligible to participate.
- Participation in the "Travere Therapeutics, Inc. Executive Officer Bonus Plan" is approved on an annual basis. Criteria for participation may be subject to change at the commencement of the Bonus Plan Year, and eligibility to participate in any Bonus Plan Year does not guarantee eligibility to participate in any subsequent Bonus Plan Year. Participants whose individual performance is deemed to not be meeting expectations by the Compensation Committee are ineligible.

Definitions

- "Bonus Plan Year" means the twelve-month period beginning on each January 1 and ending on each December 31.
- The "Board" means the Board of Directors of the Company.
- The "Compensation Committee" means the Compensation Committee of the Board, as constituted from time to time.
- The "Base Pay" is a Participant's annual rate of base salary in effect as of December 31 of the applicable Bonus Plan Year. However, bonus awards will be based on a blended rate if an employee has a reduction in work schedule and/or annualized salary during the plan year such

that the bonus calculation will take into account the time worked for both the higher and lower salary.

- The "Company Target Performance Measures" shall be determined at the sole discretion of the Compensation Committee or the Board and shall be set forth in writing, and may include, but shall not be limited to, a combination of financial, research and development and/or operational goals.
- The "Company Modifier" is determined at the sole discretion of the Compensation Committee or the Board and is designed to reflect performance against Company results. For illustration purposes only, if the Company performance significantly exceeds the Company Target Performance Measures, the Company Modifier could exceed 100%, but in no case more than 150%. Similarly, if Company performance fails to meet the Company Target Performance Measures, the Company Modifier could be less than 100%. There is a minimum Corporate Performance required of 40% for any payment under the Plan to be considered. No Participant will have any entitlement to or earn a right to receive a bonus under this Plan until the date on which such bonus is paid. The Board and/or Compensation Committee reserve the right, at any time, regardless of corporate performance to approve or not approve the payment of a Bonus during any Plan Year.
- The "Individual Modifier" is determined by the Participant's relative performance during the Plan year, and will generally fall within 0%-125%, as per the Participant's annual performance rating.
- The "Target Bonus" means the percentage of Base Pay that would be awarded to a Participant upon the achievement of the Company Target Performance Measures at a level of 100%.

Bonus Award Components

Unless otherwise specified, the components of a Bonus Award Payment (described below) are as follows:

- Company Modifier based on achievement of Company Performance Measures
- Target Percentage based on Participant's position (see below)
- Participant's Base Pay for the bonus year
    - Number of credible eligible days of service for the Bonus Plan Year
    - Participant's Individual Performance Modifier
    - Weighting of Company Performance Modifier based on level
- Weighting of Individual Performance Modifier based on level

Position	Target Bonus %	Individual Modifier Weighting	Company Modifier Weighting
Chief Executive Officer	75%	N/A	100%
Other Executive Officers	50%	N/A	100%

Bonus Award Payment Calculation

The Bonus Award Payment, if one is approved, is calculated as follows:

[(Participant's base pay x Bonus Target percentage x individual performance modifier x individual performance weighting) x Plan year tenure]	+	[(Participant's base pay x Bonus target percentage x company performance modifier x company performance weighting) x Plan year tenure]

General

- Bonus awards, if earned, will be paid between January 1 and March 15 of the calendar year after the close of the applicable Bonus Plan Year.
- In the event of a Participant's leave of absence in excess of 60 days during the Bonus Plan Year, the bonus earned for that year will be prorated. The calculation will be based on the total number of calendar days of active employment status.
- Executive officers hired after October 1 will not be eligible for a bonus award under this Plan until the following Bonus Plan Year.
- Executive officers hired during the Bonus Plan Year on or before October 1 will be eligible to receive a prorated bonus based on the number of calendar days actively at work.
- Bonus awards are based on the Participant's target percentage and Base Salary as of December 31 of the Bonus Plan Year.
- Travere Therapeutics reserves the right to modify or terminate the Plan at any time without prior notice.
- The Plan does not modify a Participant's at-will employment status or create a contract of employment for a specific term. Receipt of a bonus award is not guaranteed, and this Plan is not a promise of future or continued employment.
- The Plan does not modify a Participant's Employment Agreement.
- The Company will withhold all required taxes and make any other required deductions from payments made under the Plan. This Plan is intended to provide "short term deferrals", as described in Treasury Regulation 1.409A-1(b)(4) under section 409A of the Code or successor guidance thereto, and is intended not to be a "nonqualified deferred compensation plan", as described in Treasury Regulation 1-409A-1(a)(1) under section 409A of the Code or successor guidance thereto. In the administration and interpretation of the Plan, such intention is to govern.
- It is intended that this Plan be exempt from regulation under the Employee Retirement Income Security Act of 1974, as amended, as a "payroll practice" and a "bonus program", as described in U.S. Department of Labor Regulations 2510.3-1(b) and 2510.3-2(c), respectively.
- Any bonuses paid under the Bonus Plan shall be subject to the provisions of any claw-back policy implemented by the Company, including, without limitation, any claw-back policy adopted to comply with the requirements of the Dodd-Frank Wall Street Reform and Consumer Protection Act and any rules, regulations or interpretations thereunder.
- This Plan shall be subject to and construed in accordance with the laws of the State of California without regard to conflicts of laws.
- The Compensation Committee possesses sole discretion and authority to construe and interpret the terms and provisions of the Plan and to resolve any issue arising out of, relating to, or resulting from its administration and operation. Any disagreement or dispute by any person claiming a benefit under the Plan regarding any aspect of the Plan

or its administration must be promptly presented in writing to the Compensation Committee for determination. Payments shall be made under the Plan only if the Compensation Committee determines in its sole discretion that the claimant is entitled to them. Any determinations the Compensation Committee makes in relation to the Plan will be final, conclusive, and binding on all persons, entities and parties claiming any interest under the Plan and will be entitled to the maximum possible deference allowed by law.

- Except as explicitly provided by law, this Plan is provided at the Company's sole discretion, and the Company reserves the power at any time and from time to time, to modify, amend or terminate (in whole or in part) any or all of the provisions of the Plan at any time, prospectively or retroactively, without prior notice or obligation. Any amendment to the Plan shall be adopted by formal action of the Board.
- The Plan will be operated as an unfunded arrangement, and nothing in this document will be construed to require the Company to fund any awards or to establish a trust or purchase an insurance policy or other product for such purpose. The Company may make such arrangements as it desires to provide for the payment of bonuses under the Plan.
- Any payments made pursuant to the Plan shall not be counted as compensation for purposes of any other employee benefit plan, program or agreement sponsored, maintained or contributed to by the Company unless expressly provided for in such employee benefit plan, program, agreement, or arrangement.

EXHIBIT 19.1

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.

INSIDER TRADING POLICY

Effective Date: January 21, 2025

1. Introduction

1.1 During the course of your relationship with Travere Therapeutics, Inc. ("***Travere***" or the "***Company***"), you may receive material information that is not yet publicly available ("***material nonpublic information***") about Travere or other publicly traded companies that Travere has business relationships with. Material nonpublic information may give you, or someone you pass that information on to, a leg up over others when deciding whether to buy, sell or otherwise transact in Travere's securities or the securities of another publicly traded company. This policy sets forth guidelines with respect to transactions in Travere securities and in the securities of other applicable publicly traded companies, in each case by our employees, directors and consultants who are advised that they are subject to this policy ("***designated consultants***") and the other persons or entities subject to this policy as described below. In addition, from time to time the Company may engage in transactions in Company securities. It is the Company's policy to comply with both the letter and the spirit of applicable laws and regulations relating to insider trading.

2. Statement of Policy

2.1 It is the policy of Travere that an employee, director or designated consultant of Travere (or any other person or entity subject to this policy) who is aware of material nonpublic information relating to Travere **may not**, directly or indirectly:

2.1.1. Engage in any transactions in Travere's securities, except as otherwise specified under the heading "Exceptions to this Policy" below;

2.1.2. Recommend the purchase or sale of any Travere's securities;

2.1.3. Disclose material nonpublic information to persons within Travere whose jobs do not require them to have that information, or outside of Travere to other persons, such as family, friends, business associates and investors, unless the disclosure is made in accordance with Travere's policies regarding the protection or authorized external disclosure of information regarding Travere; or

2.1.4. Assist anyone engaged in the above activities.

2.2 The prohibition against insider trading is absolute. It applies **even if** the decision to trade is not based on such material nonpublic information. It also applies to transactions that may be necessary or justifiable for independent reasons (such as the need to raise money for an emergency expenditure) and also to very small transactions. All that matters is whether you are aware of **any** material nonpublic information relating to Travere at the time of the transaction.

2.3 The U.S. federal securities laws do not recognize any mitigating circumstances to insider trading. In addition, even the appearance of an improper transaction must be avoided to preserve Travere's reputation for adhering to the highest standards of conduct. In some circumstances, you may need to forgo a planned transaction even if you planned it before becoming aware of the material nonpublic information. So, even if you believe you may suffer an economic loss or sacrifice an anticipated profit by waiting to trade, you must wait.

2.4 It is also important to note that the laws prohibiting insider trading are not limited to trading by the insider alone; advising others to trade on the basis of material nonpublic information is illegal and squarely prohibited by this policy. Liability in such cases can extend both to the "tippee"—the person to whom the insider disclosed material nonpublic information—and to the "tipper," the insider himself or herself. In such cases, you can be held liable for your own transactions, as well as the transactions by a tippee and even the transactions of a tippee's tippee. For these and other reasons, it is the policy of Travere that no employee, director or designated consultant of Travere (or any other person or entity subject to this policy) may either (a) recommend to another person or entity that they buy, hold or sell Travere's securities **at any time** or (b) disclose material nonpublic information to persons within Travere whose jobs do not require them to have that information, or outside of Travere to other persons (unless the disclosure is made in accordance with Travere's policies regarding the protection or authorized external disclosure of information regarding Travere).

2.5 In addition, it is the policy of Travere that no person subject to this policy who, in the course of his or her relationship with Travere, learns of any confidential information that is material to another publicly traded company with which Travere does business, including a customer, supplier, partner or collaborator of Travere, may trade in that other company's securities until the information becomes public or is no longer material to that other company.

2.6 There are no exceptions to this policy, except as specifically noted above or below.

3. Transactions Subject to this Policy

3.1 This policy applies to all transactions in securities issued by Travere, as well as derivative securities that are not issued by Travere, such as exchange-traded put or call options or swaps relating to Travere's securities. Accordingly, for purposes of this policy, the terms "***trade***," "***trading***" and "***transactions***" include not only purchases and sales of Travere's common stock in the public market but also any other purchases, sales, transfers, gifts or other acquisitions and dispositions of common or preferred equity, options, warrants and other securities (including debt securities) and other arrangements or transactions that affect economic exposure to changes in the prices of these securities.

4. Persons Subject to this Policy

4.1 This policy applies to you and all other employees, directors and designated consultants of Travere and its subsidiaries. This policy also applies to members of your family who reside with you, any other persons with whom you share a household, any family members who do not live in your household but whose transactions in Travere's securities are directed by you or are subject to your influence or control and any other individuals or entities whose transactions in securities you influence, direct or control (including, e.g., a venture or other investment fund, if you influence, direct or control transactions by the fund). The foregoing persons who are deemed subject to this policy are referred to in this policy as "***Related Persons***." You are responsible for making sure that your Related Persons comply with this policy.

5. Material Nonpublic Information

5.1 Material Information

5.1.1. It is not always easy to figure out whether you are aware of material nonpublic information. But there is one important factor to determine whether nonpublic information you know about a public company is material: whether the information could be expected to affect the market price of that company's securities or to be considered important by investors who are considering trading that company's securities. If the information makes you want to trade, it would probably have the same effect on others. Keep in mind that both positive and negative information can be material.

5.1.2. There is no bright-line standard for assessing materiality; rather, materiality is based on an assessment of all of the facts and circumstances, and is often evaluated by relevant enforcement authorities with the benefit of hindsight. Depending on the specific details, the following items may be considered material nonpublic information until publicly disclosed within the meaning of this policy. There may be other types of information that would qualify as

material information as well; use this list merely as a non-exhaustive guide:

- Financial results or forecasts;
- Status of product or product candidate development or regulatory approvals;
- Clinical data relating to products or product candidates;
- Timelines for pre-clinical studies or clinical trials;
- Acquisitions or dispositions of assets, divisions or companies;
- Public or private sales of debt or equity securities;
- Stock splits, dividends or changes in dividend policy;
- The establishment of a repurchase program for Travere's securities;
- Gain or loss of a significant licensor, licensee or supplier; and
- Changes or new corporate partner relationships or collaborations.
- Notice of issuance or denial of patents;
- Regulatory developments;
- Management or control changes;
- Employee layoffs;
- A disruption in Travere's operations or breach or unauthorized access of its property or assets, including its facilities and information technology infrastructure;
- Tender offers or proxy fights;
- Accounting restatements;
- Litigation or settlements; and
- Impending bankruptcy.

5.2 When Information is Considered Public

5.2.1. The prohibition on trading when you have material nonpublic information lifts once that information becomes publicly disseminated. But for information to be considered publicly disseminated, it must be widely disseminated through a press release, a filing with the Securities and Exchange Commission (the "***SEC***"), or other widely disseminated announcement. Once information is publicly disseminated, it is still necessary to afford the investing public with sufficient time to absorb the information. Generally speaking, information will be considered publicly disseminated for purposes of this policy only after one full trading day has elapsed since the information was publicly disclosed. For example, if we announce material nonpublic information before trading begins on Wednesday, then you may execute a transaction in our securities on Thursday; if we announce material nonpublic

information after trading ends on Wednesday, then you may execute a transaction in our securities on Friday. Depending on the particular circumstances, Travere may determine that a longer waiting period should apply to the release of specific material nonpublic information.

6. Quarterly Trading Blackouts

6.1 Because our directors, officers, and such other individuals as designated on **Appendix A** attached hereto, which may be updated from time to time (collectively, our "***Covered Insiders***"), are most likely to have regular access to material nonpublic information about Travere, we require them to do more than refrain from insider trading. To minimize even the appearance of insider trading among our Covered Insiders, we have established "quarterly trading blackout periods" during which our Covered Insiders and their Related Persons—regardless of whether they are aware of material nonpublic information or not—may not conduct any trades in Travere securities. That means that, except as described in this policy, Covered Insiders and their Related Persons will be able to trade in Travere securities only during limited open trading window periods that generally will begin after one full trading day has elapsed since the public dissemination of Travere's annual or quarterly financial results and end at the beginning of the next quarterly trading blackout period. Of course, even during an open trading window period, you may not (unless an exception applies) conduct any trades in Travere securities if you are otherwise in possession of material nonpublic information.

6.2 For purposes of this policy, each "***quarterly trading blackout period***" will generally begin at the end of the day that is two weeks before the end of each fiscal quarter and end after one full trading day has elapsed since the public dissemination of Travere's financial results for that quarter. Please note that the quarterly trading blackout period may commence early or may be extended if, in the judgment of the Chief Executive Officer, Chief Financial Officer or General Counsel, there exists undisclosed information that would make trades by Covered Insiders inappropriate. It is important to note that the fact that the quarterly trading blackout period has commenced early or has been extended should be considered material nonpublic information that should not be communicated to any other person.

6.3 A Covered Insider who believes that special circumstances require him or her to trade during a quarterly trading blackout period should consult the General Counsel. Permission to trade during a quarterly trading blackout period will be granted only where the circumstances are extenuating, the General Counsel concludes that the person is not in fact aware of any material nonpublic information relating to Travere or its securities, and there appears to be no significant risk that the trade may subsequently be questioned.

7. Event-Specific Trading Blackouts

7.1 From time to time, an event may occur that is material to Travere and is known by only a few directors, officers, employees and/or designated consultants. So long as the event remains material and nonpublic, the persons designated by the Chief Executive Officer, Chief Financial Officer or General Counsel may not trade in Travere's securities. In that situation, Travere will notify the designated individuals that neither they nor their Related Persons may trade in the Travere's securities. The existence of an event-specific trading blackout should also be considered material nonpublic information and should not be communicated to any other person. Even if you have not been designated as a person who should not trade due to an event-specific trading blackout, you should not trade while aware of material nonpublic information. Exceptions will not be granted during an event-specific trading blackout.

7.2 The quarterly and event-driven trading blackouts do not apply to those transactions to which this policy does not apply, as described under the heading "Exceptions to this Policy" below.

8. Exceptions to this Policy

8.1 This policy does not apply in the case of the following transactions, except as specifically noted:

8.1.1. *Option Exercises:* This policy does not apply to the exercise of options granted under Travere's equity compensation plans for cash or, where permitted under the option, by a net exercise transaction with the Company or by delivery to Travere of already-owned Travere stock. This policy does, however, apply to any sale of stock as part of a broker-assisted cashless exercise or any other market sale, whether or not for the purpose of generating the cash needed to pay the exercise price or pay taxes.

8.1.2. *Tax Withholding Transactions:* This policy does not apply to the surrender of shares directly to Travere to satisfy tax withholding obligations as a result of the issuance of shares upon vesting or exercise of restricted stock units, performance restricted stock units, options or other equity awards granted under Travere's equity compensation plans. Of course, any market sale of the stock received upon exercise or vesting of any such equity awards remains subject to all provisions of this policy whether or not for the purpose of generating the cash needed to pay the exercise price or pay taxes.

8.1.3. *ESPP:* This policy does not apply to the purchase of stock by employees under Travere's Employee Stock Purchase Plan ("***ESPP***") on periodic designated dates in accordance with the ESPP. This policy does, however, apply to any sale of stock acquired pursuant to the ESPP.

8.1.4. *10b5-1 Automatic Trading Programs:* Under Rule 10b5-1 of the Securities Exchange Act of 1934, as amended ("***Exchange Act***"), employees and directors may establish a trading plan under which a broker is instructed to buy and sell Travere securities based on pre-determined criteria (a "***10b5-1 Trading Plan***"). So long as a 10b5-1 Trading Plan is properly established, purchases and sales of Travere securities pursuant to that 10b5-1 Trading Plan are not subject to this policy. To be properly established, an employee's or director's 10b5-1 Trading Plan must be established in compliance with the requirements of Rule 10b5-1 of the Exchange Act and any applicable 10b5-1 trading plan guidelines of Travere at a time when Travere was not in a trading blackout period and they were not otherwise aware of any material nonpublic information relating to Travere or the securities subject to the 10b5-1 Trading Plan. Moreover, all 10b5-1 Trading Plans must be reviewed and approved by Travere before being established to confirm that the 10b5-1 Trading Plan complies with all pertinent company policies and applicable securities laws.

9. Special and Prohibited Transactions

9.1 ***Inherently Speculative Transactions***: No Travere employee, director or designated consultant may engage in short sales, transactions in put options, call options or other derivative securities on an exchange or in any other organized market, or in any other inherently speculative transactions with respect to Travere's stock.

9.2 ***Hedging Transactions***: Hedging or monetization transactions can be accomplished through a number of possible mechanisms, including through the use of financial instruments such as prepaid variable forwards, equity swaps, collars and exchange funds. Such hedging transactions may permit a Travere employee, director or designated consultant to continue to own Travere's securities obtained through employee benefit plans or otherwise, but without the full risks and rewards of ownership. When that occurs, the Travere employee, director or designated consultant may no longer have the same objectives as Travere's other stockholders. Therefore, Travere employees, directors and designated consultants are prohibited from engaging in any such transactions.

9.3 ***Margin Accounts and Pledged Securities***: Securities held in a margin account as collateral for a margin loan may be sold by the broker without the customer's consent if the customer fails to meet a margin call. Similarly, securities pledged (or hypothecated) as collateral for a loan may be sold in foreclosure if the borrower defaults on the loan. Because a margin sale or foreclosure sale may occur at a time when the pledgor is aware of material nonpublic information or otherwise is not permitted to trade in Travere's securities, Travere employee, director and designated

consultants are prohibited from holding Travere's securities in a margin account or otherwise pledging Travere's securities as collateral for a loan.

9.4 ***Standing and Limit Orders***: Standing and limit orders (except standing and limit orders under approved 10b5-1 Trading Plans, as discussed above) create heightened risks for insider trading violations similar to the use of margin accounts. There is no control over the timing of purchases or sales that result from standing instructions to a broker, and as a result the broker could execute a transaction when a Travere employee, director or designated consultant is in possession of material nonpublic information. Travere therefore discourages placing standing or limit orders on Travere's securities. If a person subject to this policy determines that they must use a standing order or limit order (other than under an approved 10b5-1 Trading Plan as discussed above), the order should be limited to short duration and the person using such standing order or limit order is required to cancel such instructions immediately in the event restrictions are imposed on their ability to trade pursuant to the "Quarterly Trading Blackouts" and "Event-Specific Trading Blackouts" provisions above.

10. Pre-Clearance and Advance Notice of Transactions

10.1 In addition to the requirements above, officers, directors and other individuals who have been notified that they are subject to pre-clearance requirements face a further restriction: Even during an open trading window, they may not engage in any transaction in, or enter into, modify or terminate any contract, instruction or written plan or arrangement in, Travere's securities without first obtaining pre-clearance from Travere's General Counsel or CFO or his or her designee, which shall be requested at least two business days in advance of the desired trade. The General Counsel or CFO or his or her designee will then determine whether the individual may proceed and, if so, and if applicable, will direct the Compliance Coordinator (as identified in Travere's Section 16 Compliance Program) to help comply with any required reporting requirements under Section 16(a) of the Exchange Act. Pre-cleared transactions not completed within three business days will require new pre-clearance. It is important to remember that it is each individual's responsibility to ensure compliance with the prohibition against trading while in the possession of material nonpublic information and that the granting of pre-clearance to an individual by the Company does not relieve the individual of this responsibility.

10.2 Persons subject to pre-clearance must also give advance notice of their plans to exercise an outstanding stock option to the General Counsel.

10.3 For directors and Section 16 officers, once any transaction takes place, the officer or director must immediately notify [***] so that Travere may assist in any Section 16 reporting obligations.

11. Short-Swing Trading, Control Stock and Section 16 Reports

11.1 Officers and directors subject to the reporting obligations under Section 16 of the Exchange Act should take care to avoid short-swing transactions (within the meaning of Section 16(b) of the Exchange Act) and the restrictions on sales by control persons (Rule 144 under the Securities Act of 1933, as amended), and should file all appropriate Section 16(a) reports (Forms 3, 4 and 5), which are described in Travere's Section 16 Compliance Program, and any notices of sale required by Rule 144.

12. Policy's Duration

12.1 This policy continues to apply to your transactions in Travere's securities and the securities of other applicable public companies as more specifically set forth in this policy, even after your relationship with Travere has ended. If you are aware of material nonpublic information when your relationship with Travere ends, you may not trade Travere's securities or the securities of other applicable publicly traded companies until the material nonpublic information has been publicly disseminated or is no longer material. Further, if you leave Travere during a trading blackout period, then you may not trade Travere's securities or the securities of other applicable companies until the trading blackout period has ended.

13. Individual Responsibility

13.1 Persons subject to this policy have ethical and legal obligations to maintain the confidentiality of information about Travere and to not engage in transactions in Travere's securities or the securities of other applicable public companies while aware of material nonpublic information, as more specifically set forth in this policy. Each individual is responsible for making sure that he or she complies with this policy, and that any family member, household member or other person or entity whose transactions are subject to this policy, as discussed under the heading "Persons Subject to this Policy" above, also comply with this policy. In all cases, the responsibility for determining whether an individual is aware of material nonpublic information rests with that individual, and any action on the part of Travere or any employee or director of Travere pursuant to this policy (or otherwise) does not in any way constitute legal advice or insulate an individual from liability under applicable securities laws. You could be subject to severe legal penalties and disciplinary action by Travere for any conduct prohibited by this policy or applicable securities laws. See "Penalties" below.

14. Penalties

14.1 Anyone who engages in insider trading or otherwise violates this policy may be subject to both civil liability and criminal penalties. Violators also

risk disciplinary action by Travere, including termination of employment. Anyone who has questions about this policy should contact their own attorney or [***]. Please also see Frequently Asked Questions, which are attached as **Appendix B**.

15. Amendments

15.1 Travere is committed to continuously reviewing and updating its policies and procedures. Travere therefore reserves the right to amend, alter or terminate this policy at any time and for any reason. A current copy of the Travere's policies regarding insider trading may be obtained by contacting the General Counsel.

Appendix A:

[***]

*** Certain Confidential Information Omitted

Appendix B:

[***]

EXHIBIT 21.1

TRAVERE THERAPEUTICS, INC.
LIST OF SUBSIDIARIES

No.	Name
1	Travere Therapeutics Pharmaceutical, Inc.
2	Travere Therapeutics Ireland Limited
3	Travere Therapeutics Switzerland GmbH
4	Kyalin Biosciences, Inc.
5	Manchester Pharmaceuticals LLC

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the following Registration Statements:

1. Registration Statement (Form S-3 No. 333-281194) of Travere Therapeutics, Inc.,

2. Registration Statements (Form S-8 Nos. 333-280213, 333-273667, 333-266957, 333-258257, 333-240222, 333-232857 and 333-224848) pertaining to the Travere Therapeutics, Inc. 2017 Employee Stock Purchase Plan and Travere Therapeutics, Inc. 2018 Equity Incentive Plan, as amended,

3. Registration Statement (Form S-8 No. 333-218582) pertaining to the Travere Therapeutics, Inc. 2015 Equity Incentive Plan and Travere Therapeutics, Inc. 2017 Employee Stock Purchase Plan,

4. Registration Statements (Form S-8 Nos. 333-213599 and 333-206510) pertaining to the Travere Therapeutics, Inc. 2015 Equity Incentive Plan, and

5. Registration Statement (Form S-8 No. 333-200224) pertaining to the Travere Therapeutics, Inc. 2014 Incentive Compensation Plan;

of our reports dated February 20, 2025, with respect to the consolidated financial statements of Travere Therapeutics, Inc. and the effectiveness of internal control over financial reporting of Travere Therapeutics, Inc., included in this Annual Report (Form 10-K) of Travere Therapeutics, Inc. for the year ended December 31, 2024.

/s/ Ernst & Young LLP

San Diego, California

February 20, 2025

Exhibit 23.2

Consent of Independent Registered Public Accounting Firm

We hereby consent to the incorporation by reference in the Registration Statements on Form S-3 (No. 333-281194) and Form S-8 (Nos. 333-280213, 333-273667, 333-266957, 333-258257, 333-240222, 333-232857, 333-224848, 333-218582, 333-213599, 333-206510 and 333-200224) of our report dated February 23, 2023, except for the effects of discontinued operations discussed in Notes 1 and 19, as to which the date is February 20, 2024 and Note 20, as to which the date is February 20, 2025, relating to the consolidated financial statements of Travere Therapeutics, Inc., appearing in this Annual Report on Form 10-K.

/s/ BDO USA, P.C.

San Diego, California

February 20, 2025

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 Annual Report on Form 10-K 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: February 20, 2025

/s/ Eric Dube

Eric Dube
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF

CHIEF FINANCIAL OFFICER

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

I, Christopher Cline, certify that:

1. I have reviewed this Annual Report on Form 10-K 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: February 20, 2025

/s/ Christopher Cline

Christopher Cline
Chief Financial Officer
(Principal 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 Annual Report on Form 10-K of Travere Therapeutics, Inc. (the "Company"), for the period ended December 31, 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: February 20, 2025

/s/ Eric Dube

Eric Dube
Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION OF

CHIEF FINANCIAL OFFICER

PURSUANT TO 18 U.S.C. SECTION 1350

AS ADOPTED PURSUANT TO

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the accompanying Annual Report on Form 10-K of Travere Therapeutics, Inc. (the "Company"), for the period ended December 31, 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: February 20, 2025

/s/ Christopher Cline

Christopher Cline

Chief Financial Officer

(Principal Financial Officer)