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

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2025

OR

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

Commission File Number 001-38981

Mirum Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**83-1281555**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
989 East Hillsdale Boulevard, Suite 300, Foster City, California	**94404**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (650) 667-4085

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	MIRM	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 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 (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

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

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

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

Indicate by check mark whether the registrant 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 ☒

The aggregate market value of the registrant's common stock held by non-affiliates of the registrant as of June 30, 2025, the last business day of the registrant's most recently completed second fiscal quarter, was approximately $2.3 billion, based on the closing price of the registrant's common stock on the Nasdaq Global Market of $50.89 per share.

The number of shares of registrant's common stock, par value $0.0001 per share, outstanding as of February 20, 2026 was 60,341,617.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive proxy statement for its 2026 Annual Meeting of Stockholders, which the registrant intends to file pursuant to Regulation 14A with the Securities and Exchange Commission not later than 120 days after the registrant's fiscal year ended December 31, 2025, are incorporated by reference into Part III of this Annual Report on Form 10-K.

Table of Contents

		Page
SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS		ii
SUMMARY OF RISKS ASSOCIATED WITH OUR BUSINESS		iii
PART I		
Item 1.	Business	1
Item 1A.	Risk Factors	30
Item 1B.	Unresolved Staff Comments	86
Item 1C.	Cybersecurity	86
Item 2.	Properties	88
Item 3.	Legal Proceedings	88
Item 4.	Mine Safety Disclosures	88
PART II		
Item 5.	Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities	89
Item 6.	Reserved	90
Item 7.	Management's Discussion and Analysis of Financial Condition and Results of Operations	91
Item 7A.	Quantitative and Qualitative Disclosures About Market Risk	101
Item 8.	Financial Statements and Supplementary Data	103
Item 9.	Changes in and Disagreements with Accountants on Accounting and Financial Disclosure	137
Item 9A.	Controls and Procedures	137
Item 9B.	Other Information	138
Item 9C.	Disclosure Regarding Foreign Jurisdictions that Prevent Inspections.	138
PART III		
Item 10.	Directors, Executive Officers and Corporate Governance	139
Item 11.	Executive Compensation	139
Item 12.	Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters	139
Item 13.	Certain Relationships and Related Transactions, and Director Independence	139
Item 14.	Principal Accountant Fees and Services	139
PART IV		
Item 15.	Exhibits and Financial Statement Schedules	140
Item 16.	Form 10-K Summary	140

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K (the "Annual Report") may contain "forward-looking statements" within the meaning of the federal securities laws made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. Our actual results could differ materially from those anticipated in these forward-looking statements as a result of various factors, including those set forth under Part I, Item 1A, "Risk Factors" in this Annual Report. Except as required by law, we assume no obligation to update these forward-looking statements, whether as a result of new information, future events or otherwise. These statements, which represent our current expectations or beliefs concerning various future events, may contain words such as "may," "will," "expect," "anticipate," "intend," "plan," "believe," "estimate" or other words indicating future results, though not all forward-looking statements necessarily contain these identifying words. Such statements may include, but are not limited to, statements concerning the following:

- the commercialization of our approved medicines and our product candidates, if approved;
- our ability to obtain and maintain regulatory approval for our approved medicines and our product candidates or any of our future product candidates, and any related restrictions, limitations, and/or warnings in the label of an approved product candidate;
- our ability to develop and maintain sales and marketing capabilities, whether alone or with potential future collaborators;
- our plans to research, develop and commercialize our product candidates, including the timing of our ongoing clinical trials;
- our expectations regarding the size of target patient populations for our approved medicines and our product candidates, if approved for commercial use, and any additional product candidates we may develop;
- the size and growth potential of the markets for our approved medicines and our product candidates, and our ability to serve those markets;
- the rate and degree of market acceptance of our approved medicines and our product candidates, as well as third-party payor coverage and reimbursement for our approved medicines and our product candidates;
- our ability to attract collaborators with development, regulatory and commercialization expertise;
- our expectations regarding our ability to obtain, maintain, enforce and defend our intellectual property protection for our approved medicines and our product candidates;
- governmental, regulatory and legal developments in the United States and foreign countries;
- the performance of our third-party collaborators, suppliers and manufacturers;
- the success of competing therapies that are or may become available;
- our ability to attract and retain key scientific or management personnel;
- our ability to timely obtain funding for our operations on favorable terms and strategically deploy that funding, if and when obtained;
- our ability to timely and successfully integrate Bluejay Therapeutics, Inc. into our business and to successfully develop brelovitug; and
- the accuracy of our estimates regarding expenses, capital requirements and needs for additional financing.

Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. You should be aware that the occurrence of any of the events discussed under Part I, Item 1A, "Risk Factors" and elsewhere in this Annual Report could substantially harm our business, results of operations and financial condition and that if any of these events occurs, the trading price of our common stock could decline and you could lose all or a part of the value of your shares of our common stock.

The cautionary statements made in this Annual Report are intended to be applicable to all related forward-looking statements wherever they may appear in this Annual Report. We urge you not to place undue reliance on these forward-looking statements, which speak only as of the date of this Annual Report. Except as required by law, we assume no obligation to update our forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in any forward-looking statements, whether as a result of new information, future events or otherwise.

SUMMARY OF RISKS ASSOCIATED WITH OUR BUSINESS

An investment in shares of our common stock involves a high degree of risk. Below is a list of the more significant risks associated with our business. This summary does not address all of the risks that we face. Additional discussion of the risks listed in this summary, as well as other risks that we face, are set forth under Part I, Item 1A, "Risk Factors" in this Annual Report.

- The success of our business depends, in part, on our ability to market and sell our approved medicines profitably.
- If we are unable to adequately grow, maintain and scale our marketing and sales capabilities or enter into or maintain rights pursuant to agreements with third parties to market and sell our approved medicines, we may not be able to generate viable revenues.
- Our approved medicines or any one of our product candidates, if approved, may fail to achieve the market acceptance among physicians, patients and others in the medical community necessary for commercial success.
- We rely completely on third parties to manufacture and distribute our clinical and commercial drug supplies, including certain sole-source suppliers and manufacturers. These third parties may fail to obtain and maintain regulatory approval for their facilities, fail to provide us with sufficient quantities of drug substance, drug product, or labeled finished product in a timely fashion, or fail to do so at acceptable quality levels or prices.
- Our business depends, in part, on the success of our product candidates, each of which requires significant clinical testing before we can seek regulatory approval and potentially launch commercial sales.
- We have encountered and may continue to encounter delays and difficulties enrolling patients in our clinical trials, and as a result, our clinical development activities could be delayed or otherwise adversely affected.
- Our clinical trials may fail to adequately demonstrate the safety and efficacy of our product candidates, which could prevent or delay regulatory approval and commercialization.
- Clinical drug development involves a lengthy and expensive process with uncertain outcomes, and results of earlier studies and trials may not be predictive of future trial results.
- Any delays in the commencement or completion, or termination or suspension, of our clinical trials could result in increased costs for us, delay or limit our ability to generate revenue and adversely affect our commercial prospects.
- Our product candidates are subject to extensive regulation and compliance, which is costly and time consuming, and such regulation may cause unanticipated delays or prevent the receipt of the required approvals to commercialize our product candidates.
- We face significant competition from other biotechnology and pharmaceutical companies with products that may directly or indirectly compete with ours, and our operating results will suffer if we fail to compete effectively.
- We may fail to realize all of the anticipated benefits of our commercial and product candidate acquisitions or those benefits may take longer to realize than expected.
- We depend on intellectual property licensed from third parties and termination of any of these licenses could result in the loss of significant rights, which would harm our business.
- We may need substantial additional financing to continue our commercialization efforts for our approved medicines, develop our product candidates, license or acquire new product candidates and approved medicines, and implement our operating plans. If we fail to obtain additional financing when needed, we may be forced to delay, reduce or eliminate our product development programs or commercialization efforts.
- We do not currently have patent protection or regulatory exclusivity for certain of our approved medicines or rely on regulatory exclusivity. If we are unable to obtain and maintain sufficient intellectual property protection for our approved medicines and our product candidates, or if the scope of the intellectual property protection is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our approved medicines and our other product candidates, if approved, may be adversely affected.

[This page intentionally left blank]

PART I

Item 1. Business.

Overview

We are a biopharmaceutical company dedicated to transforming the treatment of rare diseases.

We have three approved medicines: LIVMARLI® (maralixibat) ("Livmarli"), CHOLBAM® (cholic acid) capsules ("Cholbam"), and CTEXLI® (chenodiol) tablets ("Ctexli").

Livmarli is a novel, orally administered, minimally-absorbed ileal bile acid transporter ("IBAT") inhibitor ("IBATi") that is approved for the treatment of cholestatic pruritus in patients with Alagille syndrome ("ALGS") in the United States ("U.S."), the European Union ("EU") and various other countries around the world and for cholestatic pruritus in patients with progressive familial intrahepatic cholestasis ("PFIC") in the U.S., Canada and Japan and for the treatment of PFIC in the EU. We market and commercialize Livmarli in the U.S., Canada and certain countries in Europe through our specialized and focused commercial team. We have also entered into license and distribution agreements with several rare disease companies for the commercialization of Livmarli in additional countries. We are also seeking to add to the approved indications for Livmarli by conducting the EXPAND study in settings of cholestatic pruritus due to other rare conditions.

In August 2023, we completed the acquisition of assets of Travere Therapeutics, Inc. ("Travere") that are primarily related to the development, manufacture (including synthesis, formulation, finishing or packaging) and commercialization of chenodiol and Cholbam (also known as Kolbam) (and together with chenodiol, the "Bile Acid Medicines") pursuant to an asset purchase agreement dated July 16, 2023 (such acquisition, the "Bile Acid Portfolio Acquisition").

The U.S. Food and Drug Administration ("FDA") approved Cholbam in March 2015 as the first FDA-approved treatment for pediatric and adult patients with bile acid synthesis disorders due to single enzyme defects and for adjunctive treatment of patients with peroxisomal disorders, including peroxisome biogenesis disorder-Zellweger spectrum disorder ("PBD-ZSD"). Chenodiol is standard of care for the treatment of cerebrotendinous xanthomatosis ("CTX") in the U.S. with a medical necessity recognition by the FDA and was commercialized under the brand name Chenodal. We submitted a New Drug Application ("NDA") for chenodiol for the treatment of CTX in 2024 and received FDA approval for the treatment of adults with CTX in February 2025. Chenodiol is commercialized under the brand name Ctexli. We currently market and commercialize Cholbam and Ctexli in the U.S. through our specialized and focused commercial team. We have also assumed license and distribution agreements with several rare disease companies for the commercialization of Cholbam and chenodiol in additional countries.

We are advancing our product candidate, volixibat, a novel, oral, minimally-absorbed agent designed to inhibit IBAT, for the treatment of adult patients with cholestatic liver diseases. We are developing volixibat in the setting of primary sclerosing cholangitis ("PSC") and primary biliary cholangitis ("PBC"). Volixibat has received FDA breakthrough therapy and orphan drug designation for cholestatic pruritus in PBC patients based on the positive interim analysis of the VANTAGE Phase 2b study. Volixibat has also completed a successful interim analysis of the VISTAS Phase 2b study in PSC patients. Enrollment of our VISTAS Phase 2b clinical trial in PSC was completed in the third quarter of 2025 and we expect to announce top-line results from this trial in the second quarter of 2026. Enrollment of our VANTAGE Phase 2b clinical trial in PBC is expected to be completed in the second half of 2026.

On January 23, 2026, we completed the acquisition of Bluejay Therapeutics, Inc. ("Bluejay" and such acquisition, the "Bluejay Acquisition") and its lead product candidate brelovitug (BJT-778). We are advancing brelovitug for the treatment of chronic hepatitis D virus ("HDV") infection. Brelovitug is a fully human IgG1 monoclonal antibody that binds the hepatitis B surface antigen, thereby clearing virions and subviral particles and preventing HDV infection and replication. Brelovitug has been granted FDA Breakthrough Therapy designation and European Medicines Agency ("EMA") Priority Medicines ("PRIME") scheme designation and European Commission orphan medicinal product designation. Brelovitug is currently being evaluated in the global AZURE clinical program with topline results from the AZURE-1 and AZURE-4 registration-enabling clinical trials expected in the second half of 2026.

We are also advancing our product candidate MRM-3379, a novel, oral, CNS-penetrant PDE4D inhibitor for the treatment of fragile X syndrome ("FXS"). We are currently enrolling patients in the BLOOM Phase 2 clinical study of MRM-3379 in FXS and expect topline data in 2027.

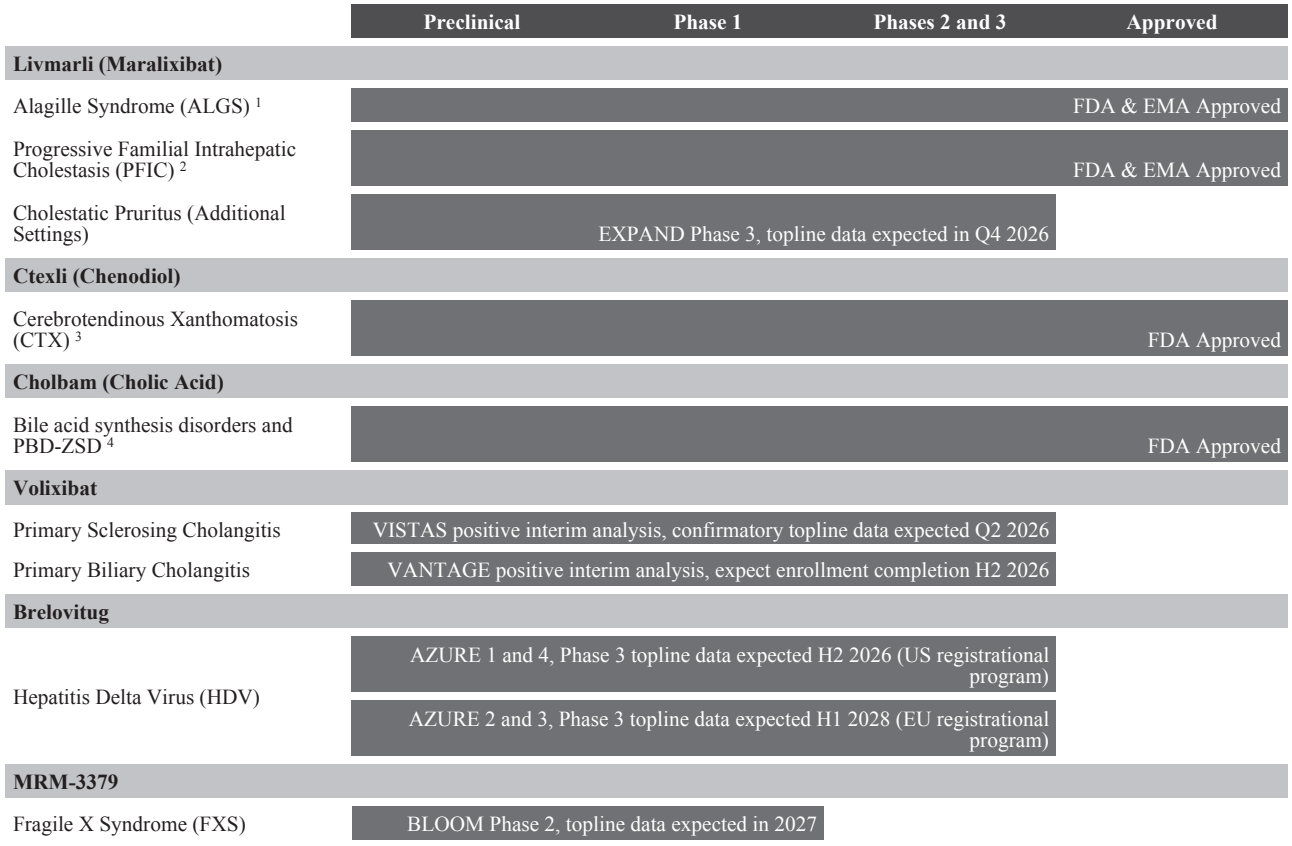
Our Strategy

Our goal is to strengthen our leadership position in rare and orphan diseases for which the unmet medical need is high. The key components of our strategy include:

- ***Commercialize medicines for rare diseases.*** Livmarli is approved by the FDA and the European Commission for the treatment of cholestatic pruritus in patients with ALGS, approved by the FDA for treatment of cholestatic pruritus in patients with PFIC and approved by EMA for the treatment of PFIC. We are commercializing Livmarli in the United States, Canada, and certain countries in Europe through direct sales. Additionally, we have entered into several distributor and licensing agreements to advance Livmarli in numerous territories outside of the markets where we commercialize Livmarli directly. Chenodiol is standard of care in the U.S. for the treatment of CTX and is being commercialized under the brand name Ctexli. Cholbam is approved for the treatment of bile acid synthesis disorders and adjunctive treatment of patients with peroxisomal disorders, including PBD-ZSD and we expect to continue to commercialize the medicine for these indications. We are commercializing Ctexli and Cholbam in the United States through one specialty pharmacy.
- ***Advance our current pipeline of clinical programs to regulatory approval.*** We are seeking to add to the approved indications for Livmarli by conducting the EXPAND study in settings of cholestatic pruritus due to other rare conditions. We plan to further leverage our understanding of cholestatic liver disease with volixibat in adult settings. We are conducting adaptive, potentially registrational, Phase 2b clinical trials of volixibat in PSC and PBC. We acquired Bluejay in January 2026 and plan to continue to develop brelovitug for the treatment of chronic HDV infection. We are conducting the global AZURE program to enable registration in the U.S., Europe and other geographies. MRM-3379 is a novel PDE4D inhibitor, which we expect to develop for FXS, a rare genetic neurocognitive disorder. We initiated a dose ranging Phase 2 trial in 2025 with data expected in 2027.
- ***Continue to acquire additional pipeline or commercial rare disease products or companies.*** We have assembled a team of scientific, clinical and business leaders with highly relevant experience to enable the advancement of therapeutics for rare and orphan diseases. We intend to leverage our collective expertise to identify, acquire, in-license and advance additional product candidates for the treatment of rare and orphan diseases.
- ***Deploy our resources efficiently to maximize value creation and long term financial performance.*** Our financial objective is value creation through long-term cash-flow generating revenue growth. We aim to accomplish this by successfully commercializing current approved medicines and successfully developing and commercializing current and future clinical-stage product candidates.

Our Product Pipeline

The following graphic depicts each of our approved medicines and product candidates, the respective indications we are pursuing, the expected next milestones and regulatory designations:



[1] Received FDA approval for cholestatic pruritus in patients with ALGS three months of age and older. European Commission has granted marketing authorization for Livmarli oral solution for the treatment of cholestatic pruritus in patients ALGS two months of age and older.

[2] Received FDA approval for cholestatic pruritus in patients with PFIC 12 months of age and older. European Commission has granted marketing authorization for Livmarli oral solution for the treatment of PFIC in patients 3 months of age and older.

[3] Received FDA approval for the treatment of adults with CTX.

[4] Bile acid synthesis disorders include PBD-ZSD.

Our Approved Medicines

Livmarli for cholestatic pruritus in patients with ALGS

Livmarli is a novel, orally administered, minimally-absorbed medicine approved by the FDA and EMA for cholestatic pruritus in patients with ALGS. Livmarli is an IBATi that prevents absorption of bile acids in the ileum, thereby lowering serum bile acid ("sBA") levels in settings of cholestasis where excess bile acids cause symptomatic and progressive disease burden.

ALGS is a rare genetic disorder of severe cholestasis in which bile ducts are abnormally narrow, malformed and reduced in number, which leads to bile accumulation in the liver and ultimately progressive liver disease. Signs and symptoms arising from cholestasis in ALGS may include jaundice, pruritus, xanthomas and growth deficit. The pruritus experienced by patients with ALGS is among the most severe in any chronic liver disease and is present in most affected children by the third year of life. In children with cholestasis due to ALGS, it is estimated that six in 10 of children progress to transplant or death by adulthood. In patients who have not received a liver transplant, 75% have active scratching, with 32% having destruction of skin, bleeding or scarring. Children with ALGS experience a markedly impaired quality of life largely due to the intense pruritus and associated skin lesions and disruptions in sleep and mood.

We believe the prevalent patient population in the United States and Europe is approximately 4,000 - 5,500 pediatric ALGS patients, which, based on our current expectations and beliefs, represents a greater than $500.0 million market opportunity. ALGS is estimated to impact one out of every 30,000 births globally.

Livmarli for cholestatic pruritus in patients with PFIC

Livmarli is a novel, orally administered, minimally-absorbed medicine approved by the FDA for cholestatic pruritus in patients with PFIC and by the EMA for treatment of patients with PFIC.

PFIC is a group of rare autosomal recessive liver diseases characterized by canalicular bile transport defects, resulting in disrupted bile formation, progressive cholestasis with elevation of sBA, and pruritus. In children, PFIC represents 10% to 15% of indications for liver transplant, either due to intractable pruritus or end-stage liver disease. The most prevalent PFIC types are bile salt export pump ("BSEP") deficiency, also known as PFIC2 (50%–60%), multidrug resistance protein 3 (MDR3) deficiency, also known as PFIC3 (30%–40%), and familial intrahepatic cholestasis-associated protein 1 (FIC1) deficiency, also known as PFIC1 (10%–20%). Other types include tight junction protein 2 (TJP2) deficiency, also known as PFIC4 and myosin VB (MYO5B) deficiency, also known as PFIC6. In addition, a minority of patients with PFIC phenotype elude genetic diagnosis using current methods, and are diagnosed clinically. Recent broadening of use of genetic testing in adult hepatology practices has resulted in an increased diagnosis of late-onset PFIC patients.

Severe pruritus is a common feature of PFIC, greatly affecting quality of life, and leading to liver transplant when symptoms are refractory. The accumulation of bile acids in PFIC is an important mediator of pruritus and driver of liver disease progression. Patients suffer from growth impairment and require fat-soluble vitamin supplementation. Interruption of the enterohepatic bile acid recirculation has the potential to alleviate pruritus and prevent liver damage. Surgical biliary diversion ("SBD") has been performed to interrupt the enterohepatic circulation of bile acids in pruritic children who are not responsive to clinical management. In a large retrospective analysis in BSEP deficiency, reduction of sBA levels by ≥75% or to <102 μmol/L after SBD was associated with long-term native liver survival.

We believe the prevalent patient population in the United States and Europe is approximately 1,000 pediatric PFIC patients.

Ctexli (Chenodiol)

Chenodeoxycholic acid, chenodiol or CDCA is a naturally occurring bile acid that was until recently approved only for the treatment of people with radiolucent stones in the gallbladder. CDCA received FDA approval to treat CTX in February 2025, which is commercialized under the brand name Ctexli. CTX is a rare, progressive and under diagnosed bile acid synthesis disorder affecting many parts of the body. We estimate there are 1,000 - 2,000 prevalent CTX patients in the United States, however only 10% are currently diagnosed.

Cholbam for the treatment of bile acid synthesis disorders and peroxisomal disorders, including PBD-ZSD

The FDA approved Cholbam (cholic acid capsules) in March 2015, as the first FDA-approved treatment for pediatric and adult patients with bile acid synthesis disorders due to single enzyme defects and for adjunctive treatment of patients with peroxisomal disorders, including PBD-ZSD. The effectiveness of Cholbam has been demonstrated in clinical trials for bile acid synthesis disorders and the adjunctive treatment of peroxisomal disorders. An estimated 200 to 300 prevalent patients in the United States are current candidates for Cholbam therapy.

Our Clinical Product Candidates

Livmarli

We are advancing our approved medicine Livmarli in additional indications. Through the EXPAND phase 3 study we are exploring the treatment of pruritus in rare cholestatic settings other than ALGS, PFIC, PSC, intrahepatic cholestasis of pregnancy and PBC. We believe that the prevalent pediatric patient population in the United States and Europe in these additional rare cholestatic settings is approximately 1,000 patients. We expect topline results from the EXPAND study in the fourth quarter of 2026.

Volixibat

We are advancing our product candidate, volixibat, a novel, oral, minimally-absorbed agent designed to inhibit IBAT, for the treatment of adult patients with cholestatic liver diseases. We are developing volixibat for the treatment of PSC and PBC. Volixibat has been studied in over 400 adults for up to 48 weeks. Clinical trials of volixibat have shown

significant activity on IBAT and bile acid markers such as 7αC4, fecal bile acids and cholesterol, demonstrating potent biological activity.

PSC is a serious, idiopathic chronic cholestatic liver disease characterized by the progressive inflammation and destruction of bile ducts, which can lead to life-threatening complications. It is estimated that approximately 54,000 people in the United States and Europe suffer from PSC with approximately 30,000 people in the United States alone. Up to approximately 65% of PSC patients suffer from pruritus during the course of the disease. Liver transplantation is the only treatment shown to improve clinical outcomes in PSC but is expensive, requires long-term administration of immunosuppressants and only a portion of the patients who require a liver transplant are able to match with a suitable donor organ. Ursodeoxycholic acid ("UDCA"), is used off-label in PSC with conflicting evidence. We are conducting the VISTAS Phase 2b clinical trial of volixibat in patients with pruritus and PSC. VISTAS is an adaptive, randomized Phase 2b clinical trial evaluating the effect of volixibat on pruritus, sBA and fibrosis markers in patients with PSC and pruritus. We expect to announce topline results from the VISTAS study in the second quarter of 2026.

PBC is a chronic, rare, cholestatic liver disease characterized by progressive liver bile flow impairment caused by immune-mediated destruction of intrahepatic bile ducts. This results in increased hepatic bile acid concentrations, which leads to a local inflammatory response in the liver that progresses to hepatic fibrosis, cirrhosis, and hepatic decompensation. The incidence rates for PBC in Europe, North America, Asia, and Australia are reported as ranging from 0.33 to 5.8 per 100,000 people, with a prevalence ranging from 1.91 to 40.2 per 100,000 people, resulting in approximately 230,000 patients across the United States and Europe suffering from PBC with approximately 85,000 people in the United States alone. Up to approximately 60% of PSC patients suffer from pruritus during the course of the disease. We are conducting the VANTAGE Phase 2b clinical trial of volixibat in patients with pruritus and PBC and we expect to complete enrollment in 2026.

There are no approved therapies for PSC in the United States. A variety of licensed and off-label therapies are currently used to reduce the impact of the progressive nature of PBC. These include UDCA, obeticholic acid, seladelpar, elafibranor, and others. However, the few therapeutic options available to manage PBC associated pruritus are temporary and/or suboptimal. We estimate a worldwide total addressable market for volixibat in PSC and PBC to be more than $1 billion.

Brelovitug

We are advancing brelovitug for the treatment of chronic HDV infection. Brelovitug is a novel fully human IgG1 monoclonal antibody that binds the hepatitis B surface antigen, thereby clearing virions and subviral particles and preventing HDV infection and replication. The results of the brelovitug Phase 2 study show the potential for this treatment to substantially decrease viral load and improve liver function. The Phase 2 study in 47 adults with chronic HDV infection demonstrated 100% virologic response, defined as a 2 log or greater reduction or target not detected ("TND") for HDV RNA. This was observed across all dose groups at 48 weeks, with nearly all patients achieving this response by 24 weeks. Additionally, 65-82% of subjects achieved the composite endpoint of virologic response and Alanine Aminotransferase ("ALT") normalization. The safety profile was favorable with no grade 3 or higher adverse events, no serious adverse events, a low rate of flu-like symptoms and no ALT elevation or neutropenia. Based on this data, brelovitug was granted FDA breakthrough designation and EMA PRIME designation.

Brelovitug is currently being evaluated in the global AZURE Phase 3 program which is designed to meet expected registrational requirements in both the U.S. and EU with a primary combined endpoint of virologic response and ALT normalization. Registration in the U.S. will be supported by the AZURE-1 and AZURE-4 studies with the primary composite endpoint of virologic response and ALT normalization. Virologic response is defined as 2 log or greater decline or undetectable HDV RNA. Studies AZURE-1 and -4 are evaluating two doses of brelovitug, a once weekly 300 mg subcutaneous dose and a once monthly 900 mg subcutaneous dose with a primary analysis after 24 weeks. Each study also includes an extension period to support long term safety and efficacy. We expect to complete the 24-week trial period of both AZURE-1 and -4 and announce topline results in the second half of 2026. AZURE-2 and AZURE-3 will compare brelovitug to an active control, bulevirtide, which is approved in the EU. AZURE-2 and -3 are 48-week and 24-week studies, respectively. These studies are intended to support EMA registration as well as long-term efficacy and safety, with final data expected in the first half of 2028.

MRM-3379

We are advancing MRM-3379 for the treatment of FXS. FXS is an X-linked rare genetic condition that represents the most common inherited single-gene cause of intellectual disability and autism spectrum disorder. While both males and females may be affected, males typically experience more severe symptoms due to the presence of only one X chromosome, with the majority of males meeting the criteria for severe intellectual disability. Patients can also present with severe behavioral alterations, including hyperactivity, impulsivity and anxiety in addition to poor language development

and seizures. It is estimated that there are approximately 50,000 males in the U.S. and Europe with FXS with an estimated worldwide total addressable market for more than $1 billion. There are currently no approved therapies for the treatment of FXS and there is a significant unmet need.

MRM-3379 is a potent and selective allosteric inhibitor of PDE4D, an enzyme predominantly expressed in brain regions associated with learning, memory, and emotional regulation. Inhibition of PDE4D increases signaling by cAMP, which is impaired in FXS patients, and may support the improved function of the biochemical cascades believed to regulate memory, learning, and cognitive processes. MRM-3379 has been granted FDA Fast Track designation for the treatment of FXS. We are currently enrolling patients in the BLOOM Phase 2 clinical study of MRM-3379 in FXS and expect topline data in 2027.

License, Finance, Royalty Agreements and Asset Purchases

Assignment and License Agreement with Shire International GmbH (Takeda)

In November 2018, we entered into an assignment and license agreement ("Shire License Agreement") with Shire International GmbH ("Shire"), which was subsequently acquired by Takeda Pharmaceutical Company Limited. Pursuant to the Shire License Agreement, Shire assigned, transferred and conveyed all of its right, title and interest in and to the license agreement, as amended ("Satiogen Agreement"), with Satiogen Pharmaceuticals, Inc. ("Satiogen"), now a wholly owned Mirum entity, under which we obtained an exclusive, worldwide license to certain patents and know-how controlled by Satiogen related to ASBTis ("ASBTi Technology") and TGR5 agonists ("TGR5 Technology"), the Pfizer Agreement and the Sanofi Agreement, both as defined below (collectively with the Satiogen Agreement, the "Assigned License Agreements").

In addition, Shire granted us an exclusive, royalty bearing, sublicensable, worldwide license under certain regulatory materials as well as patents and know-how, which we refer to collectively as the Shire IP, relating to the Livmarli compound and the volixibat compound in development by Shire as of that date, which we collectively refer to as the Shire Licensed Products, to develop, have developed, make, have made, use, sell, have sold, offer for sale or import the Shire Licensed Products worldwide for the therapeutic or prophylactic application in human health. We have sole authority and responsibility over development and commercialization activities for the Shire Licensed Products, and we are required to use commercially reasonable efforts to perform certain development, regulatory and commercialization activities with respect to the PFIC and ALGS indications for Livmarli and unspecified indications with respect to volixibat. We will solely own all inventions and discoveries arising out of activities conducted by us under the Shire License Agreement. We will also be responsible for the preparation, filing, prosecution and maintenance of patents under the Shire License Agreement and the cost thereof. We have the first right, but are not obligated, to enforce any patent licensed under the Shire License Agreement.

We are required to pay Shire up to an aggregate of $109.5 million upon the achievement of certain other clinical development and regulatory milestones for Livmarli in the PFIC and ALGS indications, and a $25.0 million payment upon regulatory approval of Livmarli for each and every other indication. Each such milestone payment will be paid only once for each such indication during the term of the Shire License Agreement, the first time Livmarli reaches such milestone event, regardless of the number of times such milestone is reached by Livmarli for the same indication. In addition, we are required to pay up to an aggregate of $30.0 million upon the achievement of certain clinical development and regulatory milestones for volixibat solely for the first indication sought. Each such milestone payment will be paid only once for the first indication for which volixibat is developed during the term of the Shire License Agreement, the first time volixibat reaches such milestone event, regardless of the number of products or the number of indications for which volixibat is developed.

Under the Shire License Agreement and Assigned License Agreements, to date, we have met clinical development, regulatory and sales milestones resulting in the payment of an aggregate of $101.5 million related to our Livmarli and volixibat programs.

Upon achievement of certain thresholds for aggregate worldwide net sales for all Shire Licensed Products, we are required to pay Shire, on a one-time, non-refundable and non-creditable basis, up to an aggregate of $30.0 million in tiered sales milestone payments. In the fourth quarter of 2023, we paid Shire $5.0 million based on the achievement of a sales milestone. Lastly, upon certain annual worldwide net sales of all Shire Licensed Products, we are required to pay Shire, on a non-refundable and non-creditable basis, tiered royalties with rates ranging from low double-digits to mid-teens ("Shire royalties"). As we make royalty payments to Satiogen under the Satiogen Agreement, the Shire royalties will be reduced by a low single digit percentage of net sales. Similarly, if we actually make royalty payments to Sanofi, which is defined below, under the Sanofi Agreement, the Shire royalties will be reduced by low to high single digit percentages of certain net sales thresholds.

Under the Shire License Agreement, we are prohibited from developing any competing product prior to the five-year anniversary of the first commercial sale of a Shire Licensed Product, or commercializing any competing product prior to the eight-year anniversary of the first commercial sale of a Shire Licensed Product. For purposes of the Shire License Agreement, a competing product is any product that is or contains a compound (A) where the primary method of action is ASBT inhibition activity, which is another term for IBAT inhibition, or (B) that is commercialized or developed for any PFIC, ALGS, or BA indication, except (B) shall not apply with respect to (1) a given indication if a product failure has occurred with respect to such indication (e.g., if a product failure has occurred for a Shire Licensed Product for the BA indication, we may thereafter develop and commercialize a product for the BA indication if such product uses a different primary method of action than ASBT inhibition activity) or (2) a given product if such product is a product that is not deleterious to the sales or pricing of a Shire Licensed Product.

The Shire License Agreement will remain in effect on a country-by-country and Shire Licensed Product-by-Shire Licensed Product basis and will continue on such basis until the later of the (i) expiration of the last patent or patent application licensed under the Shire License Agreement that covers a Shire Licensed Product, (ii) expiration of any regulatory exclusivity period, and (iii) tenth anniversary of the first commercial sale of such Shire Licensed Product in such country. The term of the last patent or patent application licensed under the Shire License Agreement ends on October 26, 2032, absent patent term adjustment or extension. We may unilaterally terminate the Shire License Agreement for any reason or no reason upon 90 days' written notice to Shire. In addition, we may also terminate the Shire License Agreement if we reasonably determine that we are precluded from further development due to materially adverse pre-clinical or clinical pathology or toxicology data. Either party may terminate the Shire License Agreement in the event of the other party's insolvency or for the other party's material breach of the Shire License Agreement that remains uncured after 90 days of receiving written notice of such breach. Shire may terminate the Shire License Agreement upon our or our affiliates' challenge to the validity of the patents licensed under the Shire License Agreement.

License Agreement with Pfizer Inc.

Through the Shire License Agreement, we were assigned the rights to the license agreement ("Pfizer Agreement"), with Pfizer Inc. ("Pfizer"), pursuant to which we obtained an exclusive, worldwide license to Pfizer's know-how related to Livmarli, (the "Pfizer Know-How"). Under the Pfizer Agreement, we are permitted to research, develop, manufacture and commercialize products utilizing the Pfizer Know-How for the diagnosis, treatment, prevention, mitigation and cure of human diseases and disorders, and to sublicense such rights. Pfizer retained the right to use the Pfizer Know-How to conduct internal research and to use a third party to conduct research on Pfizer's behalf.

We have sole responsibility and control over development and commercialization activities for the Pfizer Know-How and products utilizing the Pfizer Know-How, and we are obligated to use commercially reasonable efforts to develop and commercialize products utilizing the Pfizer Know-How. In the event we determine to sublicense to a third party our right to commercialize the Pfizer Know-How or products utilizing the Pfizer Know-How under the Pfizer Agreement, Pfizer has the first right to negotiate such a commercial license with us.

Ownership of inventions and discoveries under the Pfizer Agreement will be determined in accordance with the rules of inventorship under United States patent laws. We will own and bear all expenses incurred in preparing, filing, prosecuting and maintaining all patents for inventions that are solely invented by us.

As consideration, upon commercialization of any product utilizing the Pfizer Know-How, we are required to pay to Pfizer a low single-digit royalty on net sales of such products sold by us, our affiliates or sublicensees. Our royalty obligations continue on a licensed product-by-licensed product basis until the eighth anniversary of the first commercial sale of such licensed product anywhere in the world. We currently pay royalties to Pfizer on our sales of Livmarli.

We may unilaterally terminate the Pfizer Agreement for any reason or no reason upon 90 days' written notice to Pfizer. Either party may terminate the Pfizer Agreement in the event of the other party's insolvency or for the other party's material breach of the Pfizer Agreement which remains uncured after 60 days of receiving written notice of such breach, or 30 days in the case of a payment breach. Absent early termination, the Pfizer Agreement will automatically expire on a country-by-country basis upon the expiration of our royalty payment obligations.

License Agreement with Sanofi-Aventis Deutschland GmbH

Through the Shire License Agreement, we were assigned the rights to the license agreement, as amended ("Sanofi Agreement"), with Sanofi-Aventis Deutschland GmbH ("Sanofi"), under which we obtained an exclusive, worldwide license to certain patents and know-how controlled by Sanofi related to volixibat ("Sanofi Technology"). Under the Sanofi Agreement, we are permitted to develop and commercialize products containing volixibat utilizing the Sanofi Technology. Additionally, under the Sanofi Agreement, we are permitted to manufacture products containing volixibat utilizing the Sanofi Technology and to sublicense such rights. In addition, Sanofi granted to us, under certain conditions, an

exclusive option to obtain an exclusive license to manufacture volixibat during the term of the Sanofi Agreement. We exercised this option in May 2020 and are transferring manufacturing of volixibat to a third-party contract manufacturer. Sanofi retained the right to practice the Sanofi Technology outside the scope of the license granted to us under the Sanofi Agreement and to make and use for internal research purposes, provided that upon our request, Sanofi is obligated to provide us with a written summary of the results of any such research to the extent such results relate to the use of volixibat as an ASBT inhibitor ("ASBTi").

Under the Sanofi Agreement, we have sole authority and responsibility over development and commercialization activities for licensed products, and we are required to use diligent efforts to perform certain development, regulatory and commercialization activities.

With the exception of Sanofi's rights on its further optimization of the process of manufacturing of the product utilizing the Sanofi Technology, we will own all inventions and discoveries arising out of activities conducted by us under the Sanofi Agreement and we will be responsible for the preparation, filing, prosecution and maintenance of patents under the Sanofi Agreement. Further, we will have the first right, but will not be obligated, to enforce patents under the Sanofi Agreement. If we do not exercise our right to enforce patents under the Sanofi Agreement, Sanofi will be able to enforce the patents.

We are required to pay to Sanofi up to an aggregate of $36.0 million upon the achievement of certain regulatory, commercialization and product sales milestones. Upon commercialization of any product utilizing the Sanofi Technology, we will be required to pay to Sanofi tiered royalties in the mid to high single-digit range based upon net sales of licensed products sold by us and our affiliates and sublicensees in a calendar year, subject to adjustments in certain circumstances. Our royalty obligations continue on a licensed product-by-licensed product and country-by-country basis until the later to occur of the expiration of the last valid claim in a licensed patent or patent application covering the applicable licensed product in such country and ten years after the first commercial sale of a licensed product following regulatory approval in such country. The term of the last patent or patent application licensed under the Sanofi Agreement ends on May 26, 2030, absent patent term adjustment or extension. In the event we sublicense our right to commercialize a product utilizing the Sanofi Technology, we are obligated to pay to Sanofi a fee based on a percentage of sublicense fees received by us, which percentage ranges from the mid-teens to low-thirties, depending on the stage of development of such licensed product, and is subject to adjustment in certain circumstances.

For three years after the first commercial sale of a product utilizing the Sanofi Technology, on a licensed product-by-licensed product basis, we may not, through our own efforts or with an affiliate or third party, commercialize any product for specified indications with a method of action that reduces the reabsorption of bile acids in the intestinal tract, except for the commercialization of products utilizing the Sanofi Technology under the Sanofi Agreement.

We may unilaterally terminate the Sanofi Agreement for any reason or no reason upon 60 days' written notice to Sanofi. We may also terminate the Sanofi Agreement on a country-by-country or licensed product-by-licensed product basis upon written notice to Sanofi (1) if we reasonably determine that we are precluded from proceeding with the first Phase 2b clinical trial for a product utilizing the Sanofi Technology in certain major markets due to certain safety failures or (2) after using diligent efforts, we reasonably determine that we are precluded from proceeding with a Phase 3 clinical trial for a product utilizing the Sanofi Technology in certain major markets due to certain safety or efficacy failures. Either party may terminate the Sanofi Agreement in the event of the other party's insolvency or for the other party's material breach of the Sanofi Agreement which remains uncured after 90 days of receiving written notice of such breach, or ten business days in the case of a payment breach. Absent early termination, the Sanofi Agreement will remain in effect on a country-by-country and licensed product-by-licensed product basis until the expiration of our royalty payment obligations for such licensed product in such country.

License agreement with Novartis Pharma AG

Bluejay obtained a worldwide license to develop and commercialize brelovitug from Novartis in June 2021. Under the terms of the license agreement, Novartis is entitled to receive up to $8.0 million in development milestones and up to $27.0 million in commercial milestones. Novartis is also entitled to receive commercial royalties of mid-single digit percent of sales. In addition, Bluejay agreed to pay Novartis a milestone payment of $4.0 million upon a change of control, which was earned upon closing of the acquisition of Bluejay in January 2026.

Manufacturing and License Agreement with Lonza Ltd.

In conjunction with the manufacture and commercialization of brelovitug, Bluejay entered into a master services agreement with Lonza Ltd. ("Lonza") in October 2021 and a license agreement with Lonza Sales AG in December 2022 (the "Lonza License Agreement"). Lonza is manufacturing brelovitug using its proprietary cell line technology and related

intellectual property. Under the Lonza License Agreement, Bluejay obtained a non-exclusive, sublicensable license under Lonza's cell line technology and related know-how and patents for the manufacture and commercialization of brelovitug.

License Agreement with Enthorin Therapeutics, LLC and Dart Neuroscience LLC

On October 22, 2024, we entered into a license agreement (the "MRM-3379 License") with Enthorin Therapeutics, LLC and Dart Neuroscience LLC (collectively "Enthorin"). The MRM-3379 License grants us an exclusive, royalty bearing, sublicensable, worldwide license under certain regulatory materials as well as patents and know-how, which we refer to collectively as the Enthorin IP, to develop, have developed, make, have made, use, sell, have sold, offer for sale or import ENT-3379, an allosteric inhibitor of Phosphodiesterase 4D, (PDE4D) for any medical diagnostic, therapeutic or prophylactic application in human health, including in FXS. In exchange for the license, we paid an upfront payment of $7.5 million and are obligated to pay up to an additional $217.5 million upon the achievement of regulatory and sales-based milestones as well as mid-single digit percent royalties on any future sales of MRM-3379.

Commercial Agreements related to Cholbam

Asset Purchase Agreement with Asklepion Pharmaceuticals, LLC

Through the Bile Acid Portfolio Acquisition, we were assigned the rights to the Asset Purchase Agreement ("Asklepion APA"), dated January 10, 2015, between Travere and Asklepion Pharmaceuticals, LLC ("Asklepion"), pursuant to which we acquired all right, title and interest to certain assets of Asklepion aimed at the development of products related to cholic acid ("Cholic Acid Products"). Pursuant to the Asklepion APA, we are required to pay Asklepion high single-digit to low double-digit tiered royalties on worldwide net revenues of Cholic Acid Products.

Commercial Agreements related to chenodiol

License and Manufacturing Agreement with LGM Pharma

Through the Bile Acid Portfolio Acquisition, we were assigned the rights to the License and Manufacturing Agreement, dated November 4, 2009, between LGM Pharma, formerly Nexgen Pharma, Inc. ("LGM"), and Manchester Pharmaceuticals, Inc. related to the manufacture of chenodiol, pursuant to which we obtained an exclusive, perpetual license to market chenodiol in the U.S. under the terms of an abbreviated new drug application approved by the FDA and owned by LGM, which grants to LGM the authority to manufacture and sell chenodiol in the U.S.

Asklepion APA

Pursuant to the Asklepion APA, we are required to pay Asklepion a low single-digit royalty on net sales of chenodiol in the U.S.

Asset Purchase Agreement with Travere Therapeutics, Inc.

On August 31, 2023, we completed the Bile Acid Portfolio Acquisition. We paid $210.4 million upon closing of the transaction, and up to an additional $235.0 million is payable upon the achievement of certain milestones based on specified amounts of annual net sales of the Bile Acid Medicines. As of December 31, 2025, the Company accrued $25.0 million for the achievement of a commercial milestone associated with achievement of certain net product sales, which is expected to be paid in the first quarter of 2026.

In connection with and immediately prior to the closing of the Bile Acid Portfolio Acquisition, we completed the private placement of 8,000,000 shares of our common stock at a price per share of $26.25, resulting in net proceeds of approximately $202.2 million.

Intellectual Property

Our success depends in part on our ability to obtain and maintain proprietary protection for our product candidates and other discoveries, inventions, trade secrets and know-how that are critical to our business operations. Our success also depends in part on our ability to operate without infringing the proprietary rights of others, and in part, on our ability to prevent others from infringing our proprietary rights. A comprehensive discussion on risks relating to intellectual property is provided under "Risk Factors" under the subsection "Risks Related to Our Intellectual Property."

We have developed and continue to develop patent portfolios around our product candidates, Livmarli (maralixibat) and volixibat. We have rights to pending patent applications in the United States, Europe, South Korea, Hong Kong, and Singapore covering the methods of treating various cholestatic liver indications using maralixibat and/or volixibat which, if issued, would expire in October 2032, absent any patent term adjustments or extensions. We have rights to issued U.S. Patent No. 11,376,251, which is directed to methods of treating ALGS in a pediatric subject with maralixibat, expiring in October 2032. We also have rights to U.S. Patent No. 12,350,267, which is directed to methods of

treating PFIC in a pediatric subject with maralixibat, expiring in October 2032. We also have rights to U.S Patent Nos. 10,512,657 and 11,229,661, which are directed to methods of treating or ameliorating PFIC2 and methods of treating or ameliorating a pediatric disorder characterized by having a non-truncating BSEP mutation selected from PFIC2, BRIC2, and drug induced cholestasis in a pediatric subject comprising administering maralixibat, respectively, both of which expire in October 2032. These four U.S. patents are listed in the FDA's Orange Book: Approved Drug Products with Therapeutic Equivalence Evaluations ("Orange Book") for Livmarli. We have rights to a U.S. patent that is directed to methods of treating PBC with volixibat, expiring in October 2032.

We have rights to granted patents in Australia, Brazil, Canada, China, Israel, Japan, Mexico, South Korea, Armenia, Azerbaijan, Belarus, Kazakhstan, Kyrgyzstan, Russia, Tajikistan, Turkmenistan, South Africa and Macau covering the methods of treating cholestasis using IBATis that have limited systemic exposure, which expire in October 2032. We also have rights to pending patent applications in United States, Europe, Hong Kong and Singapore, covering methods of treating pediatric cholestatic liver diseases using IBATis that have limited systemic exposure, which, if issued, would expire in October 2032, absent any patent term adjustments or extensions. We have rights to granted patents in Australia, Brazil, Canada, China, Israel, Japan, Mexico, South Korea, South Africa, Singapore, Austria, Belgium, Czech Republic, Denmark, Estonia, Finland, France, Germany, Ireland, Italy, Netherlands, Norway, Poland, Portugal, Slovak Republic, Spain, Sweden, Switzerland, United Kingdom, Armenia, Azerbaijan, Belarus, Kazakhstan, Kyrgyzstan, Russia, Tajikistan, Macau and Turkmenistan covering methods of treating pediatric cholestatic liver diseases using IBATis that have limited systemic exposure, which expire in October 2032. We also have rights to a granted patent in South Africa covering pediatric dosage forms of IBATis that have limited systemic exposure, which expires in October 2032.

We have rights to U.S. Patent Nos. 11,229,647 and 11,497,745, which are directed to methods of treating ALGS in a pediatric subject comprising administering maralixibat to the subject expiring in February 2040. We also have rights to U.S. Patent No. 11,918,578, which is directed to a method of treating cholestatic pruritus in ALGS subjects expiring in February 2040. These three U.S. patents are listed in the Orange Book for Livmarli. We have rights to granted patents in Austria, Belgium, Bulgaria, Croatia, Denmark, Finland, France, Germany, United Kingdom, Ireland, Italy, the Netherlands, Norway, Poland, Portugal, Romania, Sweden, Switzerland, Turkey, United Kingdom, Spain, Mexico, Japan and Russia, which are directed to maralixibat or volixibat for use in treating cholestatic liver disease in a subject having BSEP deficiency but without total loss of BSEP activity, which expire in February 2040. We have rights to pending patent applications in the United States, Europe, Canada, China, Japan, South Korea, Israel, Brazil, Russia, Mexico, Australia, New Zealand, UAE, and Saudi Arabia directed to methods for treating cholestatic liver disease and to methods for using patient genotype to predict response to IBATi administration in patients with BSEP deficiency. Any patents issuing from these applications would expire in February 2040, absent any patent term adjustments or extensions.

We have rights to a granted patent in Russia directed to methods for treating ALGS comprising administering higher dosages of maralixibat. We have rights to pending applications in the United States, Europe, Canada, China, Japan, South Korea, Israel, Brazil, Russia, Mexico, Australia, New Zealand, UAE, and Saudi Arabia directed to methods for treating cholestatic liver disease comprising administering higher dosages of IBATis. We have rights to granted patents in Japan, Russia, Hong Kong, Croatia, Czech Republic, Ireland, Norway, Poland, Romania, Slovak Republic, Spain, Switzerland, and United Kingdom directed to methods of increasing growth in pediatric subjects having cholestatic liver disease by administering IBATis, which expire in February 2040. We have rights to pending applications in the United States, Europe, Canada, South Korea, Israel, Brazil, Russia, Mexico, Australia, New Zealand, UAE, and Saudi Arabia directed to methods of increasing growth in pediatric subjects having cholestatic liver disease by administering IBATis. Any patents issuing from these applications would expire in February 2040, absent any patent term adjustments or extensions.

We have rights to pending applications in the United States, Argentina, Taiwan, Europe, Canada, China, Japan, South Korea, Australia, India, Israel, Singapore, Brazil, Mexico, Eurasia, New Zealand, United Arab Emirates, Saudi Arabia, Morocco, Tunisia, Egypt, Chile, Colombia, Malaysia, Philippines, Thailand, Vietnam, South Africa and Hong Kong directed to formulations of maralixibat which, if issued, would expire in October 2042, absent any patent term adjustments or extensions. We have rights to a granted patent in Luxemburg directed to increased event-free survival of long-term maralixibat patients which expires in October 2042. We have rights to pending applications in the United States, Europe, Canada, China, Japan, Australia, Israel, Singapore, Mexico, Eurasia, South Africa, and Hong Kong directed to methods of treatment with IBATis in the fasted state which, if issued, would expire in October 2042, absent any patent term adjustments or extensions. We have rights to pending applications in the United States, Europe, Canada, China, Japan, Australia, Israel, Singapore, Mexico, Eurasia, South Africa, New Zealand, Malaysia, Saudi Arabia, United Arab Emirates, Morocco, Tunisia, Lybia, Iraq, and Hong Kong directed to increased event-free survival of long-term maralixibat patients which, if issued, would expire in November 2042, absent any patent term adjustments or extensions.

We have rights to pending applications in the United States, Argentina, Taiwan, Uruguay, Paraguay, Europe, Canada, China, Hong Kong, Japan, South Korea, Australia, India, Israel, Singapore, Brazil, Mexico, Eurasia, South Africa,

New Zealand, Chile, and United Arab Emirates directed to highly pure maralixibat forms and intermediates which, if issued, would expire in September 2043, absent any patent term adjustments or extensions. We have rights to U.S. Patent No. 12,296,050 directed to maralixibat compositions and solid dosage forms expiring in October 2043. This patent is listed in the Orange Book for Livmarli. We have rights to pending applications in the United States, Patent Cooperation Treaty ("PCT"), and Taiwan directed to maralixibat compositions and solid dosage forms which, if issued, would expire in October 2043, absent any patent term adjustments or extensions. We have rights to pending applications in the United States, Taiwan, Australia, Brazil, Canada, China, Eurasia, Europe, Hong Kong, Israel, Japan, South Korea, Mexico, New Zealand, Singapore and South Africa directed to methods of treating PFIC with maralixibat which, if issued, would expire in October 2043, absent any patent term adjustments or extensions. We have rights to pending applications in the United States, Brazil, Canada, China, Eurasia, Europe, Israel, Japan, South Korea, Mexico, New Zealand and Singapore directed to pharmaceutical compositions comprising volixibat which, if issued, would expire in May 2044, absent any patent term adjustments or extensions. We have rights to pending applications in the United States and PCT directed to methods of treating cholestatic pruritus in rare diseases which, if issued, would expire in May 2045, absent any patent term adjustments or extensions.

We have licensed patent applications in the United States, Hong Kong, and Europe from Satiogen covering therapeutic uses of IBATis that have limited systemic exposure for treating inflammatory intestinal conditions, which, if issued, would expire in May 2031, absent any patent term adjustments or extensions. Two of these Satiogen applications have issued as United States Patent No. 10,251,880 and 11,260,053, the latter being Orange-Book listed for Livmarli. We have licensed an issued United States patent, as well as issued foreign counterparts in Argentina, Austria, Australia, Belgium, Canada, Switzerland, China, Germany, Denmark, Spain, Finland, France, United Kingdom, Greece, Hong Kong, Ireland, Israel, India, Italy, Japan, South Korea, Liechtenstein, Mexico, Malaysia, the Netherlands, Norway, Portugal, Russia, Sweden, Singapore, Taiwan, Turkey, and Brazil from Sanofi, that cover the composition and methods of making volixibat and salts thereof, expiring in December 2027. Patents related to Livmarli and volixibat may be eligible for patent term extensions in certain jurisdictions, including the United States for volixibat, upon approval of a commercial use of the corresponding product by a regulatory agency in the jurisdiction where the patent was granted. Similar to the patent term-extensions in the United States, Supplementary Protection Certificates ("SPCs") serve as an extension to a patent right in the EU for up to five years. SPCs have been granted for maralixibat for EP2771003 in Austria, Denmark, France, Italy, the Netherlands, Portugal, Spain, and Sweden and are pending in Belgium, Czech Republic, Germany, Estonia, Finland, United Kingdom, Ireland, Norway, Poland, and Slovakia.

We do not have patents or patent applications covering Livmarli as a composition of matter. Therefore, the primary patent-based intellectual property protection for our Livmarli program will be any patents granted on the pending method-of-use and dosage form patent applications.

We have rights to a patent portfolio and will continue to develop a patent portfolio around our product candidate MRM-3379. We have rights to three issued U.S. patents covering MRM-3379 as a composition-of-matter, as well as a pending counterpart in the United States and issued foreign counterparts in Belgium, France, Germany, Norway, the Netherlands, Sweden, Switzerland, United Kingdom, Denmark, Italy, Ireland, Spain, United Kingdom, Australia, Canada, China, Japan, South Korea, Brazil, India, Israel, Mexico, New Zealand, Russia, Singapore, Hong Kong, and Taiwan, which are set to expire in March 2034, absent any patent term extensions. We have rights to pending PCT and Taiwan applications covering methods of treating Fragile X syndrome using MRM-3379. Patents related to MRM-3379 may be eligible for patent term extensions in certain jurisdictions, including the United States, upon approval of a commercial use of the corresponding product by a regulatory agency in the jurisdiction where the patent was granted.

Upon approval in the United States, as MRM-3379 has not previously been approved in the United States for any indication, it may be eligible for five years of NCE exclusivity, which would run concurrently with its seven years of orphan drug exclusivity if we obtain orphan drug exclusivity for FXS or another approved orphan indication.

We have rights to a patent portfolio and will continue to develop a patent portfolio around our product candidate brelovitug. We have rights to two issued U.S. patents, U.S. Patent Nos. 11,932,681 and 12,331,104, covering brelovitug as a composition-of-matter and methods of treating hepatitis B, as well as a pending counterpart in the United States and issued foreign counterparts in China and Japan, as well as pending foreign counterparts in China, Europe, Hong Kong, and Japan, which are set to expire in 2039-2041 absent any patent term extensions. We have rights to pending United States, Australia, Canada, Europe, Israel, India, Japan, Korea, Mongolia, New Zealand, Singapore, United Arab Emirates, and Taiwan applications covering methods of treating hepatitis D using brelovitug, which are set to expire in 2044, absent any patent term adjustment or extensions. We have rights to a pending United States provisional application covering the pharmaceutical compositions of brelovitug, and any patent issued claiming priority to the provisional will expire in 2046 absent any patent term adjustments or extensions. Patents related to brelovitug may be eligible for patent term extensions in certain jurisdictions, including the United States, upon approval of a commercial use of the corresponding product by a regulatory agency in the jurisdiction where the patent was granted.

Upon approval in the United States, as brelovitug has not previously been approved in the United States for any indication, it may be eligible for a 12-year biologics exclusivity which would run concurrently with its seven years of orphan drug exclusivity if we obtain orphan drug exclusivity for chronic HDV treatment or another approved orphan indication.

In addition to patent protection, we rely on trade secret protection, trademark protection and know-how to expand our proprietary position around our chemistry, technology and other discoveries and inventions that we consider important to our business. We are a party to a number of license agreements under which we are granted intellectual property rights to know-how that are important to our business. We have access to, or have licensed, know-how related to Livmarli in the United States, Europe and other countries from Pfizer and Shire. We have licensed know-how related to ASBTi Technology and TGR5 Technology from Satiogen. We have access to, or have licensed, know-how related to volixibat from Sanofi and Shire. We have licensed know-how related to brelovitug from Novartis. We have licensed know-how related to MRM-3379 from Enthorin. Our existing license agreements as related to Livmarli, volixibat, brelovitug and MRM-3379 impose various development, regulatory and/ commercial diligence obligations, payment of milestones and/or royalties and other obligations.

In addition, we currently have orphan drug exclusivity for Livmarli for the treatment of ALGS and PFIC in the United States and the EU, providing seven years of market exclusivity in the United States, which can be extended to seven and a half years if trials are conducted in accordance with an agreed-upon pediatric investigational plan, and ten years of market exclusivity in the EU, which has been extended to 12 years in the EU for the treatment of ALGS in view of a pediatric award and may be extended to 12 years in the EU for PFIC. In addition, we currently have orphan drug designation for volixibat for the treatment of PBC in the United States and in the EU, providing the opportunity to receive seven years of market exclusivity in the United States, which can be extended to seven and a half years if trials are conducted in accordance with an agreed-upon pediatric investigational plan, and ten years of market exclusivity in the EU, which can be extended to 12 years in the EU if trials are conducted in accordance with an agreed-upon pediatric investigational plan.

In the United States, maralixibat has been granted new chemical entity ("NCE") exclusivity until September 29, 2026. This five years of post-FDA approval exclusivity runs concurrently with its seven years orphan drug exclusivity for the treatment of ALGS. Upon approval in the United States, as volixibat has not previously been approved in the United States for any indication, it may be eligible for five years of NCE exclusivity, which would run concurrently with its seven years of orphan drug exclusivity if we obtain orphan drug exclusivity for an approved orphan indication.

We also seek to protect our intellectual property in part by entering into confidentiality agreements with companies with whom we share proprietary and confidential information in the course of business discussions, and by having confidentiality terms in our agreements with our employees, consultants, scientific advisors, clinical investigators and other contractors and also by requiring our employees, commercial contractors, and certain consultants and investigators, to enter into invention assignment agreements that grant us ownership of any discoveries or inventions made by them while in our employ.

Furthermore, we seek trademark protection in the United States and internationally where available and when we deem appropriate.

Sales, Marketing and Distribution

We believe we have built the commercial infrastructure necessary to effectively support the commercialization of our approved medicines in North America and certain countries in Europe and are using strategic partners, and distributors to assist in the commercialization of our approved medicines in other markets.

The commercial infrastructure for orphan products typically consists of a targeted, specialty sales force that calls on a limited and focused group of physicians supported by sales management, internal sales support, an internal marketing group and distribution support. Additional capabilities important to the marketplace include the management of key accounts such as managed care organizations, group-purchasing organizations, specialty pharmacies, government accounts and reimbursement support. Based on the number of physicians that treat cholestatic liver diseases, we have designed our commercial organization to target the relevant audience for our approved medicines in North America and certain countries in Europe primarily through an internal sales force. To maintain and further develop the appropriate commercial infrastructure, we have invested and expect to continue to invest significant amounts of financial and management resources in our commercial organization.

In addition, we have built a medical affairs organization and multiple capabilities across North America and Europe to meet the scientific and medical educational needs of the healthcare providers and patients in the rare disease community that are focused on providing accurate disease state and balanced product information for appropriate

management of patients with rare disorders. Medical affairs is comprised of medical information, patient advocacy, patient diagnosis, medical science liaisons, research and educational grants.

Our approved medicines are currently distributed in the U.S. and Canada, through a single specialty pharmacy in each country. In other geographies, our approved medicines are sold direct to pharmacies by our third-party logistics providers, authorized distributors or licensed partners. Pharmacies and authorized distributors act as intermediaries between us and the end-users and generally do not stock significant quantities of our products. In certain countries, governments place large periodic orders. The timing of these orders can be inconsistent and can create quarter-to-quarter variation in revenue.

Manufacturing

We do not own or operate manufacturing facilities for the production of our approved medicines or our product candidates that we may develop, nor do we have plans to develop our own manufacturing operations in the foreseeable future. We currently depend on third-party contract manufacturers for all of our required raw materials, active pharmaceutical ingredient and finished products, including clinical supplies. Over the course of the development of our IBATis we have used and continue to use multiple third-party contract manufacturers. We have entered into and expect to continue to enter into agreements for commercial production of our approved medicines. We do not have any current contractual arrangements for the manufacture of commercial supplies of volixibat. We have entered into an agreement with Lonza for the manufacture of brelovitug. We currently employ internal resources and third-party consultants to manage our manufacturing contractors.

Competition

The biotechnology and pharmaceutical industries are subject to rapid and intense technological change. We face, and will continue to face, competition in the development and marketing of our products and product candidates from academic institutions, government agencies, research institutions and biotechnology and pharmaceutical companies. Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approval and marketing than we do.

Competition may also arise from, among other things, new drug development technologies, new or improved treatment options for preventing or reducing the incidence of disease in diseases our products treat and new small molecule or other classes of therapeutic agents. Such developments by competitors could reduce or eliminate the use of our products or may limit the utility and application of ongoing clinical trials for our product candidates.

We are aware of two other companies pursuing clinical development and commercialization of therapies that reduce sBA levels via the IBAT pathway. GlaxoSmithKline plc (“GSK”) and Ipsen Pharma (“Ipsen”) have IBATs in clinical development for cholestatic liver diseases.

We are aware Ipsen has received approval for odevixibat (Bylvay) for the treatment of pruritus in patients with PFIC and cholestatic pruritus in patients with ALGS in the U.S., and for the treatment of PFIC in the EU and for odevixibat (Kayfanda) for the treatment of cholestatic pruritus in ALGS. In the EU, Bylvay and Kayfanda are authorized under exceptional circumstances. Ipsen has opened enrollment in their ALGS open-label extension study to infants 11 months or younger and is also conducting a study of odevixibat in biliary atresia and plans to pursue other cholestatic liver diseases. GSK announced in November 2024 that the Phase 3 GLISTEN trial with linerixibat in PBC met its primary pruritus endpoint and that GSK has submitted marketing applications to the U.S., EU and other health authorities with potential approvals expected in 2026.

Other off-label medications are also used in ALGS, PFIC, PSC and PBC for cholestatic pruritus such as Ursodeoxycholic acid (“UDCA”), cholestyramine and other bile salt resins, rifampin, naltrexone and other agents, such as selective serotonin reuptake inhibitors. Further, we may compete with companies that are developing gene therapy for the treatment of PFIC. Additionally, surgical interventions, such as partial external biliary diversion and nasobiliary drainage, and extracorporeal liver support, such as Molecular Adsorbent Recirculation System, are also employed in an attempt to lower bile acid levels, manage pruritus and improve measures of liver function.

In adult settings of cholestasis, similar to pediatric settings, cholestyramine, UDCA, rifampin and naltrexone are commonly used agents. We are aware that Alfasigma S.p.’s (formerly Intercept Pharmaceuticals, Inc.) Ocaliva, Gilead Science’s Livdelzi, and Ipsen’s Iqirvo are approved as a second-line treatment for PBC in patients with inadequate response to ursodeoxycholic acid. We are aware of several agents in clinical development for the treatment of PBC including Alfasigma’s Ocaliva and bezafibrate, Zydus Therapeutics Inc.’s saroglitazar magnesium, Calliditas Therapeutics AB’s setanaxib, COUR Pharmaceuticals’ CNP-104, Umecrine Cognition’s golexanolone, Kowa Company Ltd’s K-808, HighTide Therapeutics Inc.’s HTD-1801, Hepagene Therapeutics Inc.’s HPG-1860, Tharimmune Inc.’s TH-104, Cascade Pharmaceuticals Inc.’s CS-0159, and GSK’s linerixibat, another IBATi.

We are not aware of FDA or European Commission approved therapeutics for the treatment of PSC. We are aware of several agents in clinical development for the treatment of PSC, including Dr. Falk Pharma's Norucholic acid, HighTide Therapeutics Inc.'s HTD-1801, Alfasigma's Ocaliva, or obeticholic acid, Ipsen's elafibranor and ritivixibat, NGM Biopharmaceuticals Inc.'s NGM282, Chemomab Therapeutics Ltd.'s CM-101, Cascade Pharmaceuticals Inc.'s CS-0159, LISCure Biosciences Inc.'s LB-P8, Halo Biosciences Inc.'s HB-1614, ProQR Therapeutics N.V.'s AX-0810, Rectify Pharmaceuticals, Inc.'s RTY-694 and Pliant Therapeutics' bexotegrast.

There are other approved chenodeoxycholic acid products available outside of the U.S. Both Dr. Falk Pharma GmbH and Leadiant Biosciences, Inc. have FDA Orphan Drug Designations granted for the treatment of CTX (granted in 2004 and 2007, respectively), and we believe that Leadiant Biosciences, based on publicly available information, may be conducting a clinical study in CTX. There are currently no FDA-approved treatments in the U.S. that compete with Cholbam. There are other approved cholic acid products available outside of the U.S. and Laboratoires CTRS has received approval from the EMA for a version of cholic acid.

There are currently no approved products available in the U.S. for the treatment of chronic HDV infection. Bulevirtide, which is commercialized by Gilead Sciences, is approved for commercial sale in the EU and Gilead is seeking approval from the FDA to commercialize bulevirtide in the U.S. In addition, we are aware that Vir Biotechnology is developing tobevibart in combination with elebsiran for the treatment of chronic HDV. We are aware of several other agents in clinical development for the treatment of chronic HDV including Gilead Science's GS-4321, Shanghai HEP Pharmaceutical Co., Ltd.'s Hepalatide, Suzhou Ribo Life Science Co., Ltd.'s RBD1016, Assembly Biosciences's ABI-6250, Huahui Health Ltd.'s Libevitug and HH-1270, Replicor Inc.'s REP 2139-Mg and REP 2139-Ca, and EIT Pharma's Jitixib® (Lonafarnib).

Under the Hatch-Waxman Amendments of the Federal Food, Drug, and Cosmetic Act (the "Hatch-Waxman Act"), a pharmaceutical manufacturer may file an abbreviated new drug application ("ANDA") seeking approval of a generic copy of an approved innovator product or an NDA under Section 505(b)(2) that relies on the FDA's prior findings of safety and effectiveness in approving the innovator product. A Section 505(b)(2) NDA may be for a new or improved version of the original innovator product. Certain of our approved medicines, including Ctexli and Cholbam, are or may be subject to immediate competition from compounded and generic entrants, as the ANDA and NDA for these drug products have no remaining or current patent or non-patent exclusivity although Ctexli does have orphan designation for the CTX indication. Further, as described in more detail in Part I, Item 1A, "Risk Factors" and Note 15 in the notes to our consolidated financial statements appearing elsewhere in this Annual Report, we have initiated litigation against certain parties alleging infringement of certain "Orange Book" listed patents covering LIVMARLI in accordance with the procedures set out in the Hatch-Waxman Act following the submission by such parties of ANDAs directed to generic versions of LIVMARLI. We cannot make any predictions about the final outcome of these matters or the timing thereof.

In December 2019, the Creating and Restoring Equal Access to Equivalent Samples Act of 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. 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.

We are not aware of FDA or European Commission approved therapeutics for the treatment of FXS. We are aware of one other company, Shionogi & Co., LTD., pursuing clinical development of a PDE4D inhibitor (zatomilast/ BPN14770) in FXS. We are aware of several other companies pursuing clinical development of therapies for FXS including Harmony Biosciences Inc.'s ZYN002, Allos Pharma Inc.'s Arbaclofen, Healx Ltd.'s Gabaxodol, Spinogenix Inc.'s SPG601, Connecta Therapeutics S.L.'s CTH120, and Kaerus Therapeutics Inc.'s KER-0193.

Government Regulation and Product Approval

As a biopharmaceutical company that operates globally, we are subject to extensive regulation. Government authorities in the United States (at the federal, state and local level) and in other countries extensively regulate, among other things, the research, development, testing, manufacturing, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing and export and import of drug products such as those we are developing. Generally, our activities in other countries are or will be subject to regulation that is similar in nature and scope as that imposed in the United States, although there can be important differences. Additionally, some significant aspects of regulation in the EU are addressed in a centralized way, but country-specific regulation remains essential in many respects.

U.S. Biopharmaceutical Development Process

In the United States, the FDA regulates drugs under the Federal Food, Drug and Cosmetic Act ("FDCA"), and biologics additionally under the Public Health Service Act, and their implementing regulations. Biopharmaceuticals are also subject to other federal, state and local statutes and regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant to administrative or judicial sanctions. FDA sanctions could include, among other actions, refusal to approve pending applications, withdrawal of an approval, a clinical hold, warning letters, product recalls or withdrawals from the market, product seizures, total or partial suspension of production or distribution injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us. The process required by the FDA before a drug or biologic may be marketed in the United States generally involves the following:

- completion of extensive preclinical laboratory tests, preclinical animal studies and formulation studies in accordance with applicable regulations, including the FDA's Good Laboratory Practice ("GLP"), regulations and other applicable regulations;
- submission to the FDA of an investigational new drug ("IND"), which must become effective before human clinical trials may begin;
- approval by an independent institutional review board ("IRB"), at each clinical site before each trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with applicable regulations, including the FDA's good clinical practice ("GCP"), regulations to establish the safety and efficacy of the proposed drug for its proposed indication;
- submission to the FDA of an NDA for a new drug or Biologics License Application ("BLA") for a biologic;
- satisfactory completion of an FDA advisory committee review, if applicable;
- a determination by the FDA within 60 days of its receipt of an NDA or BLA to file the application for review;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities where the drug is produced to assess compliance with the FDA's current good manufacturing practice ("cGMP"), requirements to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity;
- potential FDA inspection of the preclinical and/or clinical trial sites that generated the data in support of the NDA or BLA; and
- FDA review and approval of the NDA or BLA prior to any commercial marketing or sale of the drug or biologic in the United States.

Before testing any compounds with potential therapeutic value in humans, the candidate enters the preclinical testing stage. Preclinical tests include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies, to assess the potential safety and activity of the candidate. The conduct of the preclinical tests must comply with federal regulations and requirements including GLPs. The sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. An IND is a request for authorization from the FDA to administer an investigational drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol(s) for human trials. Some preclinical testing may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA raises concerns or questions regarding the proposed clinical trials and places the IND on clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. The FDA may also impose clinical holds at any time before or during clinical trials due to safety concerns or non-compliance.

Clinical trials involve the administration of the investigational candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the trial sponsor's control, in accordance with GCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives

of the clinical trial, dosing procedures, subject selection and exclusion criteria and the parameters to be used to monitor subject safety and assess efficacy. Each protocol, and any subsequent amendments to the protocol, must be submitted to the FDA as part of the IND. Further, each clinical trial must be reviewed and approved by an IRB, at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of trial participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the informed consent form that must be provided to each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed. There are also requirements governing the reporting of ongoing clinical trials and completed clinical trial results to public registries.

Human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

- *Phase 1.* The candidate is initially introduced into healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution and excretion, the side effects associated with increasing doses and if possible, to gain early evidence of effectiveness. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.
- *Phase 2.* The candidate is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases or conditions and to determine dosage tolerance, optimal dosage and dosing schedule.
- *Phase 3.* Clinical trials are undertaken to further evaluate dosage, clinical efficacy and safety in an expanded patient population at geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall benefit/risk ratio of the product and provide an adequate basis for product approval. Generally, two adequate and well-controlled Phase 3 clinical trials are required by the FDA for approval of an NDA.

Post-approval studies, or Phase 4 clinical trials, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication. In certain instances, FDA may mandate the performance of Phase 4 trials. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval of an NDA or BLA.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and written IND safety reports must be submitted to the FDA and the investigators for serious and unexpected adverse events or any finding from tests in laboratory animals that suggests a significant risk for human subjects. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. The FDA, the IRB, or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the candidate has been associated with unexpected serious harm to patients. Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as a data safety monitoring board or committee. This group provides authorization for whether or not a trial may move forward at designated check points based on access to certain data from the trial.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the candidate as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product and, among other things, must develop methods for testing the identity, strength, quality and purity of the final product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product does not undergo unacceptable deterioration over its shelf life.

U.S. Review and Approval Processes

The results of product development, preclinical studies and clinical trials, along with descriptions of the manufacturing process, analytical tests conducted on the chemistry of the drug, proposed labeling and other relevant information are submitted to the FDA as part of an NDA or BLA requesting approval to market the product. Data may come from company-sponsored clinical trials intended to test the safety and effectiveness of a use of a product, or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and effectiveness of the investigational product

to the satisfaction of the FDA. The submission of an NDA or BLA is subject to the payment of substantial user fees; a waiver of such fees may be obtained under certain limited circumstances.

In addition, the Pediatric Research Equity Act ("PREA"), requires a sponsor to conduct pediatric clinical trials for most drugs, for a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration. Under PREA, original applications and supplements must contain a pediatric assessment unless the sponsor has received a deferral or waiver. The required assessment must evaluate the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations and support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The sponsor or FDA may request a deferral of pediatric clinical trials for some or all of the pediatric subpopulations. A deferral may be granted for several reasons, including a finding that the drug is ready for approval for use in adults before pediatric clinical trials are complete or that additional safety or effectiveness data needs to be collected before the pediatric clinical trials begin. The FDA must send a non-compliance letter to any sponsor that fails to submit the required assessment, keep a deferral current or fails to submit a request for approval of a pediatric formulation. Unless otherwise required by regulation, the Pediatric Research Equity Act does not apply to any product for an indication for which orphan designation has been granted. However, if only one indication for a product has orphan designation, a pediatric assessment may still be required for any applications to market that same product for the non-orphan indication(s).

The FDA reviews all NDAs and BLAs submitted before it accepts them for filing and may request additional information rather than accepting the application for filing. The FDA must make a decision on accepting an application for filing within 60 days of receipt. Once the submission is accepted for filing, the FDA begins an in-depth review. Under the PDUFA, guidelines that are currently in effect, the FDA has a goal of ten months from the date of "filing" of a standard application to review and act on the submission. This review typically takes twelve months from the date the application is submitted to FDA because the FDA has approximately two months to make a "filing" decision after it the application is submitted. The FDA does not always meet its PDUFA goal dates for standard and priority NDAs, and the review process is often significantly extended by FDA requests for additional information or clarification.

After the NDA submission is accepted for filing, the FDA reviews the application to determine, among other things, whether the proposed product is safe and effective for its intended use (safe, pure and potent for a biologic) and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, strength, quality and purity. The FDA may refer applications for novel products or products which 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 and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions and typically follows the advisory committee's recommendations.

Before approving an NDA or BLA, the FDA will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA or BLA, the FDA may inspect one or more clinical sites to assure compliance with GCP requirements. After the FDA evaluates the application, manufacturing process and manufacturing facilities, it may issue an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete and the application will not be approved in its present form. A Complete Response Letter usually describes all of the specific deficiencies in the application identified by the FDA. The Complete Response Letter may require additional clinical data and/or (an) additional pivotal Phase 3 clinical trial(s), and/or other significant and time-consuming requirements related to clinical trials, preclinical studies or manufacturing. If a Complete Response Letter is issued, the applicant may either resubmit the application, addressing all of the deficiencies identified in the letter, or withdraw the application. Even if such data and information is submitted, the FDA may ultimately decide that the application does not satisfy the criteria for approval.

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. A product may also receive accelerated approval requiring confirmatory studies for full approval. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling or may condition the approval of the NDA on other changes to the proposed labeling, development of adequate controls and specifications, or a commitment to conduct one or more post-market studies or clinical trials. For example, the FDA may require Phase 4 testing, which involves clinical trials designed to further assess a drug safety and effectiveness, and may require testing and surveillance programs to monitor the safety of approved products that have been commercialized. The FDA may also determine that a risk evaluation and mitigation strategy ("REMS") is necessary to assure the safe use of the drug. If the FDA concludes a REMS is needed, the sponsor of the NDA must submit a proposed REMS; the FDA will not approve the

NDA without an approved REMS, if required. A REMS could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug intended to treat a rare disease or condition, which is a disease or condition that affects fewer than 200,000 individuals in the United States or, if it affects more than 200,000 individuals in the United States, there is no reasonable expectation that the cost of developing and making a drug product available in the United States for this type of disease or condition will be recovered from sales of the product. Orphan designation must be requested before submitting an NDA or BLA. After the FDA grants orphan designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same drug or biological product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity or inability to manufacture the product in sufficient quantities. The designation of such drug also entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. Competitors, however, may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Orphan exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval of the same drug as defined by the FDA or if our product candidate is determined to be contained within the competitor's product for the same indication or disease. If an orphan designated product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan exclusivity. Orphan drug status in the EU has similar but not identical benefits in that jurisdiction.

Expedited Development and Review Programs

The FDA has a Fast Track designation program that is intended to expedite or facilitate the process for reviewing new drug and biologic products that are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. Unique to a Fast Track product, the FDA may consider for review sections of the NDA or BLA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the application, the FDA agrees to accept sections and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the application.

Any product submitted to the FDA for approval, including a product with a Fast Track designation, may also be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. A product is eligible for priority review if it has the potential to provide safe and effective therapy for a serious condition where no satisfactory alternative therapy exists or a significant improvement in the treatment, diagnosis or prevention of a serious condition compared to marketed products. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug designated for priority review in an effort to facilitate the review.

In addition, a product may be eligible for accelerated approval. Drug products intended to treat serious or life-threatening diseases or conditions may be eligible for accelerated approval upon a determination that the product has an effect on 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. As a condition of approval, the FDA may require that a sponsor of a drug receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product. Fast track designation, priority review and accelerated approval do not change the standards for approval but may expedite the development or approval process.

A sponsor may seek FDA designation of a drug candidate as a "breakthrough therapy" if the drug is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The designation includes intensive FDA interaction and guidance. If a drug is designated as breakthrough therapy, the FDA will expedite the development and review of such drug. Breakthrough therapy designation includes all of the Fast Track program features, as well as more intensive FDA interaction and guidance. The breakthrough therapy designation is a

distinct status from both accelerated approval and priority review, which can also be granted to the same drug if relevant criteria are met. If a product is designated as breakthrough therapy, the FDA will work to expedite the development and review of such drug.

Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened. In addition, this designation may not provide a material commercial advantage.

Post-Approval Requirements

Any drug products for which we receive FDA approvals are subject to continuing regulation by the FDA, including, among other things, record-keeping requirements, reporting of adverse experiences with the product, providing the FDA with updated safety and efficacy information, product sampling and distribution requirements, and complying with FDA promotion and advertising requirements, which include, among others, standards for direct-to-consumer advertising, restrictions on promoting drugs for uses or in patient populations that are not described in the products's approved labeling (known as "off-label use"), limitations on industry-sponsored scientific and educational activities, and requirements for promotional activities involving the internet. Although physicians may prescribe legally available products for off-label uses, manufacturers may not market or promote such off-label uses.

In addition, quality control and manufacturing procedures must continue to conform to applicable manufacturing requirements after approval to ensure the long-term stability of the drug product. We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our products in accordance with cGMP regulations. cGMP regulations require among other things, quality control and quality assurance as well as the corresponding maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP. Manufacturers and other entities involved in the manufacture and distribution of approved biopharmaceuticals are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer, or holder of an approved NDA or BLA, including, among other things, recall or withdrawal of the product from the market. In addition, changes to the manufacturing process are strictly regulated, and depending on the significance of the change, may require prior FDA approval before being implemented. Other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

The FDA also may require post-marketing testing, known as Phase 4 testing, and surveillance to monitor the effects of an approved product. Discovery of previously unknown problems with a product or the failure to comply with applicable FDA requirements can have negative consequences, including adverse publicity, judicial or administrative enforcement, warning letters from the FDA, mandated corrective advertising or communications with doctors, and civil or criminal penalties, among others. Newly discovered or developed safety or effectiveness data may require changes to a product's approved labeling, including the addition of new warnings and contraindications, and also may require the implementation of other risk management measures. Also, new government requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could delay or prevent regulatory approval of our products under development.

U.S. Patent Term Restoration and Marketing Exclusivity

Depending upon the timing, duration and specifics of the FDA approval of the use of our product candidates, some of our U.S. patents, if granted, may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years, as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time during the patent's term between the effective date of an IND and the submission date of an NDA or BLA plus the time during the patent's term between the submission date of an NDA or BLA and the approval of that application. Only one patent applicable to an approved drug is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The U.S. Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may intend to apply for restoration of patent term for one of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant NDA or BLA.

Market exclusivity provisions under the FDCA can also delay the submission or the approval of certain marketing applications. The FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to obtain approval of an NDA for a NCE. A drug is a NCE if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not approve or even accept for review an abbreviated new drug application ("ANDA"), or a 505(b)(2) NDA submitted by another company for another drug based on the same active moiety, regardless of whether the drug is intended for the same indication as the original innovative drug or for another indication, where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity and/or non-infringement to one of the patents listed in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (Orange Book). The FDCA alternatively provides three years of marketing exclusivity for an NDA, or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the modification for which the drug received approval on the basis of the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the active agent for the original indication or condition of use. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct, or obtain a right of reference to, all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Reference biological products are granted 12 years of data exclusivity from the time of first licensure of the product. The FDA will not accept an application for a biosimilar or interchangeable product based on the reference biological product until four years after the date of first licensure of the reference product.

Orphan drug exclusivity, as described above, may offer a seven-year period of marketing exclusivity for the approved indication, except in certain circumstances. Pediatric exclusivity is another type of non-patent market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric trial in accordance with an FDA-issued "Written Request" for such a trial.

Other U.S. Healthcare Laws and Compliance Requirements

We are subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which we conduct our business. In the United States, such laws include, without limitation, state and federal anti-kickback, fraud and abuse, false claims, privacy and security, price reporting, and health care provider transparency or "sunshine" laws and regulations.

The federal Anti-Kickback Statute prohibits, among other things, any person or entity, from knowingly and willfully offering, paying, soliciting or receiving any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any item or service reimbursable under Medicare, Medicaid or other federal healthcare programs. The term remuneration has been interpreted broadly to include anything of value. The Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers, and formulary managers on the other. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution. The exceptions and safe harbors are drawn narrowly and practices that involve remuneration that may be alleged to be intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exception 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 of its facts and circumstances. Our practices may not in all cases meet all of the criteria for protection under a statutory exception or regulatory safe harbor.

Additionally, the intent standard under the Anti-Kickback Statute and the criminal healthcare fraud statutes (discussed below) was amended by the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act (collectively, the "Affordable Care Act"), 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 Affordable Care Act 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 False Claims Act (discussed below).

The federal False Claims Act, as well as the civil monetary penalty law, prohibit, among other things, any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to, or approval by, the federal government or knowingly making, using, or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government. As a result of a modification to the federal False Claims Act made by the Fraud Enforcement and Recovery Act of 2009, a claim includes "any request or demand" for money or property presented to the U.S. government. Pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. Companies have also been prosecuted for causing false claims to be submitted because of the companies' marketing of the product for unapproved, and thus non-covered, uses.

The Health Insurance Portability and Accountability Act ("HIPAA") also created new federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud or to obtain, by means of false or fraudulent pretenses, representations or promises, any money or property owned by, or under the control or custody of, any healthcare benefit program, including private third-party payors and knowingly and willfully falsifying, concealing or covering up by trick, scheme or device, 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. Also, many states have similar fraud and abuse statutes or regulations that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor.

Additionally, the federal Physician Payments Sunshine Act within the Affordable Care Act, and its implementing regulations, require that certain manufacturers of drugs, devices, biological and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) annually report information related to certain payments or other transfers of value made or distributed to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other healthcare professionals (such as physician assistants and nurse practitioners), and teaching hospitals, certain ownership and investment interests held by physicians and their immediate family members.

We may also be subject to data privacy and security regulations 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 its implementing regulations, impose requirements relating to the privacy, security and transmission of individually identifiable health information. Among other things, HITECH makes HIPAA's privacy and security standards directly applicable to business associates, independent contractors or agents of covered entities that receive or obtain protected health information in connection with providing a service on behalf of a covered entity. HITECH also created four new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions. In addition, state laws govern the privacy and security of health information in specified circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

We also are or will become subject to privacy laws in the jurisdictions in which we are established or in which we sell or market our products or run clinical trials. For example, in relation to clinical trials in Europe, we are subject to Regulation (EU) 2016/679, the General Data Protection Regulation and similar laws in European countries outside of the EU (collectively, the "GDPR"), in relation to our collection, control, processing and other use of personal data (i.e., data relating to an identifiable living individual). We process personal data in relation to participants in our clinical trials in the European Economic Area, including the health and medical information of these participants. The GDPR also provides that EU Member States may introduce further conditions, including limitations which could limit our ability to collect, use and share personal data (including health and medical information), or could cause our compliance costs to increase, ultimately having an adverse impact on our business. The GDPR imposes onerous accountability obligations requiring data controllers and processors to maintain a record of their data processing and implement policies as part of its mandated privacy governance framework. It also requires data controllers to be transparent and disclose to data subjects (in a concise, intelligible and easily accessible form) how their personal data is to be used, imposes limitations on retention of personal data; defines for the first time pseudonymized (i.e., key-coded) data; introduces mandatory data breach notification requirements; and sets higher standards for data controllers to demonstrate that they have obtained valid consent for certain data processing activities. We are also subject to EU rules with respect to cross-border transfers of personal data out of the EU and European Economic Area. We are subject to the supervision of local data protection authorities in those EU jurisdictions where we are established or otherwise subject to the GDPR, and we maintain an office in Switzerland, which has similar privacy and data protection laws and regulations. Fines for certain breaches of the GDPR are significant: up to the greater of €20 million or 4% of total global annual turnover. In addition to the foregoing, a breach of the GDPR or other

applicable privacy and data protection laws and regulations could result in regulatory investigations, reputational damage, orders to cease/change our use of data, enforcement notices, or potential civil claims including class action type litigation.

In addition, 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 may include the right to access, correct, or delete certain personal data, and to opt-out of certain data processing activities, such as targeted advertising, profiling, and automated decision-making. The exercise of these rights may impact our business and ability to provide our products and services. Certain states also impose stricter requirements for processing certain personal data, including sensitive information, such as conducting data privacy impact assessments. These state laws allow for statutory fines for noncompliance. For example, California enacted the California Consumer Privacy Act ("CCPA"), which applies to personal data of consumers, business representatives, and employees who are California residents, and requires businesses to provide specific disclosures to California residents and honor individuals' requests to exercise certain privacy rights. The CCPA provides for civil penalties for violations and private right of action for certain data breaches. While certain clinical trial activities are exempt from the CCPA and other U.S. consumer privacy laws' requirements, these developments may increase our compliance costs, exposure to regulatory enforcement action and other liabilities.

In order to distribute products commercially, we must comply with state laws that require the registration of manufacturers and wholesale distributors of pharmaceutical products in a state, including, in certain states, manufacturers and distributors who ship products into the state even if such manufacturers or distributors have no place of business within the state. Some states also impose requirements on manufacturers and distributors to establish the pedigree of product in the chain of distribution, including some states that require manufacturers and others to adopt new technology capable of tracking and tracing product as it moves through the distribution chain. Several states have enacted legislation requiring pharmaceutical companies to establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales, marketing, pricing, track and report gifts, compensation and other remuneration made to physicians and other healthcare providers, clinical trials and other activities, and/or register their sales representatives, as well as to prohibit pharmacies and other healthcare entities from providing certain physician prescribing data to pharmaceutical companies for use in sales and marketing, and to prohibit certain other sales and marketing practices. All of our activities are potentially subject to federal and state consumer protection and unfair competition laws.

If our operations are found to be in violation of any of the federal and state healthcare laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including without limitation, significant civil, criminal and/or administrative penalties, damages, fines, disgorgement, exclusion from participation in government programs, such as Medicare and Medicaid, injunctions, imprisonment, private "qui tam" actions brought by individual whistleblowers in the name of the government, or refusal to allow us to enter into government contracts, contractual damages, reputational harm, administrative burdens, 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.

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any product candidates for which we or our collaborators obtain regulatory approval. In the United States and other countries, sales of pharmaceuticals, including Livmarli, depend, in part, on the extent to which third-party payors provide coverage, and establish adequate reimbursement levels for such drug products.

In the United States, third-party payors include federal and state healthcare programs, government authorities, private managed care providers, private health insurers and other organizations. Third-party payors are increasingly challenging the price, examining the medical necessity and reviewing the cost-effectiveness of medical drug products and medical services, in addition to questioning their safety and efficacy. For example, the Inflation Reduction Act ("IRA") among other things, requires the U.S. Department of Health and Human Services ("HHS") to negotiate the price of certain single-source drugs that have been on the market for at least seven (7) years covered under Medicare as part of the Medicare Drug Price Negotiation Program. Each year up to twenty (20) products will be selected by HHS for the Medicare Drug Price Negotiation Program. Products subject to the Medicare Drug Price Negotiation Program are expected to experience a significant reduction in reimbursement from the Medicare program on a per unit basis. In addition, the IRA imposes rebates on many Medicare Part B and Medicare Part D products to penalize price increases that outpace inflation on an annual basis. Additionally, such payors may limit coverage to specific drug products on an approved list, also known as a formulary, which might not include all of the FDA-approved drugs for a particular indication. Third party payors may require pharmaceutical companies to conduct expensive pharmaco-economic studies in order to demonstrate the medical necessity and cost-effectiveness of their products, in addition to the costs required to obtain the FDA approvals. Nonetheless, payors may determine that such products may not be considered medically necessary or cost-effective.

Moreover, the process for determining whether a third-party payor will provide coverage for a drug product may be separate from the process for setting the price of a drug product or for establishing the reimbursement rate that such a payor will pay for the drug product. A payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Further, one payor's determination to provide coverage for a drug product does not assure that other payors will also provide coverage for the drug product. Adequate third-party reimbursement may not be available for Livmarli and other drug products we may develop to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

If we elect to participate in certain governmental programs, we may be required to participate in discount and rebate programs, which may result in prices for our future products that will likely be lower than the prices we might otherwise obtain. For example, drug manufacturers participating under the Medicaid Drug Rebate Program must pay rebates on prescription drugs to state Medicaid programs. Under the Veterans Health Care Act ("VHCA"), drug companies are required to offer certain drugs at a reduced price to a number of federal agencies, including the U.S. Department of Veterans Affairs and Department of Defense, the Public Health Service and certain private Public Health Service designated entities in order to participate in other federal funding programs, including Medicare and Medicaid. Recent legislative changes require that discounted prices be offered for certain U.S. Department of Defense purchases for its TRICARE program via a rebate system. Participation under the VHCA also requires submission of pricing data and calculation of discounts and rebates pursuant to complex statutory formulas, as well as the entry into government procurement contracts governed by the Federal Acquisition Regulations. The overall funding of certain government programs such as Medicaid and Medicare is uncertain and there is no guarantee that funds approved by the U.S. Congress will be made available by the current administration. If our products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply.

Different pricing and reimbursement schemes exist in other countries. In the EU, governments influence the price of pharmaceutical products through their pricing and reimbursement rules and control of national health care systems that fund a large part of the cost of those products to consumers. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost-effectiveness of a particular drug candidate to currently available therapies. Other member states allow companies to fix their own prices for medicines, but monitor and control company profits. The downward pressure on health care costs in general, particularly prescription drugs, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert a commercial pressure on pricing within a country.

The marketability of Livmarli and any product candidates for which we or our collaborators receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement. In addition, emphasis on managed care in the United States has increased and we expect will continue to increase the pressure on pharmaceutical pricing. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for Livmarli or any other products for which we or our collaborators receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Healthcare Reform

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medical products and services, implementing reductions in Medicare and other healthcare funding and applying new payment methodologies. For example, in March 2010, the Affordable Care Act was enacted, which affected existing government healthcare programs and resulted in the development of new programs.

There have been amendments and executive, judicial and Congressional challenges to certain aspects of the Affordable Care Act. For example, on July 4, 2025, the One Big Beautiful Bill Act ("OBBBA") was signed into law, which narrowed access to Affordable Care Act marketplace exchange enrollment and declined to extend the Affordable Care Act enhanced advanced premium tax credits that expired at the end of 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. The OBBBA also is expected to reduce Medicaid spending and enrollment by implementing work requirements for some beneficiaries, capping state-directed payments, reducing federal funding, and limiting provider taxes used to fund the program. Congress is considering proposed legislation intended to further reduce healthcare costs with alternatives to replace the expired Affordable Care Act subsidies. It is unclear how such challenges and the healthcare reform measures of the current presidential administration will impact the Affordable Care Act and our business.

The current administration is pursuing policies to reduce regulations and expenditures across government agencies including at HHS, the FDA, Centers for Medicare & 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. For example, the current administration has announced agreements with pharmaceutical companies that require the drug manufacturers to offer, through a direct-to-consumer platform, U.S. patients and Medicaid programs prescription drug Most-Favored Nation pricing equal to or lower than those paid in other developed nations, with additional mandates for direct-to-patient discounts and repatriation of foreign revenues. Other recent actions, for example, include (1) directing agencies to reduce agency workforce and cut programs; (2) directing HHS and other agencies to lower prescription drug costs through a variety of initiatives, including by improving upon the Medicare Drug Price Negotiation Program and establishing Most-Favored-Nation pricing for pharmaceutical products; (3) imposing tariffs on imported pharmaceutical products; and (4) as part of the Make America Healthy Again Commission's Strategy Report released in September 2025, working across government agencies to increase enforcement on direct-to-consumer pharmaceutical advertising. Additionally, the current administration recently called on Congress to enact "The Great Healthcare Plan," to codify and expand Most-Favored Nation pricing, lower government subsidies to private insurance companies, increase healthcare price transparency, expand pharmaceutical drugs available for over-the-counter purchase, and enact restrictions on pharmacy benefit manager (PBM) payment methodologies, among other things. These actions and policies may significantly reduce U.S. drug prices, potentially impacting manufacturers' global pricing strategies and profitability, while increasing their operational costs and compliance risks. Additionally, in its June 2024 decision in Loper Bright Enterprises v. Raimondo ("Loper Bright"), the U.S. Supreme Court greatly reduced greatly reduced judicial deference to regulatory agencies, which could increase successful legal challenges to federal regulations affecting our operations. 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.

At the state level, individual states in the United States have also increasingly passed legislation and implemented 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 proposal to import certain drugs from Canada for specific state healthcare programs. It is unclear if and how this program will be implemented and whether it will be subject challenges in the United States or Canada. Other states have also submitted proposals that are pending review by the FDA. Any such approved importation plans, if implemented, may result in lower drug prices for products covered by those programs. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, results of operations, financial condition and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs.

We anticipate that these new laws will result in additional downward pressure on coverage and the price that we receive for any approved product, and could seriously harm our business. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our products. In addition, it is possible that there will be further legislation or regulation that could harm our business, financial condition, and results of operations.

The U.S. Foreign Corrupt Practices Act

The U.S. Foreign Corrupt Practices Act of 1977 ("FCPA"), prohibits any U.S. individual or business from paying, offering, or authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Europe / Rest of World Government Regulation

In addition to regulations in the United States, we are subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products. Whether or not we or our potential collaborators obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries.

Certain countries outside of the United States have a similar process that requires the submission of an application for a clinical trial authorization ("CTA"), much like the IND prior to the commencement of human clinical trials.

Clinical Trials in the EU

Similarly to the United States, the various phases of non-clinical and clinical research in the EU are subject to significant regulatory controls. In the EU, clinical trials are governed by the Clinical Trials Regulation (EU) No 536/2014 ("CTR"), which entered into application on January 31, 2022 repealing and replacing the former Clinical Trials Directive 2001/20 ("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 CTR, 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 ("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.

EU Review and approval process

In the EU, medicinal products can only be commercialized after a related marketing authorization has been granted. To obtain a marketing authorization 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 authorities of EU Member States (decentralized procedure, national procedure or mutual recognition procedure). A marketing authorization may be granted only to an applicant established in the EU.

The centralized procedure provides for the grant of a single marketing authorization by the European Commission that is valid throughout the EEA (which is comprised of the 27 EU Member States plus Norway, Iceland and Liechtenstein). Pursuant to Regulation (EC) No 726/2004, the centralized procedure is compulsory for specific products, including for (i) medicinal products derived from biotechnological processes, (ii) products designated as orphan medicinal products, (iii) advanced therapy medicinal products and (iv) 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 Medicinal Products for Human Use ("CHMP"), conducts the initial assessment of a product. The CHMP is also responsible for several post-authorization and maintenance activities, such as the assessment of modifications or extensions to an existing marketing authorization. The maximum timeframe for the evaluation of an MAA under the centralized procedure is 210 days, excluding clock stops when additional information or written or oral explanation is to be provided by the applicant in response to questions of the CHMP. Accelerated assessment may be granted by the CHMP in exceptional cases, when a medicinal product targeting an unmet medical need is expected to be of major interest from the point of view of public health and, in particular, from the viewpoint of therapeutic innovation. If the CHMP accepts a request for accelerated assessment, the time limit of 210 days will be reduced to 150 days (excluding clock stops). 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 marketing authorization 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 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 marketing authorization of a medicinal product by the competent authorities of other EU Member States. The holder of a national marketing authorization may submit an application to the competent authority of an EU Member State requesting that this authority recognize the marketing authorization delivered by the competent authority of another EU Member State.

A marketing authorization has, in principle, an initial validity of five years. The marketing authorization 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 marketing authorization was granted. To support the application, the marketing authorization 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 marketing authorization was granted, at least nine months before the marketing authorization 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 marketing authorization. Once subsequently definitively renewed, the marketing authorization 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 marketing authorization) or on the market of the authorizing EU Member State within three years after authorization ceases to be valid (the so-called sunset clause).

Innovative products that target an unmet medical need and are expected to be of major public health interest may be eligible for a number of expedited development and review programs, such as the Priority Medicines ("PRIME") scheme, which provides incentives similar to the breakthrough therapy designation in the U.S. PRIME is a voluntary scheme aimed at enhancing the EMA's support for the development of medicinal products that target unmet medical needs. Eligible products must target conditions for which there is an unmet medical need (there is no satisfactory method of diagnosis, prevention or treatment in the EU or, if there is, the new medicinal product will bring a major therapeutic advantage) and they must demonstrate the potential to address the unmet medical need by introducing new methods of therapy or improving existing ones. Benefits accrue to sponsors of product candidates with PRIME designation, including but not limited to, early and proactive regulatory dialogue with the EMA, frequent discussions on clinical trial designs and other development program elements, and potentially accelerated MAA assessment once a dossier has been submitted.

In the EU, a "conditional" marketing authorization may be granted in cases where all the required safety and efficacy data are not yet available. The European Commission may grant a conditional marketing authorization 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 marketing authorization 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 marketing authorization can be converted into a traditional marketing authorization. However, if the conditions are not fulfilled within the timeframe set by the EMA and approved by the European Commission, the marketing authorization will cease to be renewed.

A marketing authorization 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 marketing authorization, a marketing authorization 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 marketing authorization. However, unlike the conditional marketing authorization, an applicant for authorization in exceptional circumstances is not subsequently required to provide the missing data. Although the marketing authorization "under exceptional circumstances" is granted

definitively, the risk-benefit balance of the medicinal product is reviewed annually, and the marketing authorization will be withdrawn if the risk-benefit ratio is no longer favorable.

Pediatric Development in the EU

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 ("PIP") agreed with the EMA's Pediatric Committee ("PDCO"). The PIP sets out the timing and measures proposed to generate data to support a pediatric indication of the medicinal product for which marketing authorization 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 marketing authorization 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 ("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.

Data and Market Exclusivity

The EU provides opportunities for data and market exclusivity related to marketing authorizations. Upon receiving a marketing authorization, 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 marketing authorization 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 marketing authorization 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.

In the EU, there is a special regime for biosimilars, or biological medicinal products that are similar to a reference medicinal product but that do not meet the definition of a generic medicinal product. For such products, the results of appropriate preclinical or clinical trials must be provided in support of an application for marketing authorization. Guidelines from the EMA detail the type of quantity of supplementary data to be provided for different types of biological product.

Orphan Designation in the EU

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 5 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. A marketing authorization 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, a marketing authorization 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 in the EU

Where a marketing authorization is granted in relation to a medicinal product in the EU, the holder of the marketing authorization 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 marketing authorization 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 a marketing authorization 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 ("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 marketing authorization. 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 ("SmPC"), which may require approval by the competent national authorities in connection with a marketing authorization. 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.

Pricing, Coverage and Reimbursement in the EU

In the EU, pricing and reimbursement schemes vary widely from country to country. Some EU Member States may approve a specific price for a product, or they may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other EU Member States allow companies to fix their own prices for products but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. In addition, some EU Member States may require the completion of additional studies that compare the cost-effectiveness of a particular medicinal product candidate to currently available therapies. This Health Technology Assessment ("HTA") process is the procedure according to which the assessment of the public health impact, therapeutic impact and the economic and societal impact of use of a given medicinal product in the national healthcare systems of the individual country is conducted. The outcome of HTA regarding specific medicinal products will often influence the pricing and reimbursement status granted to these medicinal products by the competent authorities of individual EU Member States. On January 12, 2025, Regulation No 2021/2282 on Health Technology Assessment ("HTA Regulation") entered into application through a phased implementation. The HTA Regulation initially applies to new active substances for oncology and advanced therapy medicinal products. It will be expanded to orphan medicinal products in January 2028, and to all centrally authorized medicinal products as of 2030. Select high-risk medical devices also came into scope in 2026. The HTA Regulation is intended to boost cooperation among EU Member States in assessing health technologies, including

new medicinal products. The HTA Regulation establishes a framework for EU-level joint clinical assessments, increasing cooperation among Member States on clinical aspects of health technology evaluation. 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.

For other countries outside of the EU, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical trials are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

Human Capital Management

As of December 31, 2025, we employed 372 employees, of whom 369 are full time, consisting of clinical, research, operations, finance and business development personnel. Fifty-five of our employees hold Ph.D. or M.D. degrees. Further, 289 of our employees are located in the United States, and 76 in Europe and seven in Canada. As of December 31, 2025, none of our employees is subject to a collective bargaining agreement. We consider our relationship with our employees to be good.

We expect to continue to add employees in 2026, with a focus on clinical, research and development and commercialization activities. We continually evaluate the business need and opportunity to expand our team and balance in-house expertise and capacity with outsourced expertise and capacity. Currently, we outsource substantial clinical trial work to clinical research organizations and drug manufacturing to contract manufacturers.

We maintain a safety culture grounded on the premise of eliminating workplace incidents, risks and hazards. We have implemented and continue to enhance safety measures in all our facilities, including establishing clear and regular policies, safety protocols and updates to all employees.

We believe our success depends on our ability to attract, develop and retain key personnel. We invest in the growth and development of our employees through various training and development programs that build and strengthen employees' leadership and professional skills. We also have processes in place to conduct activities like performance management, succession and workforce planning in order to support our employees in their growth and development and ensure we provide learning opportunities.

To continually assess and improve our employee retention and engagement, we conduct an engagement survey on a regular basis, the results of which are discussed with our board of directors, at all-hands employee meetings and in individual functions. We take actions to address areas of employment concern and follow up routinely to share with employees what we are doing.

We strive toward having a diverse and engaged team of employees. To accomplish this, we have included questions in our engagement survey to measure employee perception of inclusive culture. In 2020, we established a Culture Team that now consists of two sub-teams, one internationally and one in the United States with over 15 employees across a representative cross-section of departments. Amongst other initiatives, our Culture Team engages in continual discussions across the various business functions to identify potential actions to address areas of improvement and is focused on building accountability across the organization.

Corporate Information

We were incorporated in Delaware in May 2018. Our principal executive offices are located at 989 East Hillsdale Boulevard, Suite 300, Foster City, California 94404, and our telephone number is (650) 667-4085. Our corporate website address is www.mirumpharma.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 Securities and Exchange Commission (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(a) 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. Information contained on or accessible through our website is not a part of this Annual Report, and the inclusion of our website address in this report is an inactive textual reference only. Our design logo, "Mirum," and our other registered and common law trade names, trademarks and service marks are the property of Mirum Pharmaceuticals, Inc.

Item 1A. Risk Factors.

An investment in shares of our common stock involves a high degree of risk. You should carefully consider the following risk factors, as well as the other information in this Annual Report on Form 10-K, before deciding whether to purchase, hold or sell shares of our common stock. The occurrence of any of the following risks could harm our business, financial condition, results of operations and/or growth prospects or cause our actual results to differ materially from those contained in forward-looking statements we have made in this Annual Report on Form 10-K and those we may make from time to time. You should consider all of the risk factors described when evaluating our business.

Risks Related to Commercialization of our Approved Medicines and Development of our Product Candidates

The success of our business depends, in part, on our ability to market and sell our approved medicines profitably.

The success of our business depends, in part, on our ability to commercialize our approved medicines profitably. Our successful commercialization of our approved medicines depends on a number of factors, including, among others, the following:

- our ability to grow and maintain our sales team in the U.S., Canada, and certain countries in Europe, as well as scale our distribution capabilities in these locations and others where our products are available;
- the availability of adequate reimbursement and a commercially viable sales price of our approved medicines;
- acceptance by physicians, payors and patients of the benefits, safety and efficacy of our approved medicines, including relative to alternative and competing treatments;
- a continued acceptable safety profile of our approved medicines;
- the effect of health care legislation and regulatory changes in the locations where our approved medicines are authorized;
- our ability to successfully obtain the substances and materials used in manufacturing our medicines from third parties and to have finished product manufactured by third parties in accordance with regulatory requirements and in sufficient quantities for our commercial needs;
- our ability to establish and enforce intellectual property rights in and to our approved medicines and avoid or successfully defend third-party patent interference or intellectual property infringement claims;
- our ability to compete successfully with the marketing and sale of compounded and generic versions of our medicines; and
- sufficient patient population that would benefit from our approved medicines as they are intended for use in rare diseases for which the patient population is small.

If one or more of the above factors is not present, many of which are beyond our control, in a timely manner or at all, we could experience significant delays or an inability to market and sell our approved medicines profitably, which would harm our business, financial condition, operating results and prospects.

If we are unable to adequately grow, maintain and scale our marketing and sales capabilities or enter into or maintain rights pursuant to agreements with third parties to market and sell our approved medicines, we may not be able to generate viable revenues.

To successfully commercialize our approved medicines, we must grow, maintain and appropriately scale our marketing, sales, distribution, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. We have established our own commercial capabilities in the U.S. to commercialize our approved medicines. We are also in the process of further establishing our capabilities related to Livmarli in certain major European markets and Canada and have entered into a limited number of partner and distributor agreements in other select geographies. We plan to continue to evaluate opportunities to partner with pharmaceutical companies that have established sales and marketing capabilities to commercialize our approved medicines and our product candidates, if approved, outside of these geographies. Our projections of the commercial and sales needs to target these markets may not be accurate. If we are materially off from our projections, our business and operating results would be harmed.

Growing and maintaining our own sales force to market Livmarli, Cholbam and Ctexli is expensive and time-consuming. Moreover, we may not be able to successfully or adequately develop this capability for our product candidates

in development. We compete with other pharmaceutical and biotechnology companies to recruit, hire, train and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our approved medicines, and any agreements with such third parties may not be on terms that are favorable to us. To the extent we do rely on third parties to commercialize our approved medicines and our other product candidates, if approved, we may have little or no control over the marketing and sales efforts of such third parties and our revenues from product sales may be lower than if we had commercialized our product candidates ourselves. In addition, we have entered into a limited number of partner and distributor agreements. Any loss, commercial failure, or termination of rights pursuant to these agreements could delay or hinder our commercialization efforts.

In the event we are unable to successfully grow and maintain our marketing and sales force or collaborate with necessary third-party marketing and sales organizations, we would not be able to commercialize our approved medicines and our business, results of operations, financial condition, and prospects would be materially adversely affected.

Our commercial success may be severely hindered if we are unable to obtain and/or maintain adequate coverage and reimbursement for our approved medicines and any future product candidates, if approved.

The availability of coverage and adequate reimbursement from private third-party payors such as pharmacy benefit managers and commercial insurers, and governmental healthcare programs, such as Medicaid in the U.S. and equivalent programs in foreign countries, is critical to the commercial success of our approved medicines in the U.S. and in international markets. Coverage may be adversely affected by a number of factors, including, but not limited to:

- increasing and intense pressure from political, social, competitive and other sources to reduce drug unit costs, access drugs from other countries to achieve better pricing or limit changes in list price;
- changes in federal, state or foreign government regulations or private third-party payors' reimbursement policies, including changes that may result from government administration changes;
- implementation of federal or state regulations;
- reimbursement decisions and price negotiations with foreign government payors;
- consolidation and increasing assertiveness of commercial payors seeking net price reduction via drug rebates and other forms of discounts linked to the placement of our approved medicines on their formularies; and
- the imposition of restrictions on access or coverage of particular drugs or pricing.

A trend in the healthcare industry is cost containment. Third-party payors are developing increasingly sophisticated methods of controlling healthcare costs by, among other methods, limiting or preventing (for example via prior authorization, prior therapy or step edit requirements) coverage for particular medications, requiring drug companies to provide them with varying levels of discounts from list prices and/or challenging the value of list prices charged for medical products. Similarly, the containment of healthcare costs has become a priority for federal, national, and state governments around the world. For example, the Inflation Reduction Act ("IRA") among other things, requires the U.S. Department of Health and Human Services ("HHS") to negotiate the price of certain single-source drugs that have been on the market for at least seven (7) years covered under Medicare as part of the Medicare Drug Price Negotiation Program. Each year up to twenty (20) products will be selected by HHS for the Medicare Drug Price Negotiation Program. Products subject to the Medicare Drug Price Negotiation Program are expected to experience a significant reduction in reimbursement from the Medicare program on a per unit basis. In addition, the IRA imposes rebates on many Medicare Part B and Medicare Part D products to penalize price increases that outpace inflation on an annual basis. If coverage and adequate reimbursement are not available, or are available only to limited levels, we may not be able to successfully commercialize our current and any future product candidates that we develop, which could have an adverse effect on our operating results and our overall financial condition. Coverage decisions may depend upon the size of a patient population, perceptions of clinical efficacy and economic standards that may disfavor new drug products when more established or lower cost therapeutic alternatives are already available or subsequently become available.

Coverage and reimbursement for drug products can differ significantly across payors. 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 approved medicines to each third-party payor separately, with no assurance that coverage will be obtained or maintained. Additionally, coverage policies and third-party reimbursement rates may change at any time. For example, rebate payments may increase, or prices be adjusted, under value-based purchasing arrangements based on evidence-based measures or outcomes-based measures for a patient or beneficiary based on use of our drug. Thus, even if favorable coverage and reimbursement status is attained for one or more drug products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

In many foreign countries, including EU Member States, the pricing of prescription drugs is subject to governmental control and the proposed pricing for a drug must be approved before it may be lawfully marketed. In such countries, pricing negotiations with governmental authorities can take considerable time after receipt of regulatory approval for a product and varies between countries. In addition, there can be considerable pressure from governments and other stakeholders on prices and reimbursement levels. For instance, governmental authorities in the EU Member States and third-party payors could base pricing and reimbursement terms on what they perceive to be comparable products, even if approved for different indications. In addition, EU Member States may restrict the range of medicines for which their national health insurance systems provide reimbursement and to control the prices of medicines for human use. An EU Member State, such as France and Germany, may approve a specific price for the medicine, 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 medicine on the market. For example, some countries, like France, may impose a total revenue cap on a product, limiting maximum sales potential at a certain threshold regardless of unit price. These pricing and reimbursement decisions may impact the pricing and reimbursement of our approved medicines in such jurisdictions. Many EU Member States also periodically review their reimbursement procedures for medicines, which could have an adverse impact on the reimbursement status of our approved medicines in the future. Moreover, political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations often continue after coverage and reimbursement have been obtained. Reference pricing or pricing comparisons to our competitors used by various countries and parallel distribution, or arbitrage between low-priced and high-priced countries, can further reduce prices. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries.

We expect that legislators, policymakers and healthcare insurance funds in the EU Member States will continue to propose and implement cost-containing measures, such as lower maximum prices, lower or lack of reimbursement coverage and incentives to use cheaper, usually generic, products as an alternative to branded products, and/or branded products available through parallel import to keep healthcare costs down. On January 12, 2025, Regulation No 2021/2282 on Health Technology Assessment, amending Directive 2011/24/EU (the "Regulation"), entered into application through a phased implementation. The Regulation initially applies to new active substances for oncology and advanced therapy medicinal products. It will be expanded to orphan medicinal products in January 2028 and to all centrally authorized medicinal products as of 2030. Select high-risk medical devices also came into scope in 2026. It is intended to boost cooperation among EU Member States in assessing health technologies, including new medicines. The Regulation establishes a framework for joint clinical assessments, increasing cooperation among EU Member States on clinical aspects of health technology evaluation. 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 are unable to maintain favorable pricing and reimbursement status in EU Member States for product candidates that we may successfully develop and for which we may obtain regulatory approval, any anticipated revenue from and growth prospects for those product candidates in the EU could be negatively affected.

Historically, products launched in the EU and other foreign countries do not follow the price structures of the U.S. and prices can be significantly lower and the time to obtain pricing and reimbursement approvals is significantly longer. If pricing is set at unsatisfactory levels or if reimbursement of our approved medicines and any future product candidates, if approved, is unavailable or limited in scope or amount, our revenues from sales by us or our partners and the potential profitability of our approved medicines or any future product candidates, if approved, in those countries would be negatively affected.

Our approved medicines or any one of our product candidates, if approved, may fail to achieve the market acceptance among physicians, patients and others in the medical community necessary for commercial success.

The commercial success of our approved medicines or any one of our product candidates, if approved, depends significantly on the market acceptance among physicians, patients, tertiary care centers, transplant centers and others in the medical community. The degree and rate of market acceptance depends on a number of factors, including, among other things:

- patient demand;
- the availability of adequate reimbursement from private third-party payors and government authorities;
- the willingness of patients to pay out-of-pocket in the absence of coverage and adequate reimbursement by third-party payors and government authorities;
- the cost of treatment in relation to alternative treatments and patients' willingness to pay for our approved medicines, including relative to discretionary items;

- our ability to successfully compete with available off-label therapies, future approved therapies, and therapies in development and available for use through expanded access programs;
- acceptance by physicians, patients, tertiary care centers, transplant centers and others in the medical community that our approved medicines are safe and effective treatments;
- physician and patient willingness to adopt a new therapy over other available therapies;
- limitations, warnings or adverse drug reactions contained in the labeling or product inserts approved by the FDA, European Commission or comparable foreign regulatory authorities, and patients' and physicians' assessment of these limitations and warnings;
- overcoming any biases physicians or patients may have toward particular therapies for the treatment of the indications our approved medicines are approved for (or, if applicable, deemed medically necessary for);
- patients and caregivers properly using our approved medicines as instructed;
- the prevalence and severity of side effects from the use or potential misuse of our approved medicines;
- relative convenience and ease of administration, including as compared to alternative treatments and competitive therapies, and patient satisfaction with the overall treatment experience;
- the ability of specialty pharmacies we contract with to process prescriptions and dispense our approved medicines and the processes required to place orders with those pharmacies;
- our ability to successfully internalize operation of our patient services hub from our third-party vendor;
- the ability of our patient services hub to provide adequate support for patients and physicians to prescribe and access our approved medicines;
- the timing of market introduction of any of our approved medicines as well as competitive products;
- the effectiveness of our sales, marketing and distribution efforts and those of the third parties with whom we contract;
- adverse publicity about our approved medicines or favorable publicity about competitive products;
- potential product liability claims;
- our ability to manage our growth and operations to effectively support our commercialization activities; and
- patient satisfaction leading to a high percentage of patients deriving clinical benefit and staying on our approved medicines chronically.

If any of our approved medicines fail to achieve the market acceptance among physicians, patients, tertiary care centers, transplant centers or others in the medical community necessary for commercial success, our operating results and financial condition will be adversely affected, which may delay, prevent or limit our ability to generate revenue and continue our business.

Our approved medicines and our product candidates may cause undesirable side effects or have other properties that could limit their commercial profile, expose us to product liability claims, delay or prevent regulatory approval of our product candidates or additional indications, or result in significant negative consequences following any additional marketing approval, any of which may adversely impact our business, financial condition, operating results and prospects.

As is the case with biopharmaceuticals generally, it is likely that there may be side effects and adverse events ("AEs") associated with use of our approved medicines and product candidates. Results of our clinical trials and expanded access program could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval of our product candidates by the FDA, European Commission or comparable foreign regulatory authorities. The drug-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. Any of these occurrences may harm our business, financial condition and prospects significantly.

In clinical trials of Livmarli in ALGS, the most commonly reported AEs were diarrhea, abdominal pain and vomiting, and were mostly mild to moderate in severity and transient in nature. Additionally, AEs reported in greater than 5% of patients included fat-soluble vitamin deficiency, nausea, liver transaminase increases, and bone fracture. The frequency of observed AEs has not increased over time. In the pivotal trial of Livmarli in PFIC, adverse reactions reported

in greater than 5% of patients and greater than placebo included diarrhea, abdominal pain, increased transaminases, hematochezia or rectal hemorrhage, and bone fractures. Prescribing information for Livmarli includes warnings and precautions related to monitoring for and the risk of hepatotoxicity, gastrointestinal adverse reactions, fat-soluble vitamin deficiency and risk of propylene glycol toxicity (pediatric patients less than 5 years of age). In clinical trials of volixibat, the most common AEs reported were mild to moderate GI events (diarrhea, abdominal pain, nausea and vomiting) observed in the volixibat groups. In the interim analysis of the PBC VANTAGE study, the incidence of diarrhea in patients on volixibat was 77% with all cases mild to moderate; one patient discontinued the study due to an AE of diarrhea. The most common adverse reactions for Cholbam (≥1%) are diarrhea, reflux esophagitis, malaise, jaundice, skin lesion, nausea, abdominal pain, intestinal polyp, urinary tract infection, and peripheral neuropathy. The most common (≥ 20%) AEs seen in patients on chenodiol in the RESTORE clinical trial included diarrhea, constipation and headache. The most common treatment emergent AEs seen in clinical trials of MRM-3379 include nausea and vomiting, all mild and moderate in severity.

In clinical studies of brelovitug, the most common treatment-related AEs were injection-related events that resolved within 1-2 days, and cases of flu-like illness that were mild-moderate in severity.

Additionally, in respect of our approved medicines or if one or more of our product candidates receives marketing approval, and we or others (including regulatory approval authorities) later identify undesirable side effects caused by our approved medicines or such product candidates or other products with the same or related active ingredients, a number of potentially significant negative consequences could result, including, among other things:

- regulatory authorities may withdraw, suspend, or vary approvals of such product, including the FDA, European Commission or comparable foreign regulatory authorities withdrawing approval for the affected medicine;
- regulatory authorities may require additional warnings on the label;
- regulatory authorities may require a recall or we or our potential partners may voluntarily recall such product;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients at significant cost or instate a Risk Evaluation and Mitigation Strategies ("REMS") or Risk Management Plan ("RMP");
- regulatory authorities may require the addition of warnings, such as black box or other warnings, or contraindications in the product labeling that could diminish the usage of the product or otherwise limit the commercial success of the affected product;
- our ability to promote our approved medicines may be limited and we could be required to change administration of, or modify, such product in some other way;
- regulatory authorities may require us to modify, suspend or terminate our clinical trials, conduct additional clinical trials or engage in costly post-marketing testing and surveillance to monitor the safety or efficacy of such product;
- undesirable side effects may limit physicians' or patients' willingness to initiate or continue therapy with such product;
- sales may decrease significantly;
- we could be sued and held liable for harm caused to patients; and
- our corporate brand and reputation or the reputation of our approved medicines may suffer.

Such events could prevent us from achieving or maintaining market acceptance of our approved medicines, and could significantly harm our business, results of operations and prospects.

If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs in the U.S., we could be subject to additional reimbursement requirements, fines, sanctions and exposure under other laws which could have a material adverse effect on our business, results of operations and financial condition.

We participate in, or are subject to, the Medicaid Drug Rebate Program, as administered by Centers for Medicare & Medicaid Services ("CMS"), and other federal and state government pricing programs in the U.S., and we may participate, or be asked to participate, in additional government pricing programs or supplemental rebates in the future. These programs generally require us to pay rebates or otherwise provide discounts to government payors in connection

with drugs that are dispensed to beneficiaries/recipients of these programs. In some cases, such as with the Medicaid Drug Rebate Program, the rebates are based on pricing that we report to the government agencies that administer the programs. Pricing requirements and rebate/discount calculations are complex, vary among products and programs, and are often subject to interpretation by governmental or regulatory agencies and the courts. The requirements of these programs, including, by way of example, their respective terms and scope, change frequently. For example, on March 11, 2021, the American Rescue Plan Act of 2021 was signed into law, which eliminated the statutory Medicaid drug rebate cap, previously set at 100% of a drug's average manufacturer's price ("AMP"), for single source and innovator multiple source drugs, effective January 1, 2024. Responding to current and future changes may increase our costs, and the complexity of compliance will be time consuming. Invoicing for rebates is provided in arrears, and there is frequently a time lag of up to several months between the sales to which rebate notices relate and our receipt of those notices, which further complicates our ability to accurately estimate and accrue for rebates related to the Medicaid program as implemented by individual states. Thus, there can be no assurance that we will be able to identify all factors that may cause our discount and rebate payment obligations to vary from period to period, and our actual results may differ significantly from our estimated allowances for discounts and rebates. Changes in estimates and assumptions may have a material adverse effect on our business, results of operations and financial condition. In addition, the HHS Office of Inspector General and other Congressional, enforcement and administrative bodies have recently increased their focus on pricing requirements for products, including, but not limited to the methodologies used by manufacturers to calculate AMP, and best price, for compliance with reporting requirements under the Medicaid Drug Rebate Program. Additionally, several states have a practice of asking, or are increasing activity in requesting supplemental rebates, for covered products. We are liable for errors associated with our submission of pricing data and for any overcharging of government payors. For example, failure to submit monthly/quarterly AMP and best price data on a timely basis could result in significant civil monetary penalties for each day the submission is late beyond the due date. Failure to make necessary disclosures and/or to identify overpayments could result in allegations against us under the civil False Claims Act and other laws and regulations. Any required refunds to the U.S. government or responding to a government investigation or enforcement action would be expensive and time consuming and could have a material adverse effect on our business, results of operations and financial condition. In addition, in the event that the CMS were to terminate our rebate agreement, no federal payments would be available under Medicaid or Medicare for our covered outpatient drugs.

We may face product liability exposure, and if claims are brought against us, we may incur substantial liability if our insurance coverage for those claims is inadequate.

We face an inherent risk of product liability suits for our approved medicines and product candidates. Our approved medicines and our product candidates are designed to affect important bodily functions and processes. Any side effects, manufacturing defects, failure to follow instructions, misuse or abuse associated with our approved medicines or our product candidates could result in injury to a patient or even death. In addition, a liability claim may be brought against us even if our approved medicines or our product candidates merely appear to have caused an injury. Product liability claims may be brought against us by, among others, consumers, their family members, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our approved medicines or product candidates. If we are the target of product liability claims, we will incur substantial legal costs, potential liabilities and could incur reputational harm if we do not successfully defend ourselves.

In addition, regardless of merit or eventual outcome, product liability claims may result in, among other things:

- the inability to commercialize our approved medicines or product candidates, if approved;
- decreased demand for our approved medicines or product candidates;
- termination of clinical trial sites or entire trial programs;
- product recall or withdrawal from the market or labeling, marketing or promotional restrictions;
- impairment of our business reputation and negative media attention;
- substantial costs of any related litigation or similar disputes;
- distraction of management's attention and other resources and employees from our primary business;
- substantial monetary awards to patients or other claimants against us that may not be covered by insurance; and
- loss of revenue.

Large judgments have been awarded in class action and individual lawsuits based on drugs that had anticipated or unanticipated side effects. Although we have obtained product liability insurance coverage, our insurance coverage may

not be sufficient to cover all of our product liability related expenses or losses and may not cover us for any expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive and, in the future, we may not be able to maintain insurance coverage at a reasonable cost, in sufficient amounts or upon adequate terms to protect us against losses due to product liability. A successful product liability claim or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could decrease our cash and could harm our business, financial condition, operating results and prospects.

If we are found to have improperly promoted off-label uses of our approved medicines, or unapproved uses of our product candidates, if approved, or if we are found to be the cause of physician misuse or off-label use of our approved medicines or our product candidates, if approved, we may become subject to prohibitions on the sale or marketing of such products, product liability claims and significant fines, penalties and sanctions, and our brand and reputation could be harmed.

The FDA, European Commission, Health Canada, competent authorities of individual EU Member States, and comparable foreign regulatory authorities strictly regulate the marketing and promotional claims that are made about drug and biologic products. In particular, a product may not be promoted for uses or indications that are not approved by the FDA, European Commission, Health Canada or comparable foreign regulatory authorities as reflected in the product's approved labeling and comparative safety or efficacy claims cannot be made without direct comparative clinical data. For example, although Livmarli may appeal to individuals who have not been diagnosed with cholestatic pruritus associated with ALGS or PFIC or suffer from other forms of cholestatic pruritus like those included in our Phase 3 EXPAND trial, we (and our collaborators, where applicable) are only able to promote Livmarli:

- in the U.S. for cholestatic pruritus associated with ALGS in patients three months of age and older and for cholestatic pruritus in PFIC patients twelve months of age and older;
- in the EU for the treatment of cholestatic pruritus in patients with ALGS two months of age and older and for the treatment of PFIC in patients three months of age and older; and
- in Japan for the treatment of cholestatic pruritus in patients with ALGS and PFIC.

Additionally, Ctexli is only indicated for adults with CTX, not for individuals under the age of 18, even though it may be prescribed by healthcare providers for that population. If we are found to have promoted off-label uses of our approved medicines or product candidates, we may receive warning or untitled letters and become subject to significant criminal and civil liability, which would materially harm our business. Further, in the U.S., both federal and state governments have levied large civil and criminal fines against companies for alleged improper off-label promotion and have enjoined several companies from engaging in off-label promotion and to undertake corrective remedies.

If we become the target of such an investigation or prosecution based on our marketing and promotional practices, we could face similar sanctions, which would materially harm our business. In addition, management's attention could be diverted from our business operations, significant legal expenses could be incurred and our brand and reputation could be damaged. In some instances, the FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed. If we are deemed by the FDA to have engaged in the promotion of off-label uses, we could be subject to FDA regulatory or enforcement actions as well as by other federal, state or foreign enforcement authorities that might take action if they consider our business activities to constitute promotion of an off-label use, which could result in significant penalties, including criminal, civil or administrative penalties, damages, fines, disgorgement, exclusion from participation in government healthcare programs and the curtailment or restructuring of our operations. For example, if such off-label promotion results in the submission of a reimbursement claim to a governmental healthcare program, we could be found liable under the U.S. False Claims Act. In cases where off-label promotion has resulted in violations of other statutes, the U.S. Department of Justice ("DOJ") has also required companies to enter into deferred prosecution agreements or corporate integrity agreements.

Our approved medicines and our product candidates are subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense. We may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with any product.

Any regulatory approvals that we receive may be subject to 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 post-market studies or clinical trials, and surveillance to monitor safety and effectiveness. The FDA may also require a REMS in order to approve a product candidate, which could entail requirements for a medication guide, physician communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. Similarly, the European Commission may require a RMP in order to collect additional information on a medicine's safety profile which may include plans for pharmacovigilance activities and

measures to minimize risks. In addition, if the FDA, European Commission or comparable foreign regulatory authorities approve a product candidate, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, post-marketing obligations, storage, advertising, promotion, import, export and recordkeeping for the approved product will be subject to extensive and ongoing regulatory requirements. For example, we are subject to ongoing FDA and European Commission obligations and continued regulatory review with respect to, among other things, the manufacturing, processing, labeling, packaging, distribution, AE reporting, storage, advertising, promotion and recordkeeping for Livmarli, which requirements include submissions of safety and other post-marketing information and reports and registration, as well as continued compliance with current good manufacturing practices ("cGMP") requirements and with the FDA's and equivalent foreign good clinical practice ("GCP").

In addition, Livmarli was the subject of a marketing authorization granted by the European Commission under exceptional circumstances in accordance with Article 14.8 of Regulation (EC) No 726/2004 relating to the authorization and supervision of medicines for human and veterinary use and establishing the EMA. This type of authorization is reviewed annually to reassess the risk-benefit balance of the medicine. The purpose of any specific procedures/obligations imposed as part of the marketing authorization granted in exceptional circumstances is to contribute to the provision of information on the safe and effective use of the product. Grant of a marketing authorization in exceptional circumstances is renewable for one-year periods and will normally not lead to the completion of a full dossier/approval.

We are subject to various FDA and EU post-marketing requirements across our approved medicines, including the conduct and submission of registry studies and the FDA's and EU's prohibition against marketing medicines in uses that are not approved. These and similar requirements could be imposed by the FDA, European Commission or comparable foreign regulatory authorities for any approved product.

In addition, manufacturers of drug and biologic products and their facilities are subject to continual review and periodic inspections by the FDA, the competent authorities of the individual EU Member States, or comparable foreign regulatory authorities for compliance with cGMP regulations. If we or a regulatory authority discovers previously unknown problems with a product, such as AEs of unanticipated severity or frequency, or problems with the facility where, or processes by which, the product is manufactured, such events may result in, among other things:

- restrictions on the marketing or manufacturing of the product, withdrawal of the product from the market, or voluntary or mandatory product recalls;
- fines, untitled letters, warning letters or holds on clinical trials;
- refusal by the FDA or European Commission to approve pending applications or supplements to approved applications filed by us or suspension or revocation of license approvals;
- product seizure or detention, or refusal to permit the import or export of a product; and
- injunctions or the imposition of civil or criminal penalties.

The occurrence of any event or penalty described above or any similar event or penalty may inhibit our ability to commercialize our approved medicines or our product candidates and generate revenue and could require us to expend significant time and resources in response and could generate negative publicity.

The FDA's, European Commission's and comparable foreign regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval for our product candidates or restrict marketing of any then-approved product. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would adversely affect our business, prospects, financial condition and results of operations.

We may pursue approval in the U.S., Canada or certain countries in Europe using accelerated, exceptional circumstances or conditional approval pathways, which typically require commitments to complete additional clinical trials. The additional clinical trials may not confirm the treatment effect, which may result in the loss of marketing authorization under accelerated approval, exceptional circumstances or conditional approval. For example, the 24-week endpoint for the AZURE-1 and AZURE-4 trials is meant to enable accelerated approval of brelovitug for the treatment of chronic HDV infection. We expect that if approved, confirmatory studies will be required for full approval. Such confirmatory studies may not show the same clinical benefit as the studies that lead to accelerated approval.

Our business depends, in part, on the success of our product candidates, each of which requires significant clinical testing before we can seek regulatory approval and potentially launch commercial sales.

Our business and future success depends, in part, on our ability to obtain regulatory approval for, and then successfully commercialize our product candidates. Our product candidates will require clinical development, regulatory review and approval in multiple jurisdictions, substantial investment, access to sufficient manufacturing capacity and significant marketing efforts before we can generate any revenue from product sales. Further, we are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA, European Commission or comparable foreign regulatory authorities, and we may never receive such regulatory approvals for our product candidates.

Our clinical trials may not be successful and may not be completed on time or at all, and the FDA, EMA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials. For example, in certain of our ongoing clinical trials, the primary efficacy endpoint is a patient-reported outcome or a caregiver-reported outcome measuring the decrease in severity of pruritus. The FDA, EMA or comparable foreign regulatory authorities may not accept such patient-reported outcomes or caregiver-reported outcomes as validated. We only recently acquired brelovitug, and all clinical development prior to the closing of the Bluejay Acquisition was conducted by other sponsors. If modifications are needed for our study design to support the submission of an application for marketing approval, incorporating such modifications may be costly and could lead to delays in obtaining approval from the FDA, European Commission or comparable foreign regulatory authorities, which may significantly, adversely and materially affect our ability to successfully commercialize our product candidates. Further, even if we make changes to the study design to address these considerations, the FDA, European Commission or comparable foreign regulatory authorities may not approve our product candidates.

Even if such regulatory authorities agree with the design and implementation of our clinical trials, such regulatory authorities may change their requirements in the future. In addition, even if the clinical trials are successfully completed, the FDA, EMA or comparable foreign regulatory authorities may not interpret the results as we do, and more clinical trials could be required before we submit our product candidates for approval.

To the extent that the results of our clinical trials are not satisfactory to the FDA, EMA or comparable foreign regulatory authorities for support of a marketing application, approval for our product candidates may be significantly delayed or prevented, or we may be required to expend significant additional resources, which may not be available to us, to conduct additional clinical trials in support of potential approval for our product candidates.

We have encountered and may continue to encounter delays and difficulties enrolling patients in our clinical trials, and as a result, our clinical development activities could be delayed or otherwise adversely affected.

Patient enrollment, a significant factor in the timing of clinical trials, is generally affected by many factors including, but not limited to, the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the clinical trial, the design of the clinical trial, competing clinical trials and clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating.

For example, each indication for which we are evaluating Livmarli, volixibat, and brelovitug is a rare liver disease with limited patient populations from which to draw participants in clinical trials. We will be required to identify and enroll a sufficient number of patients with the disease under investigation for each of our ongoing and planned clinical trials of Livmarli, volixibat, and brelovitug. Potential patients may not be adequately diagnosed or identified with the diseases which we are targeting or may not meet the entry criteria for our trials. In addition, patients may ultimately decide not to enroll in a particular clinical trial for reasons outside of our control. We may seek to conduct clinical trials in countries in which we have not previously conducted trials for our product candidates and in which we have not yet worked with the competent regulatory authorities. As a result, we could face patient recruitment issues in certain countries where such foreign regulatory authorities are not familiar with our product candidates. Additionally, other pharmaceutical companies targeting the same liver diseases are recruiting clinical trial patients from these patient populations, and have expanded access programs available, which have delayed enrollment in our clinical trials. Our inability to enroll a sufficient number of patients for any of our current or future clinical trials would result in significant delays. As a result, we may need to delay the completion of such trials beyond our expected timelines or abandon one or more clinical trials altogether.

Our clinical trials may fail to adequately demonstrate the safety and efficacy of our product candidates, which could prevent or delay regulatory approval and commercialization.

Before obtaining regulatory approvals for the commercial sale of a product candidate, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that a product candidate is both safe and effective for use in each target indication. Our clinical trials have in the past and could in the future fail to demonstrate safety and efficacy of the product candidate studied for the target indication. For example, in December 2023, we announced that our Phase 2b EMBARK clinical trial evaluating Livmarli in patients with biliary atresia ("BA") did not meet its primary or key secondary endpoints. For example, although brelovitug is being evaluated in the Phase 3 AZURE program for HDV, there can be no assurance that results will be positive or that we will be able to submit a BLA on the basis of these results. Most product candidates that commence clinical trials are never approved by regulatory authorities for commercialization. In the case of our product candidates, we are seeking to develop treatments for rare diseases for which there is limited clinical experience, and our planned clinical trials use novel end points and measurement methodologies, which add complexity to the conduct of and analysis of data from our clinical trials and may delay or prevent regulatory approval.

Clinical drug development involves a lengthy and expensive process with uncertain outcomes, and results of earlier studies and trials may not be predictive of future trial results.

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or safety profiles, notwithstanding promising results in earlier trials. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses. Further, if patients drop out of our clinical trials, miss scheduled doses or follow-up visits or otherwise fail to follow clinical trial protocols, or if our clinical trials are otherwise disrupted, the integrity of data from our clinical trials may be compromised or not accepted by the FDA, EMA or comparable foreign regulatory authorities, which would represent a significant setback for the applicable program. Additional safety data generated from our expanded access program and post-marketing studies could be different from, including less favorable than, the data generated and discussed with regulatory authorities to date. Our clinical trials may not be successful, and any safety concerns observed in any one of our clinical trials in our targeted indications could limit the prospects for regulatory approval of our product candidates in other indications.

Any delays in the commencement or completion, or termination or suspension, of our clinical trials could result in increased costs for us, delay or limit our ability to generate revenue and adversely affect our commercial prospects.

Before we can initiate clinical trials for our product candidates, we must submit the results of preclinical studies to the FDA, EMA or comparable foreign regulatory authorities along with other information, including information about product candidate chemistry, manufacturing and controls, and our proposed clinical trial protocol, as part of an IND application or similar regulatory filing. Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates in humans. Clinical testing is expensive, time consuming and uncertain as to outcome.

We do not know whether our planned clinical trials will begin on time or be completed on schedule, if at all. The commencement and completion of clinical trials can be delayed for a number of reasons, including delays related to:

- the FDA, EMA or comparable foreign regulatory authorities disagreeing as to the design or implementation of our clinical trials or agreement to commence our clinical trials;
- the FDA's, EU Member State competent authorities', or comparable foreign regulatory authorities' failure to accept our proposed manufacturing processes and suppliers and/or requirement to provide additional information regarding our manufacturing processes before providing marketing authorization;
- any failure or delay in reaching an agreement with CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;
- obtaining approval from one or more institutional review boards ("IRBs") or positive ethics committee opinions;
- IRBs or ethics committees refusing to approve or provide positive opinions, suspending or terminating the clinical trial at an investigational site, precluding enrollment of additional subjects, or withdrawing their approval of the clinical trial;

- changes to clinical trial protocol;
- selection of clinical end points that require prolonged periods of clinical observation or analysis of the resulting data;
- sites deviating from clinical trial protocol or dropping out of a clinical trial;
- manufacturing sufficient quantities of product candidate or obtaining sufficient quantities of combination therapies for use in clinical trials;
- subjects failing to enroll or remain in our trial at the rate we expect, or failing to return for post-treatment follow-up;
- subjects choosing an alternative treatment for the indication for which we are developing our product candidates, or participating in competing clinical trials;
- lack of adequate funding to continue the clinical trial;
- subjects experiencing severe or unexpected drug-related AEs;
- occurrence of serious adverse events ("SAEs") in clinical trials of the same class of agents conducted by other companies;
- a facility manufacturing our product candidates or any of their components being ordered by the FDA, EU Member State competent authorities, or comparable foreign regulatory authorities to temporarily or permanently shut down due to violations of cGMP, regulations or other applicable requirements, or infections or cross-contaminations of product candidates in the manufacturing process;
- any changes to our manufacturing process, suppliers or formulation that may be necessary or desired;
- the impact of geopolitical and macroeconomic developments on our ongoing and planned clinical trials; and
- failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA, EMA, competent authorities of individual EU Member States or comparable foreign regulatory authorities resulting in the imposition of a clinical hold, suspension or termination.

Further, conducting clinical trials in foreign countries presents additional risks that may delay completion of our clinical trials. These risks include the failure of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries.

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authorities may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA or comparable foreign regulatory authorities may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA, European Commission or comparable foreign regulatory authorities, as the case may be, and may ultimately lead to the denial of marketing approval of one or more of our product candidates.

If we experience delays in the completion of, or termination of, any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, and our ability to generate product revenue from any of these product candidates will be delayed. Moreover, any delays in completing our clinical trials will increase our costs, slow down our product candidate development and approval process and jeopardize our ability to commence product sales and generate revenue. In addition, many of the factors that cause, or lead to, termination or suspension of, or a delay in the commencement or completion of, clinical trials may also ultimately lead to the denial of regulatory approval of a product candidate. Any delays to our clinical trials that occur as a result could shorten any period during which we may have the exclusive right to commercialize our product candidates and our competitors may be able to bring products to market before we do, and the commercial viability of our product candidates could be significantly reduced. Any of these occurrences may harm our business, financial condition and prospects significantly.

Our product candidates are subject to extensive regulation and compliance, which is costly and time consuming, and such regulation may cause unanticipated delays or prevent the receipt of the required approvals to commercialize our product candidates.

The clinical development, manufacturing, labeling, storage, record-keeping, advertising, promotion, import, export, marketing and distribution of our product candidates are subject to extensive regulation by the FDA in the U.S., the EU and EU Member State competent authorities and by comparable foreign regulatory authorities in other markets. In the U.S., the EU and many foreign countries, we are not permitted to market our product candidates until we receive regulatory approval from the FDA, European Commission or comparable foreign regulatory authorities. The process of obtaining regulatory approval is expensive, often takes many years following the commencement of clinical trials and can vary substantially based upon the type, complexity and novelty of the product candidates involved, as well as the target indications and patient population. Approval policies or regulations may change, and regulatory authorities have substantial discretion in the drug approval process, including the ability to delay, limit or deny approval of a product candidate for many reasons. Despite the time and expense invested in clinical development of product candidates, regulatory approval is never guaranteed.

Prior to obtaining approval to commercialize a product candidate in the U.S. or internationally, we must demonstrate with substantial evidence from adequate and well-controlled clinical trials, and to the satisfaction of the FDA, EMA or comparable foreign regulatory authorities, that such product candidates are safe and effective for their intended uses. Results from non-clinical studies and clinical trials can be interpreted in different ways. Even if we believe the non-clinical or clinical data for our product candidates are promising, such data may not be sufficient to support approval by the FDA, European Commission or comparable foreign regulatory authorities. The FDA, EMA or comparable foreign regulatory authorities, as the case may be, may also require us to conduct additional preclinical studies or clinical trials for our product candidates either prior to or post-approval, or may object to elements of our clinical development program. If we were required to conduct such additional preclinical studies or clinical trials, the FDA, EMA or comparable foreign regulatory authorities may not agree with our interpretation of the results and we may not receive approval for our product candidates or additional indications, or marketing of our approved medicines may be subject to additional requirements.

Our product candidates could fail to receive regulatory approval for many reasons, including the following:

- the FDA, EMA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials or the validation of our caregiver and patient reported outcome instruments;
- serious and unexpected drug-related side effects may be experienced by participants in our clinical trials or by individuals using drugs similar to our product candidates;
- the population studied in the clinical trial may not be sufficiently broad or representative to assure safety in the full population for which we seek approval;
- the FDA, EMA or comparable foreign regulatory authorities may not accept clinical data from trials which are conducted at clinical facilities or in countries where the standard of care is potentially different from that of the U.S., the EU or the applicable foreign jurisdiction;
- we may be unable to demonstrate to the satisfaction of the FDA, EMA or comparable foreign regulatory authorities that a product candidate is safe and effective for any of its proposed indications;
- the results of clinical trials may not meet the level of statistical significance required by the FDA, EMA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA, EMA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to satisfy the FDA, EMA or comparable foreign regulatory authorities to support the submission of an NDA or other comparable submissions in the EU or other foreign jurisdictions or to obtain regulatory approval in the U.S. or elsewhere;
- approval or orphan status may be blocked or rejected by the FDA or the European Commission;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and

- the approval policies or regulations of the FDA, European Commission or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

Any of the above events could prevent us from achieving market approval of our product candidates and could substantially increase the costs of commercializing our product candidates. The demand for our product candidates could also be negatively impacted by any adverse effects of a competitor's product or treatment.

Of the large number of drugs in development, only a small percentage successfully complete the FDA, European Commission or comparable foreign regulatory approval processes and are commercialized. The lengthy approval process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market our product candidates, which would significantly harm our business, financial condition, results of operations and prospects.

Even if we eventually complete clinical trials and receive approval of an NDA or equivalent EU or foreign marketing application for our product candidates, the FDA, European Commission or comparable foreign regulatory authorities may grant approval contingent on the performance of costly additional clinical trials, including Phase 4 clinical trials, and/or the implementation of a REMS in the U.S. or RMP in the EU, which may be required to ensure safe use of the drug after approval. The FDA, European Commission or comparable foreign regulatory authorities also may approve a product candidate for a more limited indication or patient population than we originally requested, and the FDA, European Commission or comparable foreign regulatory authorities may not approve the labeling that we believe is necessary or desirable for the successful commercialization of a product. Any delay in obtaining, or inability to obtain, applicable regulatory approval would delay or prevent commercialization of that product candidate and would materially adversely impact our business and prospects.

If we fail to develop and commercialize additional product candidates, we may be unable to grow our business. Further, we may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

We plan to acquire rights to develop and commercialize product candidates in addition to our current approved medicines and our current product candidates. If we decide to pursue the development and commercialization of any additional product candidates, we may be required to invest significant resources to acquire or in-license the rights to such product candidates or to conduct drug discovery activities. We currently have limited drug discovery personnel or expertise, which may be inadequate to discover and develop an additional product candidate on our own. Any other product candidates will require additional, time-consuming development efforts, and significant financial resources, prior to commercial sale, including preclinical studies, extensive clinical trials and approval by the FDA, European Commission or comparable foreign regulatory authorities. All product candidates are prone to the risks of failure that are inherent in pharmaceutical product development, including the possibility that the product candidate will not be shown to be sufficiently safe and/or effective for approval by regulatory authorities. Because we have limited financial and personnel resources, we focus on specific product candidates for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or other indications that later prove to have greater commercial potential. We may focus our efforts and resources on product candidates that ultimately prove to be unsuccessful.

In addition, we may not be able to acquire, discover or develop any additional product candidates, and any additional product candidates we may develop may not be approved, manufactured or produced economically, successfully commercialized or widely accepted in the marketplace or be more effective than other commercially available alternatives. Research programs to identify and efforts to acquire new product candidates require substantial technical, financial and human resources whether or not we ultimately identify or acquire any candidates. If we are unable to acquire, develop or commercialize any other product candidates on favorable terms or at all, our business and prospects will suffer.

If generic manufacturers are successful in their use of litigation or regulatory means to obtain approval for generic versions of LIVMARLI, our revenue and results of operations would be adversely affected.

The Drug Price Competition and Patent Term Restoration Act of 1984, known as the Hatch-Waxman Amendments of the Federal Food, Drug, and Cosmetic Act (the "Hatch-Waxman Act"), permits the FDA to approve abbreviated new drug applications ("ANDAs") for generic versions of branded drugs. We refer to this process as the ANDA process. The ANDA process permits competitor companies to obtain marketing approval for a drug with the same active ingredient as a branded drug, but does not generally require the conduct and submission of clinical efficacy studies for the generic product. In place of such clinical studies, an ANDA applicant usually needs only to submit data demonstrating that its product is bioequivalent to the branded product.

Pursuant to the Hatch-Waxman Act, companies were permitted to file ANDA applications for proposed generic versions of LIVMARLI on or after September 29, 2025. We own, license or have acquired several patents that cover

LIVMARLI, and we have listed those patents in conjunction with that product in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the "Orange Book"). The Hatch-Waxman Act requires an ANDA applicant seeking FDA approval of its proposed generic product prior to the expiration of our Orange Book-listed patents to certify to the FDA, for all patents listed in the Orange Book for LIVMARLI, that (a) the applicant is not seeking approval until after the expiration date of the patent, or (b) the applicant believes the patent is invalid 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 us of such certification (a paragraph IV notice). For a method-of-treatment patent listed in the Orange Book, the applicant may also choose to submit a section viii statement certifying that its proposed product label does not contain any language regarding the patented method-of-use instead of certifying to the patent. Upon receipt of a paragraph IV notice, the Hatch-Waxman Act allows us, 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 our patents expire. If we commence a lawsuit within 45 days from receipt of the paragraph IV notice, the FDA cannot finally approve the generic's ANDA until the earlier of 30 months (known as the "30-month stay"), the expiration of the patent, settlement of the lawsuit, or a decision in the litigation resolving it in favor of the ANDA applicant. The discovery, trial and appeals process in such a lawsuit is costly, time consuming, and may result in generic competition if the ANDA applicant prevails.

On December 19, 2025, we, along with Satiogen Pharmaceuticals, Inc. and Shire Human Genetic Therapies, Inc. as co-plaintiffs, filed four complaints against Sandoz Inc. ("Sandoz"); Annora Pharma Private Limited, Hetero Labs Limited, and Hetero USA Inc. (together, "Hetero"); Zenara Pharma Private Limited and Biophore India Pharmaceuticals Private Limited (together, "Biophore"); and Zydus Lifesciences Global FZE, Zydus Lifesciences Limited, and Zydus Pharmaceuticals (USA) Inc. (together, "Zydus") (all collectively, "Defendants") in the U.S. District Court for the District of Delaware (the "LIVMARLI Patent Litigations"), alleging infringement of certain Orange Book listed patents covering LIVMARLI (the "LIVMARLI Patents"). The LIVMARLI Patent Litigations were initiated, in accordance with the procedures set out in the Hatch-Waxman Act, following the submission by Defendants of ANDAs directed to generic versions of LIVMARLI. Defendants' ANDAs seek approval to market generic versions of LIVMARLI prior to the expiration of one or more of the LIVMARLI Patents and allege that one or more of the LIVMARLI Patents are invalid, unenforceable, and/or not infringed. We are seeking, among other relief, an order that the effective date of any FDA approval of Defendants' ANDAs be no earlier than the expiration of the asserted patents, and such further and other relief as the court may deem appropriate. Based on our initiating the litigation, a 30-month stay is in place through March 29, 2029, preventing Defendants from marketing generic versions of LIVMARLI® during that time, subject to the exceptions noted above. On February 20, 2026, Sandoz asserted counterclaims against us and our co-plaintiffs seeking declaratory judgments of non-infringement and invalidity with respect to certain LIVMARLI Patents. Trial in the LIVMARLI Patent Litigations has not yet been scheduled. We cannot make any predictions about the final outcome of these matters or the timing thereof.

Any approved ANDA or related legal proceeding could have an adverse impact on our stock price. Litigation both by us to enforce our patents and by others to invalidate our patents has, and is likely to continue to, cost a substantial amount and require significant management attention. If the validity and/or enforceability of our patents covering LIVMARLI are not upheld in litigation, or if any ANDA filer we bring suit against is found to not infringe our asserted patents, the resulting generic competition following the expiration of regulatory exclusivity would have a material adverse effect on our revenue and results of operations.

We may also face generic competition for LIVMARLI in certain non-U.S. countries, and there is a process somewhat similar to the ANDA process under Article 10 of Directive 2001/83/EC in the EU. Our ability to successfully market and sell LIVMARLI in many countries in which we operate is based upon patent rights or certain regulatory forms of exclusivity, or both. The scope of our patent rights and regulatory exclusivity for LIVMARLI vary from country to country and are dependent on the availability of meaningful legal remedies in each country. If our patent rights and regulatory exclusivity for LIVMARLI are successfully challenged, expire, or otherwise terminate in a particular country, the resulting generic competition could have a material adverse effect on our revenue and results of operations.

Risks Related to Our Business and Industry

We have incurred substantial net losses since our inception and anticipate that we will continue to incur losses for the foreseeable future.

Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effectiveness in the targeted indication or an acceptable safety profile, gain regulatory approval and become commercially viable. While we have three medicines approved for commercial sale, we continue to incur significant research and development and other expenses related to our ongoing operations. As a result, we are not profitable and have incurred

substantial net losses since our inception in May 2018. For the years ended December 31, 2025, 2024 and 2023, we reported a net loss of $23.4 million, $87.9 million and $163.4 million, respectively. As of December 31, 2025, we had an accumulated deficit of $667.5 million.

While we generated net income in the third quarter of 2025, we expect to continue to incur net losses for the foreseeable future as we look to acquire products and product candidates, continue our clinical development of, and seek regulatory approvals for, our product candidates and as we continue commercializing our approved medicines in the U.S., Canada and in certain countries in Europe. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. Our prior net losses and expected future net losses have had and will continue to have an adverse effect on our stockholders' equity and working capital. Because of the numerous risks and uncertainties associated with drug development, we are unable to accurately predict the timing or amount of increased expenses, or when, if at all, we will be able to achieve profitability.

If the market opportunities for our product candidates are smaller than we believe they are, our future revenue may be adversely affected, and our business may suffer.

If the size of the market opportunities in each of our target indications or for any assets or product candidates that we may acquire, such as brelovitug and the Bile Acid Medicines, is smaller than we anticipate, we may not be able to achieve profitability and growth. We focus the clinical development and commercialization of our approved medicines on rare diseases with relatively small patient populations. Given the small number of patients who have the diseases that we are targeting, it is critical to our ability to grow and become profitable that we continue to successfully identify patients with these rare diseases. In addition, our estimates of the patient populations for our target indications have been derived from a variety of sources, including scientific literature, surveys of clinics, patient foundations, and market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. The effort to identify patients with diseases we seek to treat is in early stages, and we cannot accurately predict the number of patients for whom treatment might be possible. Additionally, the potentially addressable patient population for each of our product candidates may be limited or may not be amenable to treatment with our product candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business. Lastly, the potentially addressable patient population for any of our potential indications may even be further reduced if gene therapy products become more widely accepted and approved.

Obtaining and maintaining regulatory approval for a product candidate in one jurisdiction does not mean that we will be successful in obtaining regulatory approval for that product candidate in other jurisdictions.

Obtaining and maintaining regulatory approval for a product candidate in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction, while a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. For example, even if the FDA grants marketing approval for a product candidate, comparable foreign regulatory authorities in foreign jurisdictions must also approve the manufacturing, marketing and promotion of the product candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the U.S., including additional preclinical studies or clinical trials as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the U.S., a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our product candidates is also subject to approval.

Regulatory authorities in jurisdictions outside of the U.S. and the EU have requirements for approval for product candidates with which we must comply prior to marketing in those jurisdictions, and the regulatory approval process outside the U.S. and EU generally includes all of the risks associated with obtaining FDA and European Commission approval. Consequently, approval by the FDA does not ensure approval by the European Commission, approval by the European Commission does not assure approval by the FDA, and approval of either or both of the FDA and European Commission does not assure approval by regulatory authorities in other countries or jurisdictions. Further, marketing approvals in countries outside the U.S., including in the EU, do not ensure pricing approvals in those countries or in any other countries, and marketing approvals and pricing approvals do not ensure that reimbursement will be obtained. If we fail to comply with the regulatory requirements in international markets and/or receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of any of our approved medicines or product candidates, if approved, will be harmed, which would adversely affect our business, prospects, financial condition and results of operations.

Disruptions at the FDA, EMA and other foreign regulatory authorities caused by layoffs, funding shortages or global health concerns could negatively impact our business.

The ability of the FDA, the competent authorities of EU Member States and other foreign regulatory authorities to review and approve proposed clinical trials or new product candidates can be affected by a variety of factors, including, but not limited to, government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, statutory, regulatory, and policy changes, and other events that may otherwise affect these regulatory agencies' ability to perform routine functions. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, including executive and congressional priorities, the impacts of which are inherently fluid and unpredictable.

Disruptions at the FDA, European Commission, EMA and other foreign regulatory authorities may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary regulatory authorities, 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, have had to furlough critical FDA employees and stop critical activities to the extent they are not funded by existing available user fees. Repeated or prolonged government shutdowns or global health concerns could prevent the FDA, EMA or comparable foreign regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities and could significantly impact the ability of the FDA, European Commission, EMA or comparable foreign regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business. In addition, the current U.S. administration has enacted substantial reductions in force at various U.S. government agencies that, if applied to the FDA in a material way, could significantly reduce the FDA's capacity to perform its functions in a manner consistent with its past practices and could negatively impact our business.

Recently enacted legislation, future legislation and healthcare reform measures may increase the difficulty and cost for us to obtain marketing approval for and commercialize our product candidates and may affect the prices we may set.

In the U.S., certain European countries, and some other foreign jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes to the healthcare system, including cost-containment measures that may reduce or limit coverage and reimbursement for newly approved drugs and affect our ability to profitably sell any product candidates for which we obtain marketing approval. For example, Germany introduced changes to its laws governing reimbursement of medicines that impact, among others, orphan designation medicines. Previously, orphan designation drugs were presumed to provide an additional benefit upon obtaining a marketing authorization and, therefore, subject to a "limited assessment" by the German authority competent for reimbursement (G-BA) as long as the sales of the orphan designation medicine remained under the threshold of € 50 million in 12 months. Above this threshold, an orphan designation medicine would be subject to a full assessment by the G-BA regarding its benefits compared to an appropriate comparator therapy, just like any other medicine. This threshold will be lowered to € 30 million in the future, and if we exceed this threshold, we will need to undergo a full assessment in accordance with the German laws governing reimbursement, which may impact the reimbursement of our approved medicines. Other countries may adopt similar or new approaches that may impact the reimbursement of our product(s), increase our operating costs and impact profitability. Additionally, if adopted in the form proposed, the recent European Commission proposals to revise the existing EU laws governing authorization of medicines may result in a decrease in data and market exclusivity for our product candidates in the EU.

There have also been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare. For example, in March 2010, the Patient Protection and Affordable Care Act ("Affordable Care Act") was enacted in the U.S., which substantially changed the way healthcare is financed by both governmental and private insurers.

There have been amendments and executive, judicial and Congressional challenges to certain aspects of the Affordable Care Act. For example, on July 4, 2025, the One Big Beautiful Bill Act (the "OBBBA") was signed into law, which narrowed access to Affordable Care Act marketplace exchange enrollment and declined to extend the Affordable Care Act enhanced advanced premium tax credits that expired at the end of 2025, which, among other provisions in the law, are anticipated to reduce the number of Americans with health insurance. The OBBBA also is expected to reduce Medicaid spending and enrollment by implementing work requirements for some beneficiaries, capping state-directed payments, reducing federal funding, and limiting provider taxes used to fund the program. Congress is considering proposed legislation intended to further reduce healthcare costs with alternatives to replace the expired Affordable Care Act subsidies. Any of these measures could reduce the number of insured individuals in the U.S. and thereby reduce access to reimbursement for our commercial medicines.

Further, there has been heightened governmental scrutiny in the U.S. of pharmaceutical pricing practices in light of the rising cost of prescription drugs. Such scrutiny has resulted in several recent Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products.

The current administration is pursuing policies to reduce regulations and expenditures across government agencies including at HHS, the FDA, 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. For example, the current administration has announced agreements with pharmaceutical companies that require the drug manufacturers to offer, through a direct to consumer platform, U.S. patients and Medicaid programs prescription drug Most-Favored Nation pricing equal to or lower than those paid in other developed nations, with additional mandates for direct-to-patient discounts and repatriation of foreign revenues. Other recent actions, for example, include (1) directing agencies to reduce agency workforce and cut programs; (2) directing HHS and other agencies to lower prescription drug costs through a variety of initiatives, including by improving upon the Medicare Drug Price Negotiation Program and establishing Most-Favored-Nation pricing for pharmaceutical products; (3) imposing tariffs on imported pharmaceutical products; and (4) as part of the Make America Healthy Again Commission's Strategy Report released in September 2025, working across government agencies to increase enforcement on direct-to-consumer pharmaceutical advertising. Additionally, the current administration recently called on Congress to enact "The Great Healthcare Plan,"to codify and expand Most-Favored Nation pricing, lower government subsidies to private insurance companies, increase healthcare price transparency, expand pharmaceutical drugs available for over-the-counter purchase, and enact restrictions on pharmacy benefit manager payment methodologies, among other things. These actions and policies may significantly reduce U.S. drug prices, potentially impacting manufacturers' global pricing strategies and profitability, while increasing their operational costs and compliance risks. 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. Additionally, in its June 2024 decision in Loper Bright Enterprises v. Raimondo ("Loper Bright"), the U.S. Supreme Court greatly reduced judicial deference to regulatory agencies, which could increase successful legal challenges to federal regulations affecting our operations. We expect that additional U.S. federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that the U.S. federal government will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures. It is unclear how any such additional health care reform measures will impact our business.

At the state level, individual states in the U.S. have also increasingly passed legislation and implemented 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. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, results of operations, financial condition and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for our approved medicines and our other product candidates, if approved, or put pressure on our product pricing, which could negatively affect our business, results of operations, financial condition and prospects.

On December 11, 2025, the European Commission, the Parliament and the European Council reached a political agreement on a comprehensive overhaul of EU pharmaceutical legislation (the "Pharma Package"). The reform has been under negotiation since the European Commission submitted its proposal in April 2023. This package, comprised of a new directive and regulation to replace existing legislation, aims to modernize the EU framework. The political agreement is still subject to formal approval by the European Parliament and Council. If approved in the form proposed, the Pharma Package will, among other changes, reduce the baseline market protection period by one year, with limited opportunities for extensions, capped at a maximum of eleven years; reshape the incentives regime for orphan medicinal products, by introducing "breakthrough" orphan medicinal products (those addressing diseases with no available medicinal treatment) which will benefit from 11 years of market exclusivity; and expand the "Bolar" exemption to permit generic and biosimilar manufacturers to conduct preparatory activities for regulatory submissions, including pricing and reimbursement, and participate in procurement tenders while patent protection remains in force. A decrease in market exclusivity opportunities for our product candidates in the EU, combined with the expanded Bolar exemption, could open them to generic or biosimilar competition earlier than under the current regime, potentially impacting reimbursement status and the commercial prospects of our product candidates.

A variety of risks associated with marketing our product candidates internationally could materially adversely affect our business.

We already have and plan to seek further regulatory approval for our product candidates internationally and, accordingly, we are subject to additional risks related to operating in foreign countries if and when we obtain the necessary approvals, including:

- differing regulatory requirements in foreign countries, including differing reimbursement, pricing and insurance regimes;
- the potential for regulatory approvals in other countries to result in re-examination of previously approved regulatory submissions in other countries;
- the potential for so-called parallel importing, which is what happens when a local seller, either with government approval or faced with high or higher local prices, opts to import goods from a foreign market (with low or lower prices) rather than buying them locally;
- changes in tariffs (including tariffs imposed by the U.S. and retaliatory tariffs, if any, imposed by U.S. trading partners), trade barriers, price and exchange controls and other regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets, including as a result of high interest rates and ongoing military conflicts, as well as any related political or economic responses and counter-responses or otherwise by various global actors;
- compliance with tax, employment, immigration and labor laws for employees living or traveling internationally;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the U.S.;
- potential liability under the FCPA or comparable foreign regulations;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the U.S.;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities internationally; and
- business interruptions resulting from geo-political actions, including war and terrorism.

In addition, some countries, such as Brazil, Israel and Chile, require that clinical trial participants receive the product at no cost even after the clinical trial has ended. We would not be able to recover any profit for these patients and depending on the number of patients, duration of the treatment and numerous other factors, such regulations could harm our business, prospects, financial condition and results of operations significantly. These and other risks associated with our international operations may materially adversely affect our ability to attain or maintain profitable operations.

We face significant competition from other biotechnology and pharmaceutical companies with products that may directly or indirectly compete with ours, and our operating results will suffer if we fail to compete effectively.

The biotechnology and pharmaceutical industries are characterized by intense competition and rapid innovation. Our potential competitors include major multinational pharmaceutical companies, established biotechnology companies, specialty pharmaceutical companies, generic pharmaceutical companies and universities and other research institutions who are active in rare disease. Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations and well-established sales forces. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors may succeed in developing, acquiring or licensing, on an exclusive basis, drug products that are more effective or less costly than our product candidates, which may negatively affect our commercial opportunities. We believe the key competitive factors that will affect the development and commercial success of our product candidates are efficacy, safety and tolerability profile, reliability, convenience of dosing, price and reimbursement. There may also be

competitors for our product candidates that we are unaware of at this point. Additionally, while our product candidates and approved medicines are generally protected for a defined period per jurisdiction by various patents, the loss of patent or regulatory exclusivity for our pharmaceutical products may open such products to competition from generic substitutes that are typically priced significantly lower than the original products. The introduction of generic versions of a pharmaceutical product frequently leads to a swift and substantial decline in the sales of the original product. Our continued innovation efforts cannot fully mitigate the impact of competition from generics.

We are aware of two other companies pursuing clinical development and commercialization of therapies that reduce sBA levels via the IBAT pathway. GSK and Ipsen have IBATs in clinical development for cholestatic liver diseases.

We are aware Ipsen has received approval for odevixibat (Bylvay) for the treatment of pruritus in patients with PFIC and cholestatic pruritus in patients with ALGS in the U.S., and for the treatment of PFIC in the EU and for odevixibat (Kayfanda) for the treatment of cholestatic pruritus in ALGS. In the EU, Bylvay and Kayfanda are authorized under exceptional circumstances. Ipsen has opened enrollment in their ALGS open-label extension study to infants 11 months or younger and is also conducting a study of odevixibat in biliary atresia and plans to pursue other cholestatic liver diseases. GSK announced in November 2024 that the Phase 3 GLISTEN trial with linerixibat in PBC met its primary pruritus endpoint and that GSK has submitted marketing applications to the U.S., EU and other health authorities with potential approvals expected in 2026.

Other off-label medications are also used in ALGS, PFIC, PSC and PBC for cholestatic pruritus such as Ursodeoxycholic acid (“UDCA”), cholestyramine and other bile salt resins, rifampin, naltrexone and other agents, such as selective serotonin reuptake inhibitors. Further, we may compete with companies that are developing gene therapy for the treatment of PFIC. Additionally, surgical interventions, such as partial external biliary diversion and nasobiliary drainage, and extracorporeal liver support, such as Molecular Adsorbent Recirculation System, are also employed in an attempt to lower bile acid levels, manage pruritus and improve measures of liver function.

In adult settings of cholestasis, similar to pediatric settings, cholestyramine, UDCA, rifampin and naltrexone are commonly used agents. We are aware that Alfasigma S.p.’s (formerly Intercept Pharmaceuticals, Inc.) Ocaliva, Gilead Science’s Livdelzi, and Ipsen’s Iqirvo are approved as a second-line treatment for PBC in patients with inadequate response to ursodeoxycholic acid. We are aware of several agents in clinical development for the treatment of PBC including Alfasigma’s Ocaliva and bezafibrate, Zydus Therapeutics Inc.’s saroglitazar magnesium, Calliditas Therapeutics AB’s setanaxib, COUR Pharmaceuticals’ CNP-104, Umecrine Cognition’s golexanolone, Kowa Company Ltd’s K-808, HighTide Therapeutics Inc.’s HTD-1801, Hepagene Therapeutics Inc.’s HPG-1860, Tharimmune Inc.’s TH-104, Cascade Pharmaceuticals Inc.’s CS-0159, and GSK’s linerixibat, another IBATi.

We are not aware of FDA or European Commission approved therapeutics for the treatment of PSC. We are aware of several agents in clinical development for the treatment of PSC, including Dr. Falk Pharma's Norucholic acid, HighTide Therapeutics Inc.’s HTD-1801, Alfasigma’s Ocaliva, or obeticholic acid, Ipsen’s elafibranor and ritivixibat, NGM Biopharmaceuticals Inc.’s NGM282, Chemomab Therapeutics Ltd.’s CM-101, Cascade Pharmaceuticals Inc.’s CS-0159, LISCure Biosciences Inc.’s LB-P8, Halo Biosciences Inc.’s HB-1614, ProQR Therapeutics N.V.’s AX-0810, Rectify Pharmaceuticals, Inc.’s RTY-694 and Pliant Therapeutics’ bexotegrast.

There are other approved chenodeoxycholic acid products available outside of the U.S. Both Dr. Falk Pharma GmbH and Leadiant Biosciences, Inc. have FDA Orphan Drug Designations granted for the treatment of CTX (granted in 2004 and 2007, respectively), and we believe that Leadiant Biosciences, based on publicly available information, may be conducting a clinical study in CTX. There are currently no FDA-approved treatments in the U.S. that compete with Cholbam. There are other approved cholic acid products available outside of the U.S. and Laboratories CTRS has received approval from the EMA for a version of cholic acid.

There are currently no approved products available in the U.S. for the treatment of chronic HDV infection. Bulevirtide, which is commercialized by Gilead Sciences, is approved for commercial sale in the EU and Gilead is seeking approval from the FDA to commercialize bulevirtide in the U.S. In addition, we are aware that Vir Biotechnology is developing tobevibart in combination with elebsiran for the treatment of chronic HDV. We are aware of several other agents in clinical development for the treatment of chronic HDV including Gilead Science’s GS-4321, Shanghai HEP Pharmaceutical Co., Ltd.’s Hepalatide, Suzhou Ribo Life Science Co., Ltd.'s RBD1016, Assembly Biosciences’s ABI-6250, Huahui Health Ltd.’s Libevitug and HH-1270, Replicor Inc.’s REP 2139-Mg and REP 2139-Ca, and EIT Pharma’s Jitixib® (Lonafarnib).

Under the Hatch-Waxman Act, a pharmaceutical manufacturer may file an ANDA seeking approval of a generic copy of an approved innovator product or an NDA under Section 505(b)(2) that relies on the FDA’s prior findings of safety and effectiveness in approving the innovator product. A Section 505(b)(2) NDA may be for a new or improved version of

the original innovator product. Certain of our approved medicines, including Ctexli and Cholbam, are or may be subject to immediate competition from compounded and generic entrants, as the ANDA and NDA for these drug products have no remaining or current patent or non-patent exclusivity although Ctexli does have orphan designation for the CTX indication. Further, as described in more detail in this Part I, Item 1A and Note 15 in the notes to our consolidated financial statements appearing elsewhere in this Annual Report, we have initiated litigation against certain parties alleging infringement of certain "Orange Book" listed patents covering LIVMARLI in accordance with the procedures set out in the Hatch-Waxman Act following the submission by such parties of ANDAs directed to generic versions of LIVMARLI. We cannot make any predictions about the final outcome of these matters or the timing thereof.

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. 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.

We are not aware of FDA or European Commission approved therapeutics for the treatment of FXS. We are aware of one other company, Shionogi & Co., LTD., pursuing clinical development of a PDE4D inhibitor (zatomilast/ BPN14770) in FXS. We are aware of several other companies pursuing clinical development of therapies for FXS including Harmony Biosciences Inc.'s ZYN002, Allos Pharma Inc.'s Arbaclofen, Healx Ltd.'s Gabaxodol, Spinogenix Inc.'s SPG601, Connecta Therapeutics S.L.'s CTH120, and Kaerus Therapeutics Inc.'s KER-0193.

Even though we have obtained orphan drug designation for several of our product candidates, we may not be able to obtain or maintain the benefits associated with orphan drug status, including market exclusivity.

Regulatory authorities in some jurisdictions, including the U.S. and the EU, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the U.S., or a patient population of greater than 200,000 individuals in the U.S., but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. In the EU, the European Commission, on the basis of the opinion of the EMA Committee for Orphan Medicinal Products ("COMP"), grants orphan drug designation for medicines to be developed for the diagnosis, prevention or treatment of diseases that are life-threatening or chronically debilitating, for which either no satisfactory method of diagnosis, prevention, or treatment exists, or if such method exists, the medicine is of significant benefit to those affected by such condition. To benefit from such designation, either the prevalence of such condition must not be more than five in 10,000 people across the EU or, if more prevalent, it must be unlikely that the marketing of the medicine would generate sufficient returns to justify the investment needed for its development. In September 2013, the FDA granted orphan drug status to Livmarli for the treatment of patients with PFIC and ALGS in the U.S. We also received orphan drug designation for Livmarli for PFIC and ALGS in the EU. In 2025, the FDA and COMP granted orphan drug designation for volixibat for PBC and PSC. The FDA granted orphan drug designation for chenodiol for the treatment of CTX in 2010. COMP granted orphan drug designation for brelovitug for hepatitis D virus infection in 2024. Generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug may be entitled to a period of marketing exclusivity, which precludes the FDA or the European Commission from approving another marketing application for the same drug (or, in the case of the European Commission, a similar drug) for the same indication for that time period. The applicable period is seven years in the U.S. and ten years in the EU, which may be extended by six months and two years, respectively, in the case of product candidates that have complied with the respective regulatory authority's agreed upon pediatric investigation plan ("PIP"). There is, however, a legislative proposal pending in the EU that may modify the length of orphan market exclusivity, change the way in which market exclusivity is awarded to drugs with more than one approved orphan indication. The exclusivity period in the EU can be reduced to six years if at the end of the fifth year a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or European Commission determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. In addition, even after a drug is granted orphan exclusivity and approved, the FDA can subsequently approve another drug for the same condition before the expiration of the seven-year exclusivity period including the same active ingredient, if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In the EU, the European Commission may approve another drug for the same indication despite its orphan exclusivity on the basis that it is not a similar medicinal product or if it is considered safer, more effective, or otherwise clinically superior. Conversely, the European Commission may deny marketing approval for a product candidate if it determines such product candidate is structurally similar to an approved product for the same indication. In addition, if an orphan designated product

receives marketing approval for an indication broader than or different from what is designated, such product may not be entitled to orphan exclusivity. Even though the FDA has granted orphan drug designation to Livmarli for the treatment of PFIC and ALGS, and for volixibat for the treatment of PBC, our current orphan drug designations may not provide exclusivity for approval for Livmarli or volixibat for modified or different indications.

Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process in the U.S. or the EU. Also, regulatory approval for any product candidate may be withdrawn, and other product candidates may obtain approval before us and receive orphan drug exclusivity, which could block us from entering the market.

Even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the candidate from competition because different drugs can be approved for the same condition before the expiration of the orphan drug exclusivity period.

We have formed and may continue to form or seek strategic alliances or enter into additional licensing arrangements in the future, and we may not realize the benefits of such alliances or licensing arrangements.

We have formed and may continue to form or seek strategic alliances, create joint ventures or collaborations or enter into additional licensing arrangements with third parties that we believe will complement or augment our development and commercialization efforts with respect to our approved medicines, our product candidates and any future product candidates that we may develop. We also have commercial partnerships outside of North America as well as in major European markets.

Any of our existing relationships or any future relationships we enter into may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders or disrupt our management and business. In addition, we face significant competition in seeking appropriate strategic partners and the negotiation process is time-consuming and complex. If we license products or businesses, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture. Following a strategic transaction or license, we may not achieve the revenues or specific net income that justifies such transaction. Any delays in entering into new strategic partnership agreements related to our product candidates could delay the development and commercialization of our product candidates in certain geographies for certain indications, which would harm our business prospects, financial condition and results of operations.

Our failure to successfully in-license, acquire, develop and market additional product candidates or approved medicines would impair our ability to grow our business.

Although a substantial amount of our efforts are focused on the clinical development, potential regulatory approval and commercialization of our approved medicines and product candidates, a key element of our long-term strategy is to in-license, acquire, develop, market and commercialize a portfolio of products to treat patients with rare diseases. Because we do not have the necessary internal research and development capabilities, unless we build such capabilities internally, we will be dependent upon pharmaceutical companies, academic scientists and other researchers to sell or license products or technology to us. The success of this strategy depends partly upon our ability to identify and select promising biopharmaceutical product candidates and products, negotiate licensing or acquisition agreements with their current owners and finance these arrangements. The process of proposing, negotiating and implementing a license or acquisition of a product candidate or approved product is lengthy and complex. Other companies, including some with substantially greater financial, marketing, sales and other resources, may compete with us for the license or acquisition of product candidates and approved medicines. We have limited resources to identify and execute the acquisition or in-licensing of third-party products, businesses and technologies and integrate them into our current infrastructure. Moreover, we may devote resources to potential acquisitions or licensing opportunities that are never completed, or we may fail to realize the anticipated benefits of such efforts. We may not be able to acquire the rights to additional approved medicines or product candidates on terms that we find acceptable, or at all. Further, any product candidate that we acquire may require additional development efforts prior to commercial sale, including preclinical or clinical testing and approval by the FDA, the European Commission and other similar regulatory authorities. For example, we are conducting the Phase 3 AZURE program for brelovitug to treat chronic HDV infection before we can seek approval by the competent regulatory authorities. All product candidates are prone to risks of failure during biopharmaceutical product development, including the possibility that a product candidate will not be shown to be sufficiently safe and effective for approval by regulatory authorities. In addition, any approved medicines that we acquire may not have the market potential we believe, be manufactured or sold profitably, or achieve market acceptance.

We may fail to realize all of the anticipated benefits of our commercial and product candidate acquisitions or those benefits may take longer to realize than expected.

We believe that there are significant benefits that may be realized from the Bluejay Acquisition or any other product or product candidate acquisition. For example, the full benefits of the Bluejay Acquisition including the anticipated financial contribution of newly acquired assets or businesses, may not be realized as expected, may be diminished due to competition or may not be achieved within the anticipated time frame, or at all. Failure to achieve the anticipated benefits of either the Bluejay Acquisition or any other product or product candidate acquisition, could adversely affect our results of operations or cash flows, divert needed resources away from our current approved medicines and product candidates, decrease or delay any accretive effect of an acquisition and negatively impact the price of our common stock.

We may not be able to integrate our acquired products and product candidates successfully. We have transferred and entered into new contracts for a number of vendors that support the manufacture and distribution of the acquired assets. We acquired all of Bluejay in the Bluejay Acquisition, including many of its employees, contracts and the operations of Bluejay. If we do not successfully integrate those employees, continue the operations and maintain the existing contractual relationships, we may fail to successfully develop brelovitug. If we fail to successfully integrate our acquired commercial products and product candidates, our results of operations could be adversely affected. The integration processes will continue to require significant time and resources, require significant attention from management and may disrupt the ordinary functioning of our business, and we may not be able to manage the processes successfully, which could harm our business.

We are highly dependent on our key personnel, and if we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.

Our ability to compete in the highly competitive biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. The loss of the services of any of our executive officers or other key employees and our inability to find suitable replacements could potentially harm our business, prospects, financial condition or results of operations.

We conduct many of our operations at our facility in Foster City, California. This region serves as the headquarters to many other biopharmaceutical companies and many academic and research institutions. Competition for skilled personnel in our market is intense and may limit our ability to hire and retain highly qualified personnel on acceptable terms or at all.

To induce valuable employees to remain at our company, in addition to salary and cash incentives, we have provided stock awards that vest over time. The value to employees of stock 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. In addition, in response to competition, high inflation rates and labor shortages, we may need to adjust employee cash compensation, which would affect our operating costs and our margins, or equity compensation, which would affect our outstanding share count and cause dilution to existing stockholders. Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us on short notice. Although we have offer letters with our key employees, these offer letters provide for at-will employment, which means that any of our employees could leave our employment at any time, with or without notice. We do not maintain "key man" insurance policies on the lives of these individuals or the lives of any of our other employees. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level, and senior managers as well as junior, mid-level, and senior scientific and medical personnel.

Many of the other biotechnology and pharmaceutical companies that we compete against for qualified personnel have greater financial and other resources, different risk profiles and a longer history in the industry than we do. They may also provide more diverse opportunities and better chances for career advancement. Some of these characteristics are more appealing to high quality candidates than what we can offer. If we are unable to continue to attract and retain high quality personnel, the rate and success at which we can discover, develop and commercialize product candidates will be limited.

We will need to grow the size of our organization, and we may experience difficulties in managing this growth.

As of December 31, 2025, we had 369 full-time employees. As our development and commercialization plans and strategies develop, we expect to need additional development, managerial, operational, financial, sales, marketing and other personnel. Future growth would impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, maintaining and motivating additional employees;
- managing our commercialization efforts while focusing on other areas of our business;

- managing our internal development efforts effectively, including the clinical and regulatory review process for our approved medicines and our product candidates, while complying with our contractual obligations to contractors and other third parties; and
- improving our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to commercialize our approved medicines, any then-approved product and product candidates depends, in part, on our ability to effectively manage any future growth, and our management may also have to divert a disproportionate amount of its attention away from day-to-day activities in order to devote a substantial amount of time to managing these growth activities. To date, we have used the services of outside vendors to perform tasks including clinical trial management, statistics and analysis, regulatory affairs, formulation development and other drug development functions. Our growth strategy may entail expanding our group of contractors or consultants to implement these tasks going forward. Because we rely on numerous consultants, effectively outsourcing many key functions of our business, we will need to be able to effectively manage these consultants to ensure that they successfully carry out their contractual obligations and meet expected deadlines. We may not be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all. Our growth strategy also includes transitioning certain outsourced functions of our business, such as our patient services hub, from third-party vendors to our employees. If we are unable to effectively manage our outsourced activities, if the quality or accuracy of the services provided by consultants is compromised for any reason, or if we are not able to successfully internalize certain functions of our business, our clinical trials may be extended, delayed or terminated, we may not successfully commercialize our approved medicines or obtain regulatory approval for our product candidates, and we may not otherwise advance our business. As we grow our organization to internalize operation of our patient services hub, we may experience adverse changes that we would not have experienced had our patient services hub remained with our third-party vendor, including the potential loss of patient data. If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to further develop and commercialize our approved medicines, any then-approved product and product candidates and, accordingly, may not achieve our research, development and commercialization goals.

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

Our operations, and those of our CROs and other contractors and consultants, could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics 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. We rely on third-party manufacturers to produce our approved medicines and product candidates. Our ability to obtain clinical supplies of these products could be disrupted if the operations of these suppliers are affected by a man-made or natural disaster or other business interruption. Our corporate headquarters is located in California near major earthquake faults and fire zones. The ultimate impact on us, our significant suppliers and our general infrastructure of being located near major earthquake faults and fire zones and being consolidated in certain geographical areas is unknown, but our operations and financial condition could suffer in the event of a major earthquake, fire or other natural disaster.

Our employees, independent contractors, principal investigators, CROs, consultants, strategic partners and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk that employees, independent contractors, principal investigators, CROs, consultants and vendors may engage in fraudulent or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violates: (1) the laws of the FDA, EU, individual EU Member States or comparable foreign regulatory authorities, including those laws that require the reporting of true, complete and accurate information to the FDA, EMA, the competent authorities of individual EU Member States or comparable foreign regulatory authorities; (2) manufacturing standards; (3) healthcare fraud and abuse laws in the U.S. and similar foreign fraudulent misconduct laws; or (4) laws that require the true, complete and accurate reporting of our financial information or data. These laws may impact, among other things, our current activities with principal investigators and research subjects, as well as proposed and future sales, marketing and education programs. In particular, the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, structuring and commission(s), certain customer incentive programs and other business arrangements generally.

Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials. If we obtain regulatory approval for any of our product candidates and begin commercializing those products in the U.S. or other foreign jurisdictions, our potential exposure under such laws will increase significantly, and our costs associated with compliance with such laws are also likely to increase. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, damages, disgorgement, monetary fines, imprisonment, possible exclusion from participation in the U.S. in Medicare, Medicaid and other federal healthcare programs and in equivalent foreign programs, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations.

Our relationships with customers, physicians and third-party payors may be subject, directly or indirectly, to federal, state and equivalent foreign healthcare fraud and abuse laws, false claims laws, transparency laws, health information privacy and security laws, monopoly and anti-trust laws, and other healthcare laws and regulations. If we or our employees, independent contractors, consultants, commercial partners or vendors violate these laws, we could face substantial penalties.

These laws may impact, among other things, our clinical research program, as well as sales, marketing and education programs. In particular, the promotion, sales and marketing of healthcare items and services is subject to extensive laws and regulations designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive and other business arrangements. We may also be subject to federal, state and foreign laws governing the privacy and security of identifiable patient information. The U.S. healthcare laws and regulations that may affect our ability to operate include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, any person or entity from knowingly and willfully, offering, paying, soliciting or receiving any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, the purchasing, leasing, ordering or arranging for the purchase, lease, or order of any item or service reimbursable under Medicare, Medicaid or other federal healthcare programs. The term "remuneration" has been broadly interpreted to include anything of value. Although there are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, the exceptions and safe harbors are drawn narrowly. Practices that may be alleged to be intended to induce prescribing, purchases or recommendations, include the provision of in-kind services, genetic testing services, or products or any payments of more than fair market value, and may be subject to scrutiny if they do not qualify for an exception or safe harbor. In addition, a person or entity does not need to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. In addition, 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 federal civil False Claims Act and the civil monetary penalties statute;
- federal civil and criminal false claims laws, including the federal civil False Claims Act, and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other federal government programs that are false or fraudulent or knowingly making a false statement to improperly avoid, decrease or conceal an obligation to pay money to the federal government, including federal healthcare programs;
- the Health Insurance Portability and Accountability Act ("HIPAA"), which created new federal civil and criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, including private third-party payors and knowingly and willfully falsifying, concealing or covering up by any trick, scheme or device, a material fact or making any materially false, fictitious or fraudulent statements in connection with the delivery of, or payment for, healthcare benefits, items or services. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), and their respective implementing regulations, which impose requirements on certain healthcare providers, health plans, and healthcare clearinghouses, known as covered entities and their

respective business associates that perform services for them as well as their covered subcontractors that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information;

- federal civil and criminal anti-trust or anti-monopoly laws that restrict or limit corporate actions and practices in order to regulate the conduct and organization of businesses in order to promote competition may apply to exclusive contractual relationships between manufacturers, distributors, and specialty pharmacies which dispense the manufactured products; and
- the federal Physician Payments Sunshine Act, which requires 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 report annually to CMS information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other healthcare professionals (such as physician assistants and nurse practitioners) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members.

We may also be subject to state and foreign equivalents of each of the healthcare laws described above, among others, some of which may be broader in scope. For example, we may be subject to the following: state and foreign anti-kickback and false claims laws that may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third party payors, including private insurers, or that apply regardless of payor; state and foreign laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government; state and foreign laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers, marketing expenditures, or drug pricing; state and local laws requiring the registration of pharmaceutical sales representatives; and state and foreign laws, such as the EU's and the United Kingdom's General Data Protection Regulations (respectively, the "EU GDPR" and "UK GDPR", together, the "GDPR") governing the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Additionally, we may be subject to federal and comparable foreign consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available, it is possible that some of our business activities, or our arrangements with physicians, could be subject to challenge under one or more of such laws. If we or our employees, independent contractors, consultants, commercial partners and vendors violate these laws, we may be subject to investigations, enforcement actions and/or significant penalties. We have adopted a code of conduct and healthcare compliance policies, but it is not always possible to identify and deter employee misconduct or business noncompliance, and the precautions we take to detect and prevent inappropriate conduct may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. Efforts to ensure that our business arrangements will comply with applicable healthcare laws may involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against us, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, damages, disgorgement, monetary fines, imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs or comparable foreign programs, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations. In addition, the approval and commercialization of any of our product candidates outside the U.S. will also likely subject us to foreign equivalents of the healthcare laws mentioned above, among other foreign laws.

Our business is subject to complex, stringent and evolving U.S. and foreign laws, regulations, and rules, contractual obligations, industry standards, policies and other obligations relating to privacy and data protection. Our actual or perceived failure to comply with such obligations could result in regulatory investigations or actions, litigations (including class claims) and mass arbitration demands, fines and penalties, disruptions of and changes to our business practices, monetary penalties, reputational harm, loss of revenue or profits, 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 U.S., federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal data privacy laws, consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), and other similar laws (e.g., wiretapping laws). For example, HIPAA, as amended by HITECH, imposes specific requirements relating to the privacy, security, and transmission of individually identifiable health information. Additionally, the Controlling the Assault of Non-Solicited Pornography and Marketing Act of 2003 ("CAN-SPAM") and Telephone Consumer Protection Act ("TCPA") imposes specific requirements on communications with customers. In particular, the TCPA imposes various consumer consent requirements and other restrictions relating to marketing to individuals using technology such as telephones, mobile devices, and text messages. TCPA violations can result in significant financial penalties, including penalties or criminal fines imposed by the Federal Communications Commission or fines of up to $1,500 per violation imposed through private litigation or by state authorities.

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 may include the right to access, correct, or delete certain personal data, and to opt-out of certain data processing activities, such as targeted advertising, profiling, and automated decision-making. The exercise of these rights may impact our business and ability to provide our products and services. Certain states also impose stricter requirements for processing certain personal data, including sensitive information, such as conducting data privacy impact assessments. These state laws allow for statutory fines for noncompliance. For example, at the state level, the California Consumer Privacy Act, as amended, ("CCPA"), applies to personal data of California consumers, business representatives and employees, and requires businesses to provide specific disclosures in privacy notices and honor requests of such individuals to exercise certain privacy rights. The CCPA provides for fines for violations and allows private litigants affected by certain data breaches to recover significant statutory damages. Although the CCPA and other comprehensive state privacy laws exempt some data processed in the context of clinical trials, these laws may increase compliance costs and potential liability for us and the third parties with whom we work. Similar laws are being considered in several other states, as well as at the federal and local levels, and we expect more states to pass similar laws in the future.

Outside the U.S., an increasing number of laws, regulations, and industry standards apply to data privacy and security. For example, the EU GDPR and the UK GDPR (together "GDPR"), Brazil's General Data Protection Law (Lei Geral de Proteção de Dados Pessoais, or "LGPD") (Law No. 13,709/2018), Canada's Personal Information Protection and Electronic Document Act ("PIPEDA") and China's Personal Information Protection Law ("PIPL") impose strict requirements for processing personal data, and violators of these laws may face significant penalties.

For example, the GDPR imposes stringent requirements for controllers and processors of personal data, including, for example, more robust disclosures to individuals and a strengthened individual data rights regime, mandatory data breach notifications in certain circumstances, limitations on retention of information, increased requirements pertaining to special categories of data, such as health data, and additional obligations when we contract with third-party processors in connection with the processing of personal data. In addition, the definition of "personal data" under the GDPR is broad and captures "pseudonymized" or key-coded data that is commonly processed in a clinical trial-related context.

We are subject to the GDPR because of our data processing activities that involve the personal data of individuals residing in the EEA and UK, such as in connection with our clinical trials in Europe, and early access program in multiple EU countries, and because of certain processing of personal data carried out in the context of the activities of our relevant European subsidiaries. In addition, we maintain an office in Switzerland, which subjects us to privacy and data protection laws and regulations similar to the GDPR under the Swiss Federal Act on Data Protection, or the FADP. The

FADP applies to the collection and processing of personal data, including health-related information, by companies located in Switzerland, or in certain circumstances, by companies located outside of Switzerland.

Furthermore, the EU GDPR provides that EEA Member States may introduce specific requirements related to the processing of "special categories of personal data", including the personal data related to health and genetic information, which we may process in connection with clinical trials or otherwise; as well as personal data related to criminal offenses or convictions where allowed under local law and confirmed by potential employee in employment situations. Under the GDPR, companies that do not comply may face temporary or definitive bans on data processing and other corrective actions; fines of up to 20 million Euros under the EU GDPR or 17.5 million pounds sterling under the UK GDPR, or in each case, 4% of annual global revenue, whichever is greater; or private litigation related to processing of personal data brought by classes of data subjects or consumer protection organizations authorized at law to represent their interests.

Additionally, in Canada, the Personal Information Protection and Electronic Documents Act ("PIPEDA") and various related provincial laws, as well as Canada's Anti-Spam Legislation ("CASL"), may apply to our operations.

In the ordinary course of business, we transfer personal data from Europe (including from our European subsidiaries) and other jurisdictions to the U.S. 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 EEA and the UK have significantly restricted the transfer of personal data to the U.S. and other countries whose privacy laws it generally believes are inadequate. Other jurisdictions may adopt or have already adopted similarly stringent data localization and/or cross-border data transfer laws. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and UK to the U.S. in compliance with law, such as the EEA standard contractual clauses, the UK's International Data Transfer Agreement / Addendum, 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 Framework), these mechanisms are subject to legal challenges, and there is no assurance that we can satisfy or rely on these measures to lawfully transfer personal data to the U.S. If there is no lawful manner for us to transfer personal data from the EEA, the UK, or other jurisdictions, to the U.S., or if the requirements for a legally-compliant transfer are too onerous, we could face significant adverse consequences, including limitations of our ability to conduct clinical trial activities in Europe and/or elsewhere, the interruption or degradation of our operations, the need to relocate part of or all of our business or data processing activities to other jurisdictions (such as Europe) at significant expense, increased exposure to regulatory actions, substantial fines and penalties, the inability to transfer personal data and work with partners, vendors and other third parties, and/or injunctions against our processing or transferring of personal data necessary to operate our business. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers of personal data to recipients outside Europe for allegedly violating the EU GDPR's cross-border data transfer limitations. For example, in May 2023, the Irish Data Protection Commission determined that a major social media company's use of the standard contractual clauses to transfer personal data from Europe to the U. S. was insufficient and levied a 1.2 billion Euro fine against the company and prohibited the company from transferring personal data to the U.S. Additionally, companies that transfer personal data to recipients outside of the EEA and/or UK to other jurisdictions, particularly to the U.S., are subject to increased scrutiny from regulators, individual litigants and activist groups.

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

In addition, 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, some of our customer contracts require us to host personal data locally. We also publish privacy policies, marketing materials and other statements, such as statements related to compliance with certain certifications or self-regulatory principles, concerning artificial intelligence, data privacy and security. Regulators in the United States may scrutinize these statements, and 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 adverse consequences.

Obligations related to data privacy and security (and consumers' data privacy expectations) are quickly changing, becoming increasingly stringent, and creating uncertainty. Additionally, these obligations may be subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions. Preparing for and complying with these obligations requires significant resources and may necessitate changes to our information technologies, systems, and practices and to those of any third parties that process personal data on our behalf.

Although we endeavor to comply with our 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.

Our, or the third parties with whom we work, actual or perceived failure to adequately comply with applicable laws and regulations relating to privacy and data protection, or to protect personal data and other data we process or maintain, could result in adverse consequences, including regulatory fines and bans on processing personal data, government enforcement actions (e.g., investigations, fines, penalties, audits, inspections, and similar), litigation (including class action claims), mass arbitration demands, and other liabilities, claims for damages by affected individuals, orders to destroy or not use personal data, imprisonment of company officials, additional reporting requirements and/or oversight, interruptions or stoppages in our business operations (including, as relevant, clinical trials), and damage to our reputation. Any of these consequences could have a material adverse effect on our business, financial condition, results of operations and growth prospects, including but not limited to: loss of customers; interruptions or stoppages in our business operations (including, clinical trials); inability to process personal data or to operate in certain jurisdictions; limited ability to develop or commercialize our approved medicines; expenditure of time and resources to defend any claim or inquiry; adverse publicity; or substantial changes to our business model or operations. 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 collaboration arrangements that we have or may enter into in the future may not be successful or may result in product diversion, which could adversely affect our ability to develop and commercialize our approved medicines and any then-approved product.

Any existing or future collaborations that we enter into may not be successful. The success of our collaboration arrangements depends and will depend heavily on the efforts and activities of our collaborators. Collaborations are subject to numerous risks, which may include that:

- collaborators have significant discretion in determining the efforts and resources that they will apply to collaborations;
- collaborators may conduct their own clinical trials which may not be compliant, may not be successful or may generate contradictory results;
- collaborators may not pursue development and commercialization of our approved medicines and any then-approved product or may elect not to continue or renew development or commercialization programs based on trial or test results, changes in their strategic focus due to the acquisition of competitive products, availability of funding or other external factors, such as a business combination that diverts resources or creates competing priorities;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product or product candidates;
- a collaborator with marketing, manufacturing and distribution rights to one or more products may not commit sufficient resources to or otherwise not perform satisfactorily in carrying out these activities;
- we could grant exclusive rights to our collaborators that would prevent us from collaborating with others;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;
- a collaborator or series of collaborators may improperly or unknowingly sell product directly (or indirectly to a potential customer) into the "gray market" whereby our branded products are diverted from authorized sales channels into the hands of dealers, brokers or the open market, and may result in unauthorized sale of our product in a specific country or region;

- disputes may arise between us and a collaborator that causes the delay or termination of the research, development or commercialization of our current or future products or that results in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated, and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable current or future products;
- collaborators may own or co-own intellectual property covering our approved medicines and any then-approved product that results from our collaborating with them, and in such cases, we would not have the exclusive right to develop or commercialize such intellectual property; and
- a collaborator's sales and marketing activities or other operations may not be in compliance with applicable laws resulting in civil or criminal proceedings.

Risks Related to Our Reliance on Third Parties

We depend on intellectual property licensed from third parties and termination of any of these licenses could result in the loss of significant rights, which would harm our business.

We are dependent on patents, know-how and proprietary technology, both our own and licensed from others. For example, we entered into an assignment and license agreement with Shire pursuant to which we were assigned exclusive global rights to license intellectual property and know-how related to Livmarli and volixibat, rights to license know-how related to Livmarli from Pfizer, certain patents and know-how related to volixibat from Sanofi and certain patents and know-how related to Livmarli and volixibat from Satiogen, which we subsequently acquired in May 2022. We also acquired licensed rights to commercialize Cholbam and chenodiol from certain parties via the Bile Acid Portfolio Acquisition. We acquired licensed rights to develop brelovitug from Novartis, and the manufacture of brelovitug depends on a cell-line license from Lonza. We are required to use commercially reasonable efforts or diligent efforts to commercialize products based on the licensed rights and to pay certain royalties based off our net sales. We may not meet these requirements, which could result in a loss or termination of any rights under such agreements. Any termination of these licenses will result in the loss of significant rights and will restrict our ability to commercialize our product candidates.

We are generally also subject to all of the same risks with respect to protection of intellectual property that we license, as we are for intellectual property that we own, which are described below under "Risks Related to Our Intellectual Property." If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize products could suffer.

We rely on third parties to conduct our clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize our product candidates.

We currently rely on, and intend to continue relying on, third-party CROs in connection with our clinical trials. We control or will control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our clinical trials is conducted in accordance with applicable protocol, legal, regulatory and scientific standards, and our reliance on our CROs does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with GCPs, which are regulations and guidelines enforced by the FDA, the competent authorities of the individual EU Member States, or comparable foreign regulatory authorities for product candidates in clinical development. Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of these CROs fail to comply with applicable GCP regulations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, EMA, the competent authorities of the individual EU Member States, or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. Upon inspection, such regulatory authorities may not determine that any of our clinical trials comply with the GCP regulations. In addition, our clinical trials must be conducted with drug product produced under cGMP regulations and will require a large number of test subjects. Our failure or any failure by our CROs to comply with these regulations or to recruit a sufficient number of patients may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, our business may be implicated if any of our CROs violates federal, state or foreign fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

Our CROs are not our employees and, except for requirements and remedies available to us under our agreements with such CROs, we have limited control whether or not they devote sufficient time and resources to our ongoing preclinical, clinical and non-clinical programs. These CROs may also have relationships with other commercial

entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities, which could affect their performance on our behalf. If our CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to complete development of, obtain regulatory approval for or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed.

Switching or adding CROs involves substantial cost and requires extensive management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur when CROs are switched or added, which can materially impact our ability to meet our desired clinical development timelines. Although we carefully manage our relationships with our CROs, we may encounter challenges or delays in the future and these delays or challenges may have a material adverse impact on our business, prospects, financial condition and results of operations.

We rely completely on third parties to manufacture and distribute our clinical and commercial drug supplies, including certain sole-source suppliers and manufacturers. These third parties may fail to obtain and maintain regulatory approval for their facilities, fail to provide us with sufficient quantities of drug substance, drug product, or labeled finished product in a timely fashion, or fail to do so at acceptable quality levels or prices.

We do not currently have, nor do we plan to acquire, the infrastructure or capability internally to manufacture or distribute our clinical and commercial drug supplies. Instead, we rely on contracted third parties.

We do not currently have any long-term agreements or commitment with a manufacturer to produce raw materials, APIs and the finished products of our product candidates or the associated packaging. We will need to continue to identify and qualify a third-party manufacturer prior to commercialization of our product candidates, and we intend to enter into agreements for commercial production with third-party suppliers. For our approved medicines, we are reliant on third parties to successfully manufacture drug substance components and the finished drug product in accordance with regulatory requirements and in sufficient quantities for commercialization. We are also reliant on third parties for the manufacture of packaging, labeling and oral dosing dispensers for our approved medicines. As our approved medicines are intended to treat rare diseases, we will only require a low-volume of raw materials and APIs, and in some cases with single-source suppliers and manufacturers. Our reliance on third-party suppliers and manufacturers, including single-source suppliers, could harm our ability to develop our product candidates or to commercialize our currently approved medicines and any product candidates that are approved in the future.

The manufacturing of biologics involves complex, multi-step processes, including the use of living cells and biological materials that are inherently variable and difficult to control. Small changes in raw materials, equipment, process parameters, environmental conditions, or operator performance can result in material differences in product quality, yield, stability, or potency, including the risk of contamination or batch failure. As a result, biologic manufacturing processes may be more susceptible to interruptions, delays, and quality issues than the manufacture of small-molecule drugs. Moreover, scaling up biologic manufacturing from clinical to commercial quantities involves additional risks, including unanticipated process challenges, reduced yields, or the need for process changes that may require regulatory review or approval. Any such changes could delay commercialization, increase costs, or adversely impact product comparability or regulatory acceptance.

Any of our existing or future suppliers or manufacturers may, among other things:

- fail to supply us with our approved medicines and product candidates on a timely basis or in the requested amount due to unexpected damage to or destruction of facilities, equipment or deliveries, labor disputes or otherwise, including “acts of God”;
- fail to increase manufacturing capacity and produce drug product and components in larger quantities and at higher yields in a timely or cost-effective manner, or at all, to sufficiently meet our clinical and commercial needs;
- be unable to meet our production demands, including due to issues related to their reliance on sole-source suppliers and manufacturers;
- become unavailable through business interruption or financial insolvency; or
- be unable or unwilling to supply or manufacture for us, or to renew current supply or manufacturing agreements when such agreements expire on a timely basis, on acceptable terms or at all.

In the event of any of the foregoing or in the event such third parties fail to meet our needs, if we do not have an alternative supplier or manufacturer in place, we would be required to expend substantial management time and expense to identify, qualify and transfer processes to alternative suppliers or manufacturers. Transferring technology to other sites may require additional processes, technologies and validation studies, which are costly, may take considerable amounts of time, may not be successful and, in most cases, require review and approval by the FDA, the competent authorities of the individual EU Member States or comparable foreign regulatory authorities. Any need to find and qualify new suppliers or manufacturers could adversely impact our ability to commercialize our approved medicines or our product candidates, if approved. Additionally, we and our manufacturers do not currently maintain significant inventory of drug substances and other materials. Any delay or interruption in the supply of clinical trial supplies could delay the completion of our clinical trials, increase the costs associated with maintaining clinical trial programs and, depending upon the period of delay, require us to commence new clinical trials at additional expense or terminate clinical trials completely.

Although we are ultimately responsible for ensuring compliance with regulatory requirements such as cGMPs, we are dependent on our contract suppliers and manufacturers for day-to-day compliance with cGMP for production of both drug substances and finished products. Facilities used by our contract suppliers and manufacturers to produce the drug substances and materials or finished products for commercial sale must pass inspection and be approved by the FDA and other relevant regulatory authorities including the competent authorities of the individual EU Member States. A number of our contract suppliers and manufacturers must comply with cGMP requirements enforced by the FDA, the competent authorities of the individual EU Member States, and other equivalent foreign authorities, through their facilities inspection program and review of submitted technical information. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the FDA's, the competent authorities of the individual EU Member States, and other equivalent foreign authorities' strict regulatory requirements, they will not be able to secure or maintain FDA approval for the manufacturing facilities and our ability to secure supplies of our approved medicines or our product candidates will be negatively affected.

In addition, we have limited control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the safety of our approved medicines is compromised due to a failure to adhere to applicable laws or for other reasons, the manufacturing facilities may need to be closed for an extended period of time and we may need to find alternative manufacturing facilities, in which case we might not be able to identify manufacturers for clinical or commercial supply on acceptable terms, or at all, which would significantly impact our ability to develop, obtain regulatory approval for or market our approved medicines.

We and our third-party suppliers and manufacturers are vulnerable to geopolitical and macroeconomic developments, such as potential future bank failures, tariffs and trade tensions, the ongoing shutdown of the federal government and the resulting effects on its regulatory agencies, geopolitical tensions, the ongoing conflicts between Ukraine and Russia and in the Middle East, and increasing tensions between the U.S. and China, as well as any related political or economic responses and counter-responses or otherwise by various global actors or the general effect on the global economy and supply chain, future pandemics, high inflation rates and the responses by central banking authorities to control such inflation, which could negatively impact the availability or cost of materials and the third parties on which we rely. Similarly, the manufacturing facilities of a majority of our suppliers are located outside of the U.S. This may give rise to difficulties in importing our product into the U.S. or other countries as a result of, among other things, regulatory agency approval requirements, taxes, tariffs, local import requirements such as import duties or inspections, incomplete or inaccurate import documentation, defective packaging or negative impacts on global shipping due to geopolitical and macroeconomic developments. If such events result in any interruption in the supply of a drug substance or other material or in the manufacture of our approved medicines, such interruption could have a material adverse effect on our business, financial condition, operating results and prospects.

We rely on a specialty pharmacy for all of our sales of our approved medicines in each of the U.S. and Canada and use 3PLs, authorized distributors and licensed partners outside of the U.S. Switching or adding a specialty pharmacy or distributor involves substantial cost and requires extensive management time and focus. In addition, there is a natural transition period when a new specialty pharmacy or distributor commences work. If either the specialty pharmacy or the distributor becomes subject to bankruptcy or is acquired by a company that wants to terminate the relationship with us, and we are required to transition to a new specialty pharmacy or distributor, such transition may result in an inability for us to collect outstanding receivables and a decline in our revenue, results of operations and cash flows.

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

We operate in a global economy, and our business depends on a global supply chain for the development, manufacturing, and distribution of our pharmaceutical products, and for the advancement of our preclinical and clinical development programs. There is inherent risk, based on the complex relationships among the U.S. and the countries in

which we conduct our business, that political, diplomatic, and national security factors can lead to global trade restrictions and changes in trade policies and export regulations that may adversely affect our business and operations. The current international trade and regulatory environment is subject to significant ongoing uncertainty.

Tariff policies, particularly those affecting China and pharmaceutical products, could increase our costs and reduce our profitability, including as a result of our inability to adjust pricing in formulary-based markets. Recent and potential future changes in international trade policies, particularly regarding U.S.-China trade relations and pharmaceutical-specific tariffs, present risks to our operations and financial performance.

The ongoing trade tensions between the U.S. and other jurisdictions have resulted in multiple rounds of tariffs and anticipated tariffs affecting pharmaceuticals and pharmaceutical ingredients, including finished products, manufacturing equipment, and related supplies. Tariffs on these inputs may increase our manufacturing costs for certain products. Moreover, the dynamic and unpredictable tariff and trade landscape creates uncertainty and planning challenges for our operations. Changes in tariff classifications, country-of-origin requirements, or customs procedures can occur with limited notice. Further, the Bureau of Industry and Security, U.S. Department of Commerce, has initiated an investigation to determine whether pharmaceutical ingredients, including finished drug product, manufactured outside the U.S. pose a national security risk and should be subject to additional tariffs. Unlike consumer goods, pharmaceuticals face unique regulatory constraints that make rapid supply chain adjustments particularly difficult and costly. This uncertainty complicates our long-term investment decisions regarding manufacturing facilities, supply chain optimization, and research and development locations.

Unlike many industries, our ability to pass increased costs to customers is limited by the structure of pharmaceutical pricing and reimbursement systems. Many of our products are included in formularies with pricing established through annual or multi-year contracts with commercial, third-party payors and pharmacy benefit managers, and reimbursement methodologies established by government programs, such as Medicare. These arrangements typically include fixed pricing terms that were negotiated prior to the implementation of the recently announced tariffs. As a result, and depending on the timing and scope of the implementation of these tariffs, cost increases due to tariffs may be difficult or impossible to pass through to customers until the next negotiation cycle, which could be several months or years away.

Current or future tariffs may also result in increased research and development expenses, including with respect to increased costs associated with APIs and raw materials. Trade restrictions affecting the import of materials necessary for clinical trials could result in delays to our development timelines. Increased development costs and extended development timelines could place us at a competitive disadvantage compared to companies operating in regions with more favorable trade relationships and could reduce investor confidence and negatively impact our business, results of operations, financial condition and growth prospects.

The complexity of announced or future tariffs may also increase the risk that we or our customers or suppliers may be subject to civil or criminal enforcement actions in the U.S. or foreign jurisdictions related to compliance with trade regulations. Foreign governments may also adopt non-tariff measures, such as procurement preferences or informal disincentives to engage with, purchase from or invest in U.S. entities, which may limit our ability to compete internationally and attract non-U.S. investment, employees, customers and suppliers. Foreign governments may also take other retaliatory actions against U.S. entities, such as decreased intellectual property protection, increased enforcement actions, or delays in regulatory approvals, which may result in heightened international legal and operational risks. In addition, the U.S. and other governments have imposed and may continue to impose additional sanctions, such as trade restrictions or trade barriers, which could restrict us from doing business directly or indirectly in or with certain countries or parties and may impose additional costs and complexity to our business.

Trade disputes, tariffs, restrictions and other political tensions between the U.S. and other countries may also exacerbate unfavorable macroeconomic conditions including inflationary pressures, foreign exchange volatility, financial market instability, and economic recessions or downturns. The ultimate impact of current or future tariffs and trade restrictions remains uncertain and could materially and adversely affect our business, financial condition, and prospects. While we actively monitor these risks, any prolonged economic downturn, escalation in trade tensions, or deterioration in international perception of U.S.-based companies could materially and adversely affect our business, ability to access the capital markets or other financing sources, results of operations, financial condition and prospects. In addition, tariffs and other trade developments have and may continue to heighten the risks related to the other risk factors described elsewhere in this Annual Report.

Risks Related to Our Financial Position and Capital Requirements

We may need substantial additional financing to continue our commercialization efforts for our approved medicines, develop our product candidates, license or acquire new product candidates and approved medicines, and implement our operating plans. If we fail to obtain additional financing when needed, we may be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

Our operations have consumed substantial amounts of cash since inception. We expect to continue to spend substantial amounts to continue the clinical development and seek regulatory approval of our product candidates. We will require significant additional amounts in order to continue our marketing and sales efforts for our approved medicines, prepare for commercialization for our product candidates, and, if approved, to launch and commercialize our product candidates.

Based on our current and anticipated level of operations, we believe our existing unrestricted cash, cash equivalents and investments will be sufficient to fund current operations through at least the next 12 months. However, changing circumstances may cause us to consume capital significantly faster than we currently anticipate, and we may need to spend more money than currently expected because of circumstances beyond our control. We may require additional capital for the further development and commercialization of our product candidates and may need to raise additional funds sooner if we choose to expand more rapidly than we presently anticipate.

Additional funding may not be available on acceptable terms, or at all. As a result of adverse geopolitical and macroeconomic developments, such as potential future disruptions in access to bank deposits or lending commitments due to bank failures, tariffs and trade tensions, the ongoing shutdown of the federal government and the resulting effects on its regulatory agencies, geopolitical tensions, the ongoing conflicts between Ukraine and Russia and in the Middle East, and increasing tensions between the U.S. and China, as well as any related political or economic responses and counter-responses or otherwise by various global actors or the general effect on the global economy and supply chain, actual and anticipated changes in interest rates, economic inflation and the responses by central banking authorities to control such inflation, the global credit and financial markets have experienced extreme volatility and disruptions, including severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates and uncertainty about economic stability. If the equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly and more dilutive. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of our approved medicines and product candidates or other research and development initiatives. We also could be required to seek collaborators for our product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to our product candidates in markets where we otherwise would seek to pursue development or commercialization ourselves.

Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

We may seek additional capital through a combination of public and private equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. For example, in April 2023, we issued and sold $316.3 million aggregate principal amount of the Notes. In August 2023, in connection with and immediately prior to the closing of the Bile Acid Portfolio Acquisition, we completed a private placement of our common stock, pursuant to which we issued 8,000,000 shares of our common stock. In November 2023, we entered into a Sales Agreement (the "2023 Sales Agreement") with Leerink Partners LLC and Cantor Fitzgerald & Co., pursuant to which we may elect to issue and sell, from time to time, shares of common stock having an aggregate offering price of up to $200.0 million. In January and February 2026, we issued 4,673,597 shares of our common stock in connection with the closing of the Bluejay Acquisition, subject in certain cases to deduction to satisfy applicable taxes, and may issue up to 522,375 shares of our common stock in the future subject to certain conditions. Further, immediately following the closing of the Bluejay Acquisition, we issued and sold in two private placement offerings an aggregate of (i) 3,385,149 shares of our commons stock and (ii) pre-funded warrants to purchase 536,412 shares of our common stock, for aggregate gross proceeds of approximately $268.5 million. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our stockholders as such. For example, pursuant to the terms of the Notes and the related indenture (the "Indenture"), between us and U.S. Bank Trust Company, National Association, as trustee, our Notes are subject to conversion at the election of the holders for the quarterly period ending March 31, 2026, and if such an election

is made and we elect to settle such conversion obligation under the Notes in shares of our common stock or a combination of cash and shares of our common stock as we have done in the past, the issuance of such common stock would dilute the ownership interests of our stockholders and sales in the public market could adversely affect prevailing market prices. The incurrence of indebtedness would result in increased fixed payment obligations and could involve certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or product candidates, or grant licenses on terms unfavorable to us.

If we raise additional funds through collaboration, strategic partnerships and licensing arrangements with third parties, we may have to relinquish valuable rights to our approved medicines and product candidates, our intellectual property, future revenue streams or grant licenses on terms that are not favorable to us. If our cash flows and capital resources are insufficient to allow us to make required payments, we may have to reduce or delay capital expenditures, sell assets or seek additional capital.

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

Holders of the Notes may, subject to a limited exception described in certain provisions in the Notes and the related Indenture require us to repurchase the Notes following a fundamental change at a cash repurchase price generally equal to the principal amount of the Notes to be repurchased, plus accrued and unpaid interest, if any. In addition, upon conversion, we will satisfy part or all of our conversion obligation in cash unless we elect 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 Notes or pay any cash amounts due upon conversion. In addition, applicable law, regulatory authorities and agreements governing any future indebtedness may restrict our ability to repurchase the Notes or pay any cash amounts due upon conversion. Our failure to repurchase the Notes or to pay any cash amounts due upon conversion when required will constitute a default under the Indenture. A default under the Indenture or the fundamental change itself could also lead to a default under agreements governing any future indebtedness, which may result in any future indebtedness becoming immediately payable in full. We may not have sufficient funds to satisfy all amounts due under any future indebtedness and the Notes.

Our ability to utilize our net operating loss carryforwards and certain other tax attributes may be limited.

We have incurred substantial losses during our history and do not expect to become profitable in the near future, and we may never achieve profitability. As of December 31, 2025, we had federal and California and other state net operating loss ("NOL") carryforwards of approximately $185.9 million, $33.7 million and $71.4 million, respectively. The federal NOL carryforwards do not expire, and the California and other state NOL carryforwards will begin to expire in 2038, unless previously utilized. Our ability to utilize our NOL carryforwards and certain other tax attributes may be limited. As of December 31, 2025, we also had federal and state research and development credit carryforwards totaling $57.3 million and $10.8 million, respectively. The federal research and development credit carryforwards will begin to expire in 2039, unless previously utilized. The state research and development credits do not expire.

Under the current U.S. federal income tax law, federal NOLs generated in taxable years beginning after December 31, 2017 may be carried forward indefinitely, but the deductibility of such federal NOLs in a taxable year is limited to 80% of taxable income in such year. Similar rules may apply under state tax laws. Our NOL carryforwards and other applicable tax attributes are subject to review and possible adjustment by the U.S. Internal Revenue Service and state tax authorities and may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant stockholders over a three-year period in excess of 50 percentage points (by value), as defined under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended. It is possible that we have experienced one or more such ownership changes in the past, and we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership. We may therefore be limited in the portion of NOL carryforwards and other applicable tax attributes that we can use in the future to offset future taxable income. At the state level, California has enacted legislation that, with certain exceptions, suspends the ability to use California NOLs to offset California income and limits the ability to use California business tax credits to offset California taxes, for taxable years beginning after 2023 and before 2027. Other states may also suspend or place limitation on the NOL utilization. We have recorded a full valuation allowance related to our NOLs and other deferred tax assets due to the uncertainty of the ultimate realization of the future benefits of those assets.

Changes in tax laws or regulations that are applied adversely to us or our customers could have a material adverse effect on our business, cash flow, financial condition or results of operations.

New income, sales, use, excise or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could affect the tax treatment of our (and our subsidiaries') domestic and foreign financial results. Any new taxes could adversely affect our domestic and international business operations, and our business and financial performance. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us. Future guidance from the U.S. Internal Revenue Service and other tax authorities with respect to such legislation may adversely affect us, and certain aspects of such legislation could be repealed or modified in the future, which could have an adverse effect on us. For example, the OBBBA made a number of changes to U.S. federal income tax law, including 100% bonus depreciation, domestic research cost expensing, and modifications to the international tax framework. We are currently evaluating the impact of the OBBBA upon our future tax liabilities and continuing to monitor changes in tax laws and regulations.

Our indebtedness and liabilities could limit the cash flow available for our operations, expose us to risks that could adversely affect our business, financial condition and results of operations and impair our ability to satisfy our obligations under our indebtedness.

As of December 31, 2025, we had $316.2 million aggregate principal amount of indebtedness under the Notes.

We may also incur additional indebtedness to meet future financing needs. Our indebtedness could have significant negative consequences for our security holders and our business, results of operations and financial condition by, among other things:

- increasing our vulnerability to adverse economic and industry conditions;
- limiting our ability to obtain additional financing;
- requiring the dedication of a substantial portion of our cash flow from operations to service our indebtedness, which will reduce the amount of cash available for other purposes;
- limiting our flexibility to plan for, or react to, changes in our business;
- diluting the interests of our existing stockholders as a result of issuing shares of our common stock upon conversion of the Notes; and
- placing us at a possible competitive disadvantage with competitors that are less leveraged than us or have better access to capital.

Our ability to make scheduled payments of the principal of, to pay interest on or to refinance our indebtedness, including the Notes, depends on our future performance, which is subject to economic, financial, competitive and other factors beyond our control. Our business may not generate sufficient funds, and we may otherwise be unable to maintain sufficient cash reserves, to pay amounts due under our indebtedness, including the Notes, and our cash needs may increase in the future.

The conditional conversion feature of the Notes may adversely affect our financial condition and operating results, and conversion of our outstanding Notes may result in the dilution of existing stockholders, create downward pressure on the price of our common stock, and restrict our ability to take advantage of future opportunities.

The conditional conversion feature of the Notes entitles holders of the Notes to convert the Notes at any time during specified periods at their option if such conditions are met. For example, the conditional conversion feature of the Notes has been met previously, including in December 2025. Consequently, for prior quarterly periods, the Notes were, and for the quarterly period ending March 31, 2026, the Notes are, subject to conversion at the election of the holders. If one or more holders elect to convert their Notes, unless we elect to satisfy our conversion obligation by delivering solely shares of our common stock (other than paying cash in lieu of delivering any fractional share), we would be required to settle a portion or all of our conversion obligation in cash, which could adversely affect our liquidity.

The Notes may be converted into cash, shares of our common stock or a combination of cash and shares of our common stock. If shares of our common stock are issued to the holders of the Notes upon conversion as they have been in the past, there will be dilution to our stockholders' equity and the market price of our shares may decrease due to the additional selling pressure in the market. Any downward pressure on the price of our common stock caused by the sale or potential sale of shares issuable upon conversion of the Notes could also encourage short sales by third parties, creating additional selling pressure on our stock. The existence of the Notes and the obligations that we incurred by issuing them

may restrict our ability to take advantage of certain future opportunities, such as engaging in future debt or equity financing activities.

Also, ASU No. 2020-06 requires the application of the if-converted method to calculate the impact of convertible instruments on diluted earnings per share when the instruments may be settled in cash or shares. See Note 2, Summary of Significant Accounting Policies. During the three months ended December 31, 2025, the conditional conversion feature of the Notes was triggered and the Notes are convertible, in whole or in part, at the option of the holders between January 1, 2026 through March 31, 2026. We use the if-converted method for calculating any potential dilutive effect of the conversion options embedded in the Notes on diluted net income per share.

The accounting method for the Notes could adversely affect our reported financial condition and results.

In August 2020, the Financial Accounting Standards Board published an Accounting Standards Update, which we refer to as ASU 2020-06, which simplifies certain of the accounting standards that apply to convertible notes. In accordance with ASU 2020-06, the Notes are reflected as a liability on our balance sheets, with the initial carrying amount equal to the principal amount of the Notes, net of issuance costs. The issuance costs are treated as a debt discount for accounting purposes, which will be amortized into interest expense over the term of the Notes. As a result of this amortization, the interest expense that we expect to recognize for the Notes for accounting purposes will be greater than the cash interest payments we will pay on the Notes, which will result in lower reported income.

In addition, the shares underlying the Notes are reflected in our diluted earnings per share using the "if converted" method, in accordance with ASU 2020-06. Under that method, diluted earnings per share would generally be calculated assuming that all the Notes were converted solely into shares of common stock at the beginning of the reporting period, unless the result would be anti-dilutive. The application of the if-converted method may reduce our reported diluted earnings per share.

In the future, we may, in our sole discretion, irrevocably elect to settle the conversion value of the Notes in cash up to the principal amount being converted. Following such an irrevocable election, if the conversion value of the Notes exceeds their principal amount for a reporting period, then we will calculate our diluted earnings per share by assuming that all of the Notes were converted at the beginning of the reporting period and that we issued shares of our common stock to settle the excess, unless the result would be anti-dilutive.

Risks Related to Our Intellectual Property

We do not currently have patent protection or regulatory exclusivity for certain of our approved medicines or rely on regulatory exclusivity. If we are unable to obtain and maintain sufficient intellectual property protection for our approved medicines and our product candidates, or if the scope of the intellectual property protection is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our approved medicines and our other product candidates, if approved, may be adversely affected.

Our commercial success will depend in part on obtaining and maintaining a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our technologies. We do not have, and do not expect to obtain, patent protection for any commercial form of chenodiol or Cholbam. Any unauthorized disclosure to or misappropriation by third parties of our confidential proprietary information could enable competitors to quickly duplicate or surpass our technological achievements, thus eroding our competitive position in our market.

The patent positions of biotechnology and pharmaceutical companies can be highly uncertain and involve complex legal and factual questions for which important legal principles remain unresolved. No consistent policy regarding the breadth of claims allowed in pharmaceutical patents has emerged to date in the U.S. or in many jurisdictions outside of the U.S. Changes in either the patent laws or interpretations of patent laws in the U.S. and other jurisdictions may diminish the value of our intellectual property. Accordingly, we cannot predict the breadth of claims that may be enforced in the patents that may be issued from the applications we currently or may in the future own or license from third parties. Further, if any patents we obtain or license are deemed invalid and unenforceable, our ability to commercialize or license our technology could be adversely affected.

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or any of our actual or potential future collaborators will be successful in protecting our approved medicines or product candidates, proprietary technologies and their uses by obtaining and defending patents. These risks and uncertainties include the following:

- the U.S. Patent and Trademark Office ("USPTO") and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process, the noncompliance with which can result in abandonment or lapse of a patent or patent application, and partial or complete loss of patent rights in the relevant jurisdiction;
- patent applications may not result in any patents being issued;
- patents that may be issued or in-licensed have been and may again in the future be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable or otherwise may not provide any competitive advantage;
- our competitors, many of whom have substantially greater resources than we do and many of whom have made significant investments in competing technologies, may seek or may have already obtained patents that will limit, interfere with or eliminate our ability to make, use, import, and sell our approved medicines or our product candidates;
- other parties may have designed around our claims or developed technologies that may be related or competitive to our platform, may have filed or may file patent applications and may have received or may receive patents that overlap or conflict with our patent applications, either by claiming the same methods or devices or by claiming subject matter that could dominate our patent position;
- any successful opposition or other post-grant proceeding to any patents owned by or licensed to us could deprive us of rights necessary for the practice of our technologies or the successful commercialization of any products or product candidates that we may develop;
- because patent applications in the U.S. and most other jurisdictions are confidential for a period of time after filing, we cannot be certain that we or our licensors were the first to file any patent application related to our approved medicines or our product candidates, proprietary technologies and their uses;
- an interference proceeding can be provoked by a third party or instituted by the USPTO to determine who was the first to invent any of the subject matter covered by the patent claims of our applications for any application with an effective filing date before March 16, 2013;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the U.S. for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns; and
- jurisdictions other than the U.S. may have patent laws less favorable to patentees than those upheld by U.S. courts, allowing foreign competitors a better opportunity to create, develop and market competing products.

The patent prosecution process is also expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach such agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection. We may also rely on trade secrets to protect our technology, especially where we do not believe patent protection is appropriate or feasible. However, trade secrets are difficult to protect. Although we use reasonable efforts to protect our trade secrets, our employees, consultants, contractors, outside scientific collaborators and other advisors may unintentionally or willfully disclose our information to competitors. Enforcing a claim that a third party illegally obtained and is using any of our trade secrets is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the U.S. are sometimes less willing to protect trade secrets. Moreover, our competitors may independently develop equivalent knowledge, methods and know-how.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. Only limited protection may be available and may not adequately protect our rights or permit us to gain or keep any competitive advantage. If we do not adequately protect our intellectual property and proprietary technology, competitors may be able to

use our approved medicines, our product candidates and proprietary technologies and erode or negate any competitive advantage we may have, which could have a material adverse effect on our financial condition and results of operations. For example:

- others may be able to make compounds that are similar to our approved medicines and our product candidates but that are not covered by the claims of our patents;
- we might not have been the first to make the inventions covered by our pending patent applications;
- we might not have been the first to file patent applications for these inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies;
- any patents that we obtain may not provide us with any competitive advantages;
- we may not develop additional proprietary technologies that are patentable;
- our competitors might conduct research and development activities in jurisdictions where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we cannot ensure that any of our patents, or any of our pending patent applications, if issued, or those of our licensors, will include claims having a scope sufficient to protect our approved medicines and any then-approved medicine;
- we cannot ensure that we will be able to successfully commercialize our approved medicines and any then-approved product on a substantial scale, if approved, before the relevant patents that we own or license expire; or
- the patents of others may have an adverse effect on our business.

Others have filed, and in the future are likely to file, patent applications covering products and technologies that are similar, identical or competitive to ours or important to our business. We cannot be certain that any patent application owned by a third party will not have priority over patent applications filed or in-licensed by us, or that we or our licensors will not be involved in additional interference, opposition or other patent office proceedings before the USPTO or non-U.S. patent offices.

We cannot be certain that the claims in our issued patents and pending patent applications covering our approved medicines or our product candidates will be considered patentable by the USPTO, courts in the U.S., or by patent offices and courts in foreign jurisdictions. Furthermore, the laws of some foreign jurisdictions do not protect proprietary rights to the same extent or in the same manner as the laws of the U.S. As a result, we may encounter significant problems in protecting and defending our intellectual property.

The strength of patents in the biotechnology and pharmaceutical fields involves complex legal and scientific questions and can be uncertain. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our approved medicines or our product candidates in the U.S. or in foreign jurisdictions. Even if such patents do successfully issue, third parties have and may again in the future challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. Any successful opposition or other post-grant proceeding to our patents could deprive us of exclusive rights necessary for the successful commercialization of our approved medicines or our product candidates. Furthermore, even if they are unchallenged, our patents may not adequately protect our intellectual property, provide exclusivity for approved medicines or our product candidates or prevent others from designing around our claims. If the breadth or strength of protection provided by the patents we hold with respect to our approved medicines or our product candidates is threatened, it could dissuade companies from collaborating with us to develop, or threaten our ability to commercialize, our approved medicines or our product candidates.

Further, if we encounter delays in our development efforts, including our clinical trials, the period of time during which we could market our approved medicines or our product candidates under patent protection would be reduced. In addition, patents have a limited lifespan. In the U.S., the natural expiration of a patent is generally 20 years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. A patent term extension of up to five years based on regulatory delay may be available in the U.S. under the Hatch-Waxman Act. However, only a single patent can be extended for each marketing approval, and any patent can be extended only once, for a single product. Moreover, the scope of protection during the period of the patent term extension does not extend to the full scope of the claim, but instead only to the scope of the product as approved. Further, a patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and

only those claims covering such approved drug product, an approved method for using it or a method for manufacturing it may be extended. Laws governing analogous patent term extensions in foreign jurisdictions vary widely, as do laws governing the ability to obtain multiple patents from a single patent family. Additionally, we may not receive an extension if we fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration, or the term of any such extension is less than we request, the period during which we will have the right to exclusively market our product will be shortened and our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced.

For U.S. patent applications in which claims are entitled to a priority date before March 16, 2013, an interference proceeding can be provoked by a third party or instituted by the USPTO to determine who was the first to invent any of the subject matter covered by the patent claims of our patents or patent applications. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our participation in an interference proceeding may fail and, even if successful, may result in substantial costs and distract our management and other employees.

For U.S. patent applications containing a claim not entitled to priority before March 16, 2013, there is greater level of uncertainty in the patent law. In September 2011, the Leahy-Smith America Invents Act, or America Invents Act, was signed into law. The America Invents Act includes a number of significant changes to U.S. patent law, including provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. The USPTO has developed regulations and procedures to govern the administration of the America Invents Act, and many of the substantive changes to patent law associated with the America Invents Act, and in particular, the "first to file" provisions, were enacted on March 16, 2013. The America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable, or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our approved medicines and our product candidates and drug discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. We require all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology, such as third parties involved in the manufacture of our approved medicines and our product candidates and third parties involved in our clinical trials to enter into confidentiality agreements. We cannot be certain that all such agreements have been duly executed, that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets could impair our competitive position and may have a material adverse effect on our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. For example, the FDA, as part of its Transparency Initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all. If we are unable to prevent unauthorized material disclosure of our intellectual property to third parties, we may not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, operating results and financial condition.

We currently rely on method-of-use and formulation patents to protect Livmarli and composition-of-matter and method-of-use patents to protect volixibat.

We currently have rights to patents and patent applications in the U.S., Europe and other jurisdictions covering methods of treating certain cholestatic liver diseases using certain IBATis, including maralixibat (the API of Livmarli) and volixibat. Patent applications may never issue as patents. We do not have patents or patent applications covering maralixibat as a composition-of-matter. Therefore, the primary patent-based intellectual property protection for our Livmarli program are granted method-of-use patents and any patents that may grant on currently pending method-of-use and formulation patent applications.

Composition-of-matter patents on APIs are generally considered to be the strongest form of intellectual property protection for pharmaceutical products, as such patents provide protection without regard to any method of use. Method-of-use patents protect the use of a product for the specified method. Method-of-use patents do not prevent a competitor from making and marketing a product that is identical to our product for an indication that is outside the scope of the patented

method. Moreover, even if competitors do not actively promote their products for our targeted indication(s), physicians may prescribe these products "off-label." Although off-label prescriptions may infringe or contribute to the infringement of method-of-use patents, the practice is common and such infringement is difficult to prevent or prosecute.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent process. Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on any issued patents and/or applications are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patents and/or applications. We have systems in place to remind us to pay these fees, and we employ an outside firm and rely on our outside counsel to pay these fees due to foreign patent agencies. While an inadvertent lapse may sometimes be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, our competitors might be able to enter the market earlier than should otherwise have been the case, which would have a material adverse effect on our business.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect Livmarli and our product candidates.

As is the case with other biotechnology and pharmaceutical companies, our success is heavily dependent on intellectual property, particularly on obtaining and enforcing patents. Our patent rights may be affected by developments or uncertainty in U.S. or foreign patent statutes, patent case law, USPTO rules and regulations or the rules and regulations of foreign patent offices. Obtaining and enforcing patents in the biotechnology industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. In addition, the U.S. may, at any time, enact changes to U.S. patent law and regulations, including by legislation, by regulatory rule-making, or by judicial precedent, that adversely affect the scope of patent protection available and weakened the rights of patent owners to obtain patents, enforce patent infringement and obtain injunctions and/or damages. For example, the scope of patentable subject matter under 35 U.S.C. 101 has evolved significantly over the past several years as the Court of Appeals for the Federal Circuit and the Supreme Court issued various opinions, and the USPTO modified its guidance for practitioners on multiple occasions. Other jurisdictions may likewise enact changes to their patent laws in ways that adversely diminish the scope of patent protection and weaken the rights of patent owners to obtain patents, enforce patent infringement and obtain injunctions and/or damages. Further, the U.S. and other governments may, at any time, enact changes to laws and regulations that create new avenues for challenging the invalidity of issued patents. For example, the America Invents Act created new administrative post-grant proceedings, including post-grant review, inter partes review, and derivation proceedings that allow third parties to challenge the validity of issued patents. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

We may not be able to protect our intellectual property rights throughout the world.

Patents are of national or regional effect. Filing, prosecuting and defending patents on Livmarli and our product candidates in all jurisdictions throughout the world would be prohibitively expensive. In addition, the laws of some foreign jurisdictions do not protect intellectual property rights in the same manner and to the same extent as laws in the U.S. Consequently, we may not be able to prevent third parties from practicing our inventions in all jurisdictions outside the U.S. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and further, may export otherwise infringing products to territories where we have patent protection, but enforcement of such patent protection is not as strong as that in the U.S. These products may compete with Livmarli and any then-approved product and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

The requirements for patentability may differ in certain jurisdictions. For example, unlike other jurisdictions, China has a heightened requirement for patentability, and specifically requires a detailed description of medical uses of a claimed drug. In India, unlike the U.S., there is no link between regulatory approval for a drug and its patent status. In addition to India, certain jurisdictions in Europe and developing jurisdictions, including China, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In those jurisdictions, we may have

limited remedies if patents are infringed or if we are compelled to grant a license to a third party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we own or license.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology or pharmaceutical products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly, and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

In addition, geo-political actions in the U.S. and in foreign countries could increase the uncertainties and costs surrounding the prosecution or maintenance of our patent applications or those of any current or future licensors and the maintenance, enforcement or defense of our issued patents or those of any current or future licensors. For example, due to the Russia-Ukraine conflict, the U.S. and other foreign governments have implemented various economic sanctions and trade and activity restrictions involving Russia and Belarus. It is possible that additional sanctions and restrictions will be imposed by the U. S. or other jurisdictions as the Russia-Ukraine conflict continues, and such actions may include limiting or preventing filing, prosecution, and/or maintenance of patent applications in Russia. Government actions may also prevent maintenance of issued patents in Russia. These actions could result in abandonment or lapse of our patents or patent applications, resulting in partial or complete loss of patent rights in Russia. If such an event were to occur, it could have a material adverse effect on our business. In addition, a decree was adopted by the Russian government in March 2022, allowing Russian companies and individuals to exploit inventions owned by patentees from the U.S. without consent or compensation. Consequently, we would not be able to prevent third parties from practicing our inventions in Russia or from selling or importing products made using our inventions in and into Russia. Accordingly, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

Finally, Europe's Unified Patent Court may in particular present uncertainties for our ability to protect and enforce our patent rights against competitors in Europe. On June 1, 2023, the EU unitary patent system was launched, providing a single pan-European Unitary Patent and a new European Unified Patent Court ("UPC"), for litigation involving European patents. Under the UPC, all European patents, including those issued prior to ratification of the European Patent Package, will by default automatically fall under the jurisdiction of the UPC. The UPC will provide our competitors with a new forum to centrally revoke our European patents that have not been opted out of the UPC, and allow for the possibility of a competitor to obtain pan-European injunctions. It will be several years before we will understand the scope of patent rights that will be recognized and the strength of patent remedies that will be provided by the UPC. Under the EU unitary patent system, we will have the right to opt our patents out of the UPC over the first seven years of the court's existence, but doing so may preclude us from realizing the benefits of the new unified court.

If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We are a party to a number of license agreements under which we are granted intellectual property rights that are important to our business. For example, certain trade secrets related to maralixibat are licensed from Pfizer, and patents, patent applications and trade secrets related to volixibat are licensed from Sanofi. Our existing license agreements as related to our approved medicines and product candidates impose various development, regulatory and/or commercial diligence obligations, payment of milestones and/or royalties and other obligations. If we fail to comply with our obligations under a license agreement, or we are subject to a bankruptcy, the license agreement may be terminated, in which event we would not be able to develop, commercialize or market our approved medicines or other product candidates, as the case may be.

Licensing of intellectual property rights is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise between us and our licensors regarding intellectual property rights subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;

- whether and the extent to which our technology and processes infringe on intellectual property rights of the licensor that are not subject to the licensing agreement;
- our right to sublicense intellectual property rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our approved medicines and our product candidates, and what activities satisfy those diligence obligations; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

If disputes over intellectual property rights that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, our business, results of operations, financial condition and prospects may be adversely affected. We may enter into additional licenses in the future and if we fail to comply with obligations under those agreements, we could suffer adverse consequences.

We may become subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may be subject to claims that former employees (including former employees of our licensors), collaborators or other third parties have an interest in our patents or other intellectual property as an inventor or co-inventor. The failure to name the proper inventors on a patent application can result in the patents issuing thereon being unenforceable. Inventorship disputes may arise from conflicting views regarding the contributions of different individuals named as inventors, the effects of foreign laws where foreign nationals are involved in the development of the subject matter of the patent, conflicting obligations of third parties involved in developing Livmarli or our product candidates or as a result of questions regarding co-ownership of potential joint inventions. Litigation may be necessary to resolve these and other claims challenging inventorship and/or ownership. Alternatively, or additionally, we may enter into agreements to clarify the scope of our rights in such intellectual property. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

We may not be successful in obtaining or maintaining necessary rights to product components and processes for our development pipeline through acquisitions and in-licenses.

Presently we have intellectual property rights, through licenses from third parties related to our approved medicines and our product candidates. For example, we have license agreements with Shire and Satiogen for both maralixibat and volixibat. We have our license agreement with Shire, Satiogen and Pfizer for our intellectual property rights covering maralixibat. Further, we have our license agreement with Sanofi for our intellectual property rights covering volixibat. Because our programs may require the use of additional proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license or use these proprietary rights. In addition, Livmarli or our product candidates may require specific formulations to work effectively and efficiently and these rights may be held by others. We may be unable to acquire or in-license proprietary rights related to any compositions, formulations, methods of use, processes or other intellectual property rights from third parties that we identify as being necessary for Livmarli or our product candidates. Even if we are able to obtain a license to such proprietary rights, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement technology.

Where we obtain licenses from or collaborate with third parties, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from third parties, or such activities, if controlled by us, may require the input of such third parties. We may also require the cooperation of our licensors and collaborators to enforce any licensed patent rights, and such cooperation may not be provided. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business, in compliance with applicable laws and regulations, which may affect the validity and enforceability of such patents or any patents that may issue from such application. Moreover, if we do obtain necessary licenses, we will likely have obligations under those licenses, including making royalty and milestone payments, and any failure to satisfy those obligations could give our licensor the right to terminate the license. Termination of a necessary license, or expiration of licensed patents or patent applications, could have a material adverse impact on our business. Our business would suffer if any such licenses terminate, if the licensors fail to abide by the terms of the license, if the licensors

fail to enforce licensed patents against infringing third parties, if the licensed patents or other rights are found to be invalid or unenforceable, or if we are unable to enter into necessary licenses on acceptable terms. Furthermore, if any licenses terminate, or if the underlying patents fail to provide the intended exclusivity, competitors or other third parties may gain the freedom to seek regulatory approval of, and to market, products identical to ours. Moreover, our licensors may own or control intellectual property that has not been licensed to us and, as a result, we may be subject to claims, regardless of their merit, that we are infringing or otherwise violating the licensor's rights. In addition, while we cannot currently determine the amount of the royalty obligations we would be required to pay on sales of future products, if any, the amounts may be significant. The amount of our future royalty obligations will depend on the technology and intellectual property we use in products that we successfully develop and commercialize, if any. Therefore, even if we successfully develop and commercialize products, we may be unable to achieve or maintain profitability.

The licensing and acquisition of third-party proprietary rights is a competitive area, and companies, which may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third-party proprietary rights that we may consider necessary or attractive in order to commercialize our approved medicines or our product candidates. More established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

For example, we have collaborated and may in the future collaborate with U.S. and foreign academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate an exclusive license to any of the institution's proprietary rights in technology resulting from the collaboration. Regardless of such option to negotiate a license, we may be unable to negotiate a license within the specified time frame or under terms that are acceptable to us. If we are unable to do so, the institution may offer, on an exclusive basis, their proprietary rights to other parties, potentially blocking our ability to pursue our program.

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us, either on reasonable terms, or at all. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment, or at all. If we are unable to successfully obtain rights to required third-party intellectual property rights on commercially reasonable terms, our ability to commercialize our approved medicines and any then-approved product, and our business, financial condition and prospects for growth could suffer.

Third-party claims alleging intellectual property infringement may prevent or delay our drug discovery and development efforts.

Our success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the U.S., involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including inter partes review, post-grant proceedings, interference and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. The America Invents Act introduced new procedures including inter partes review and post grant review. The implementation of these procedures brings uncertainty to the possibility of challenges to our patents in the future and the outcome of such challenges. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are marketing our approved medicines and developing our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our activities related to our approved medicines and our product candidates may give rise to claims of infringement of the patent rights of others.

The pharmaceutical and biotechnology industries have produced a proliferation of patents, and it is not always clear to industry participants, including us, which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. Any of our approved medicines or our current or future product candidates may infringe existing or future patents. We may not be aware of patents that have already issued that a third party might assert are infringed by our approved medicines or one of our current or future product candidates. Nevertheless, we are not aware of any issued patents that will prevent us from marketing our approved medicines or our product candidates.

Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents of which we are currently unaware with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our approved medicines or our product candidates. Because patent applications can take many years to issue and may be confidential for 18 months or more after filing, there may be currently pending third-party patent applications which may later result in issued patents that our approved medicines, our product candidates or our technologies may infringe, or which such third parties claim are infringed by the use of our

technologies. Parties making claims against us for infringement or misappropriation of their intellectual property rights may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our approved medicines or one or more of our product candidates. Defense of these claims, regardless of their merit, could involve substantial expenses and could be a substantial diversion of employee resources from our business.

If we collaborate with third parties in the development of technology in the future, our collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to litigation or potential liability. Further, collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability. In the future, we may agree to indemnify our commercial collaborators against certain intellectual property infringement claims brought by third parties.

Any claims of patent infringement asserted by third parties would be time consuming and could:

- result in costly litigation;
- divert the time and attention of our technical personnel and management;
- cause development delays;
- prevent us from commercializing our approved medicines or our product candidates until the asserted patent expires or is held finally invalid or not infringed in a court of law;
- require us to develop non-infringing technology, which may not be possible on a cost-effective basis;
- require us to pay damages to the party whose intellectual property rights we may be found to be infringing, which may include treble damages if we are found to have been willfully infringing such intellectual property;
- require us to pay the attorney's fees and costs of litigation to the party whose intellectual property rights we may be found to be infringing; and/or
- require us to enter into royalty or licensing agreements, which may not be available on commercially reasonable terms, or at all.

If we are sued for patent infringement, we would need to demonstrate that our approved medicines and any then-approved product or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid, and we may not be able to do either. Proving invalidity is difficult. For example, in the U.S., proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and divert management's time and attention in pursuing these proceedings, which could have a material adverse effect on us. If we are unable to avoid infringing the patent rights of others, we may be required to seek a license, which may not be available, defend an infringement action or challenge the validity of the patents in court. Patent litigation is costly and time consuming. We may not have sufficient resources to bring these actions to a successful conclusion. In addition, if we do not obtain a license, develop or obtain non-infringing technology, fail to defend an infringement action successfully or have infringed patents declared invalid, we may incur substantial monetary damages, encounter significant delays in bringing our product candidates to market and be precluded from manufacturing or selling our approved medicines or our product candidates.

We do not always conduct independent reviews of pending patent applications of and patents issued to third parties. We cannot be certain that others have not filed patent applications for technology covered by our pending applications, or that we were the first to invent the technology, because:

- some patent applications in the U.S. may be maintained in secrecy until the patents are issued;
- patent applications in the U.S. and elsewhere can be pending for many years before issuance, or unintentionally abandoned patents or applications can be revived;
- pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our technologies, our approved medicines, our product candidates or the use thereof;
- identification of third-party patent rights that may be relevant to our technology is difficult because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims;

- patent applications are typically not published until 18 months after the priority date; and
- publications in the scientific literature often lag behind actual discoveries.

Furthermore, the scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history and can involve other factors such as expert opinion. Our interpretation of the relevance or the scope of claims in a patent or a pending application may be incorrect, which may negatively impact our ability to market our approved medicines and any then-approved product. Further, we may incorrectly determine that our technologies, our approved medicines and any then-approved product, or product candidates are not covered by a third-party patent or may incorrectly predict whether a third party's pending patent application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the U.S. or internationally that we consider relevant may be incorrect, which may negatively impact our ability to develop and market our approved medicines or our product candidates.

Our competitors may have filed, and may in the future file, patent applications covering technology similar to ours, and others may have or obtain patents or proprietary rights that could limit our ability to make, use, sell, offer for sale or import our approved medicines, our product candidates and future approved medicines or impair our competitive position. Numerous third-party U.S. and foreign issued patents and pending patent applications exist in the fields in which we are marketing our approved medicines and developing product candidates. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our approved medicines and our product candidates. Any such patent application may have priority over our patent applications, which could further require us to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on inventions similar to ours, we may have to participate in an interference proceeding declared by the USPTO to determine priority of invention in the U.S. The costs of these proceedings could be substantial, and it is possible that such efforts would be unsuccessful if, unbeknownst to us, the other party had independently arrived at the same or similar invention prior to our own invention, resulting in a loss of our U.S. patent position with respect to such inventions. Other jurisdictions have similar laws that permit secrecy of patent applications and may be entitled to priority over our applications in such jurisdictions.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations.

If a third party prevails in a patent infringement lawsuit against us, we may have to stop making and selling the infringing product, pay substantial damages, including treble damages and attorneys' fees if we are found to be willfully infringing a third party's patents, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure.

We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of our approved medicines and our product candidates. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize our approved medicines and our product candidates, which could harm our business significantly. Even if we were able to obtain a license, the rights may be nonexclusive, which may give our competitors access to the same intellectual property.

We may be subject to claims that we have wrongfully hired an employee from a competitor or that we or our employees have wrongfully used or disclosed alleged confidential information or trade secrets of their former employers.

As is common in the biotechnology and pharmaceutical industries, in addition to our employees, we engage the services of consultants to assist us in the development of our product candidates. Many of these consultants, and many of our employees, were previously employed at, or may have previously provided or may be currently providing consulting services to, other pharmaceutical companies including our competitors or potential competitors. We may become subject to claims that we, our employees or consultants inadvertently or otherwise used or disclosed trade secrets or other information proprietary to their former employers or their former or current clients. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely affect our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to our management team and other employees.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time consuming, and unsuccessful. Further, our issued patents could be found invalid or unenforceable if challenged in court, and we may incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights.

Third parties including competitors may infringe, misappropriate or otherwise violate our patents, patents that may issue to us in the future, or the patents of our licensors that are licensed to us. To counter infringement or unauthorized use, we may need to or choose to file infringement claims, which can be expensive and time-consuming. We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in jurisdictions where the laws may not protect those rights as fully as in the U.S. Further, because of the expense and uncertainty of litigation, we may conclude that even if a third party is infringing our issued patent, any patents that may be issued as a result of our pending or future patent applications or other intellectual property rights, the risk-adjusted cost of bringing and enforcing such a claim or action may be too high or not in the best interest of our company or our stockholders. In such cases, we may decide that the more prudent course of action is to simply monitor the situation or initiate or seek some other non-litigious action or solution.

If we choose to go to court to stop another party from using the inventions claimed in our patents, that individual or company has the right to ask the court to rule that such patents are invalid, unenforceable, or should not be enforced against that third party for any number of reasons. In patent litigation in the U.S., defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge include an alleged failure to meet any of several statutory requirements for patentability, including lack of novelty, obviousness, lack of written description, indefiniteness, or non-enablement. Grounds for an unenforceability assertion could include an allegation that someone connected with prosecution of the patent withheld material information from the USPTO or made a misleading statement during prosecution, i.e. committed inequitable conduct. Third parties may also raise similar claims before the USPTO, even outside the context of litigation. Similar mechanisms for challenging the validity and enforceability of a patent exist in foreign patent offices and courts and may result in the revocation, cancellation, or amendment of any foreign patents we or our licensors hold now or in the future. The outcome following legal assertions of invalidity and unenforceability is unpredictable, and prior art could render our patents or those of our licensors invalid. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on such product or product candidate. Such a loss of patent protection would have a material adverse impact on our business.

Interference or derivation proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms or at all, or if a non-exclusive license is offered and our competitors gain access to the same technology. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties or enter into development or manufacturing partnerships that would help us bring our product candidates to market.

Even if resolved in our favor, litigation or other legal proceedings relating to our intellectual property rights may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

Our ability to enforce our patent rights depends on our ability to detect infringement. It may be difficult to detect infringers who do not advertise the components or methods that are used in connection with their products and services. Moreover, it may be difficult or impossible to obtain evidence of infringement in a competitor's or potential competitor's product or service. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded if we were to prevail may not be commercially meaningful.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed. Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

We rely on trade secrets to protect our proprietary technologies, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect. We rely in part on confidentiality agreements with our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors, and inventions agreements with employees, consultants and advisors, to protect our trade secrets and other proprietary information. In addition to contractual measures, we try to protect the confidential nature of our proprietary information using commonly accepted physical and technological security measures. Despite these efforts, we cannot provide any assurances that all such agreements have been duly executed, and these agreements may not effectively prevent disclosure of confidential information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its Transparency Initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all. Costly and time-consuming litigation could be necessary to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

In addition, such security measures may not provide adequate protection for our proprietary information, for example, in the case of misappropriation of a trade secret by an employee, consultant, customer or third party with authorized access. Our security measures may not prevent an employee, consultant or customer from misappropriating our trade secrets and providing them to a competitor, and recourse we take against such misconduct may not provide an adequate remedy to protect our interests fully. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. Unauthorized parties may also attempt to copy or reverse engineer certain aspects of our approved medicines and any then-approved product that we consider proprietary. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret can be difficult, expensive and time-consuming, and the outcome is unpredictable. Even though we use commonly accepted security measures, the criteria for protection of trade secrets can vary among different jurisdictions.

Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the U.S. are less willing or unwilling to protect trade secrets. Moreover, third parties may still obtain this information or may come upon this or similar information independently, and we would have no right to prevent them from using that technology or information to compete with us. Trade secrets may 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 company to company or academic to industry scientific positions. Though our agreements with third parties typically restrict the ability of our advisors, employees, collaborators, licensors, suppliers, third-party contractors and consultants to publish data potentially relating to our trade secrets, our agreements may contain certain limited publication rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position. Because from time to time we expect to rely on third parties in the development, manufacture, and distribution of our approved medicines and any then-approved product and provision of our services, we must, at times, share trade secrets with them. Despite employing the contractual and other security precautions described above, the need to share trade secrets increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. If any of these events occur or if we otherwise lose protection for our trade secrets, the value of this information may be greatly reduced and our competitive position would be harmed. If we do not apply for patent protection prior to such publication or if we cannot otherwise maintain the confidentiality of our proprietary technology and other confidential information, then our ability to obtain patent protection or to protect our trade secret information may be jeopardized.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Our current or future trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. We have and may continue to license our trademarks and trade names to third parties, such as distributors. Though these license agreements may provide guidelines for how our trademarks and trade names may be used, a breach of these agreements or misuse of our trademarks and tradenames by our licensees may jeopardize our rights in or diminish the goodwill associated with our trademarks and trade names. Our efforts to enforce or protect our proprietary rights related to trademarks, trade names, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely affect our financial condition or results of operations.

Moreover, any name we have proposed to use with our product or product candidates in the U.S. must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark.

The FDA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA (or an equivalent administrative body in a foreign jurisdiction) objects to any of our proposed proprietary product names, we may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark or regulatory laws, not infringe the existing rights of third parties and be acceptable to the relevant administrative body. Furthermore, in many jurisdictions, owning and maintaining a trademark registration may not provide an adequate defense against a subsequent infringement claim asserted by the owner of a senior trademark. At times, competitors or other third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. If we assert trademark infringement claims, a court may also determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights denying our claim. In this case, we could ultimately be forced to cease use of such trademarks. Similar requirements exist in most jurisdictions worldwide.

Risks Related to Ownership of Our Common Stock

The trading price of our common stock may be volatile, and you could lose all or part of your investment.

The trading price of our common stock is likely to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. For example, the closing price of our common stock since January 1, 2025 to February 24, 2026 has ranged from a low of $38.39 to a high of $107.48. In addition to the factors discussed in this "Risk Factors" section, these factors include, among others:

- the degree of physician and patient adoption of our approved medicines and use of our approved medicines necessary for commercial success;
- our failure to grow and maintain our own sales force to market our approved medicines;
- our ability to market and sell our approved medicines, where approved;
- our ability to scale our distribution capabilities;
- any delay in our regulatory filings for our product candidates and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such filings, including without limitation the FDA's issuance of a "refusal to file" letter or a request for additional information;
- our failure to commercialize our product candidates;
- the commencement, enrollment or results of our ongoing clinical trials of our product candidates or any future clinical trials we may conduct, or changes in the development status of our product candidates;

- adverse results or delays in clinical trials;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- our ability to successfully integrate Bluejay into our business;
- adverse regulatory decisions, including failure to receive regulatory approval for our product candidates;
- changes in laws or regulations applicable to our approved medicines and our product candidates, including but not limited to clinical trial requirements for approvals;
- changes in the structure of health care payment systems;
- the failure to obtain coverage and adequate reimbursement of our approved medicines and our product candidates, if approved;
- adverse developments concerning our manufacturers;
- our inability to obtain adequate product supply for any approved drug product or inability to do so at acceptable prices;
- our inability to maintain or establish collaborations if needed;
- our ability to in-license, acquire, develop and market additional product candidates or approved medicines;
- management transitions and additions or departures of key scientific or management personnel;
- unanticipated serious safety concerns related to the use of our approved medicines or our product candidates;
- introduction of new products or services offered by us or our competitors;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- our ability to effectively manage our growth;
- the size and growth, if any, of the markets for our approved medicines with approved indications;
- our ability to successfully enter new markets or develop additional product candidates;
- actual or anticipated variations in quarterly operating results;
- our cash position;
- our failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;
- publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- changes in the market valuations of similar companies;
- overall performance of the equity markets;
- issuances of debt or equity securities;
- sales of our common stock by us or our stockholders in the future;
- trading volume of our common stock;
- changes in accounting practices;
- ineffectiveness of our internal controls;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- geopolitical and macroeconomic developments, including the ongoing military conflicts, economic slowdowns, recessions, inflation, tariffs and trade tensions, the ongoing shutdown of the federal government and the resulting effects on its regulatory agencies, bank failures, high interest rates and tightening of credit markets; and

- other events or factors, many of which are beyond our control.

Volatility in the trading price of our common stock could also prohibit or delay us from executing on our strategy, including in-licensing or acquiring additional product candidates or approved medicines using our common stock as consideration or raising additional capital on favorable terms or at all, any of which could exacerbate the volatility of the trading price of our common stock. In addition, the stock market in general, and Nasdaq-listed and biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. In the past, securities class action litigation has often been instituted against companies following periods of volatility in the market price of a company's securities. This type of litigation, if instituted, could result in substantial costs and a diversion of management's attention and resources, which would harm our business, operating results or financial condition.

We do not intend to pay dividends on our common stock so any returns will be limited to the value of our stock.

We have never declared or paid any cash dividend on our common stock. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will therefore be limited to the appreciation of their stock.

Our principal stockholders and management own a significant percentage of our stock and are able to exert significant control over matters subject to stockholder approval.

Our executive officers and directors, combined with our stockholders who own more than 5% of our outstanding capital stock, beneficially own shares representing a significant percentage of our common stock. Therefore, these stockholders have the ability to influence us through this ownership position. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

Future sales and issuances of our common stock or rights to purchase common stock could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

We expect that significant additional capital may be needed in the future to continue our planned operations, including conducting clinical trials, commercialization efforts, expanded research and development activities and costs associated with operating a public company, including costs resulting from our no longer qualifying as an emerging growth company and a smaller reporting company and becoming a large accelerated filer. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time, including through our automatic shelf registration statement on Form S-3 filed with the SEC in September 2025. For example, in November 2023, we entered into the 2023 Sales Agreement, pursuant to which we may elect to issue and sell, from time to time, shares of common stock having an aggregate offering price of up to $200.0 million through the sales agents. The remaining capacity under the 2023 Sales Agreement was $200.0 million as of December 31, 2025. Further, in connection with and immediately prior to the closing of the Bile Acid Portfolio Acquisition, we completed a private placement of our common stock, pursuant to which we issued 8,000,000 shares of our common stock, and we filed a registration statement registering 7,937,448 of these shares for resale. In January and February 2026, we issued 4,673,597 shares of our common stock in connection with the closing of the Bluejay Acquisition, subject in certain cases to deduction to satisfy applicable taxes, and may issue up to 522,375 shares of our common stock in the future subject to certain conditions. Also, immediately following the closing of the Bluejay Acquisition, we issued and sold in two private placement offerings an aggregate of (i) 3,385,149 shares of our commons stock and (ii) pre-funded warrants to purchase 536,412 shares of our common stock, for aggregate gross proceeds of approximately $268.5 million. We committed to register for resale all the shares of our common stock (or, in the case of the pre-funded warrants, all of the shares of our common stock issuable upon exercise of such pre-funded warrants) that we issued in connection with the Bluejay Acquisition and the private placement offerings immediately following the closing of the Bluejay Acquisition. If these additional shares of common stock are resold, or if it is perceived that they will be resold, in the public market, the trading price of our common stock could decline. Subject to the limitations on our ability to sell common stock described above, if we sell common stock, convertible securities or other equity securities, investors may be materially diluted by

subsequent sales. Such sales may also result in material dilution to our existing stockholders, including noteholders who have received shares of our common stock upon conversion of their notes, and new investors could gain rights, preferences and privileges senior to the holders of our common stock.

Pursuant to our 2019 Equity Incentive Plan ("2019 Plan"), our management is authorized to grant equity incentive awards to our employees, directors and consultants. We also maintain a 2019 Employee Stock Purchase Plan ("ESPP") pursuant to which our management is authorized to grant options to purchase shares of our common stock to our employees. In addition, pursuant to our 2020 Inducement Plan, our board of directors, or a committee thereof, is authorized to grant inducement awards to new hires as a material inducement to their employment with us.

Additionally, the number of shares of our common stock reserved for issuance under our 2019 Plan is subject to an automatic increase on January 1 of each year through and including January 1, 2029, by 5.0% of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by our board of directors. The number of shares of our common stock reserved for issuance under our ESPP is subject to an automatic increase on January 1 of each year through and including January 1, 2029, by the lesser of (i) 1.0% of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, and (ii) 1,500,000 shares of common stock. Unless our board of directors elects not to increase the number of shares available for future grant each year, our stockholders may experience additional dilution, which could cause our stock price to fall. Shares of common stock that are either subject to outstanding options or reserved for future issuance under our employee benefit plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, Rule 144 and Rule 701 under the Securities Act of 1933, as amended (the "Securities Act"). If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

Further, certain holders of our common stock are entitled to rights with respect to the registration of their shares under the Securities Act. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares held by affiliates, as defined in Rule 144 under the Securities Act. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

Our business could be negatively affected as a result of actions by activist stockholders, and such activism could impact the trading value of our securities.

Stockholders may, from time to time, engage in proxy solicitations or advance stockholder proposals, or otherwise attempt to effect changes and assert influence on our board of directors and management. Activist campaigns that contest or conflict with our strategic direction or seek changes in the composition of our board of directors could have an adverse effect on our operating results and financial condition. A proxy contest would require us to incur significant legal and advisory fees, proxy solicitation expenses and administrative and associated costs and require significant time and attention by our board of directors and management, diverting their attention from the pursuit of our business strategy. Any perceived uncertainties as to our future direction and control, our ability to execute on our strategy, or changes to the composition of our board of directors or senior management team arising from a proxy contest could lead to the perception of a change in the direction of our business or instability which may result in the loss of potential business opportunities, make it more difficult to pursue our strategic initiatives, or limit our ability to attract and retain qualified personnel and business partners, any of which could adversely affect our business and operating results. If individuals are ultimately elected to our board of directors with a specific agenda, it may adversely affect our ability to effectively implement our business strategy and create additional value for our stockholders. We may choose to initiate, or may become subject to, litigation as a result of the proxy contest or matters arising from the proxy contest, which would serve as a further distraction to our board of directors and management and would require us to incur significant additional costs. In addition, actions such as those described above could cause significant fluctuations in our stock price based upon temporary or speculative market perceptions or other factors that do not necessarily reflect the underlying fundamentals and prospects of our business.

Our failure to meet Nasdaq's continued listing requirements could result in a delisting of our common stock.

If we fail to satisfy the continued listing requirements of Nasdaq, such as the corporate governance requirements or the minimum closing bid price requirement, Nasdaq may take steps to delist our common stock. Such a delisting would likely have a negative effect on the price of our common stock and would impair your ability to sell or purchase our common stock when you wish to do so. In the event of an actual or threatened delisting, we may take actions in an attempt to restore compliance with listing requirements, but any such actions may not allow our common stock to become listed again, stabilize the market price or improve the liquidity of our common stock, prevent our common stock from dropping

below the Nasdaq minimum bid price requirement or prevent future non-compliance with the listing requirements of Nasdaq.

Anti-takeover provisions under our charter documents and Delaware law could delay or prevent a change of control, which could limit the market price of our common stock and may prevent or frustrate attempts by our stockholders to replace or remove our current management.

Our amended and restated certificate of incorporation and amended and restated bylaws contain provisions that could delay or prevent a change of control of our company or changes in our board of directors that our stockholders might consider favorable. Some of these provisions include:

- a board of directors divided into three classes serving staggered three-year terms, such that not all members of the board will be elected at one time;
- a prohibition on stockholder action through written consent, which requires that all stockholder actions be taken at a meeting of our stockholders;
- a requirement that special meetings of stockholders be called only by the chairman of the board of directors, the chief executive officer or by a majority of the total number of authorized directors;
- advance notice requirements for stockholder proposals and nominations for election to our board of directors;
- a requirement that no member of our board of directors may be removed from office by our stockholders except for cause and, in addition to any other vote required by law, upon the approval of not less than two-thirds of all outstanding shares of our voting stock then entitled to vote in the election of directors;
- a requirement of approval of not less than two-thirds of all outstanding shares of our voting stock then entitled to vote in the election of directors to amend our amended and restated bylaws by stockholder action or to amend specific provisions of our amended and restated certificate of incorporation; and
- the authority of the board of directors to issue preferred stock on terms determined by the board of directors without stockholder approval and which preferred stock may include rights superior to the rights of the holders of common stock.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the General Corporation Law of the State of Delaware ("DGCL"), which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These anti-takeover provisions and other provisions in our amended and restated certificate of incorporation and amended and restated bylaws could make it more difficult for stockholders or potential acquirors to obtain control of our board of directors or initiate actions that are opposed by the then-current board of directors and could also delay or impede a merger, tender offer or proxy contest involving our company. These provisions could also discourage proxy contests and make it more difficult for you and other stockholders to elect directors of your choosing or cause us to take other corporate actions you desire. Any delay or prevention of a change of control transaction or changes in our board of directors could cause the market price of our common stock to decline.

In addition, certain provisions in the Notes and the related Indenture could make a third party's attempt to acquire us more difficult or expensive. For example, if a takeover constitutes a fundamental change under our Indenture, then noteholders will have the right to require us to repurchase their Notes for cash. In addition, if a takeover constitutes a make-whole fundamental change under our Indenture, then we may be required to temporarily increase the conversion rate. In either case, and in other cases, our obligations under the Notes and the Indenture could increase the cost of acquiring us or otherwise discourage a third party from acquiring us or removing incumbent management, including in a transaction that noteholders or holders of our common stock may view as favorable.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that the Court of Chancery of the State of Delaware and the federal district courts of the U.S. will be the exclusive forums for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: (i) any derivative action or proceeding brought on our behalf; (ii) any action or proceeding asserting a claim of breach of a fiduciary duty owed by any of our current or former directors, officers or other employees to us or our stockholders; (iii)

any action or proceeding asserting a claim against us or any of our current or former directors, officers or other employees, arising out of or pursuant to any provision of the DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws; (iv) any action or proceeding to interpret, apply, enforce or determine the validity of our amended and restated certificate of incorporation or our amended and restated bylaws; (v) any action or proceeding as to which the DGCL confers jurisdiction to the Court of Chancery of the State of Delaware; and (vi) any action asserting a claim against us or any of our directors, officers or other employees governed by the internal affairs doctrine, in all cases to the fullest extent permitted by law and subject to the court's having personal jurisdiction over the indispensable parties named as defendants. These provisions would not apply to suits brought to enforce a duty or liability created by the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or any other claim for which the federal courts have exclusive jurisdiction. Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our amended and restated certificate of incorporation and amended and restated bylaws further provides that the federal district courts of the U.S. will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act. While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our amended and restated certificate of incorporation and amended and restated bylaws. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions.

These exclusive-forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees and may discourage these types of lawsuits. If a court were to find either exclusive-forum provision in our amended and restated certificate of incorporation or amended and restated bylaws to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving the dispute in other jurisdictions, all of which could seriously harm our business.

General Risk Factors

Unfavorable geopolitical and macroeconomic developments could adversely affect our business, financial condition or results of operations.

Our business could be adversely affected by conditions in the U.S. and global economies, the U.S. and global financial markets and adverse geopolitical and macroeconomic developments, including potential future disruptions in access to bank deposits or lending commitments due to bank failures, tariffs and trade tensions, the ongoing shutdown of the federal government and the resulting effects on its regulatory agencies, geopolitical tensions and military conflicts, such as the ongoing conflicts between Ukraine and Russia and in the Middle East, and increasing tensions between the U.S. and China. The effects caused by these factors could be exacerbated by any related political or economic responses and counter-responses or otherwise by various global actors or the general effect on the global economy and supply chain. General business and economic conditions that could affect our business, financial condition or results of operations include fluctuations in economic growth, inflation and interest rates, debt and equity capital markets, liquidity of the global financial markets, the availability and cost of credit, investor and consumer confidence, and the strength of the economies in which we, our manufacturers, suppliers and other collaborators operate. A weak or declining global economy could also strain our suppliers and manufacturers, possibly resulting in supply disruption. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

If our information technology systems, or those used by our CMOs, CROs, commercial vendors or other contractors, consultants or third parties with whom we work, or our data are or were compromised, we could experience material adverse consequences, including but not limited to regulatory investigation, actions, litigation, fines and penalties, disruptions of our business operations, reputation harm, loss of revenue or profits, and other adverse consequences.

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

The sensitive information processed and stored in our technology systems, and those of our research collaborators, CROs, contractors, consultants and other third parties with whom we work, may be vulnerable to cyberattacks, malicious internet-based activity, online and offline fraud and other similar activities. These threats are prevalent and continue to rise, are increasingly difficult to detect, and come from a variety of sources, including traditional

computer "hackers," threat actors, "hacktivists," organized criminal threat actors, personnel (such as through theft or misuse), 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 cyberattacks that could materially disrupt our systems and operations, supply chain, and ability to produce, sell and distribute our goods and services. We and the third parties with whom we work may be subject to a variety of threats, including but not limited to errors or malfeasance by our personnel or the personnel of the third parties, malware (including as a result of advanced persistent threat intrusions), malicious code (such as viruses and worms), software vulnerabilities, hacking, denial of service attacks, credential stuffing, social engineering (including through deep fakes, which may be increasingly more difficult to identify as fake, and phishing attacks), ransomware, supply-chain attacks, server malfunctions, software or hardware failure, loss of data or other information technology assets, adware, telecommunications failures, attacks enhanced or facilitated by artificial intelligence (AI) and other similar threats. Threat actors are continuing to develop and use more sophisticated tools and techniques (including AI) that are specifically designed to circumvent security controls, evade detection, and obfuscate forensic evidence, making it more difficult for us to identify, investigate and recover from incidents. Ransomware attacks, including by organized criminal threat actors, nation-states, and nation-state-supported actors, are becoming increasingly prevalent and severe and can lead to significant interruptions in our operations, loss of data and income, reputational harm, and diversion of funds. Extortion payments may alleviate the negative impact of a ransomware attack, but we may be unwilling or unable to make such payments due to, for example, applicable laws or regulations prohibiting such payments. 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 work with third parties and technologies that operate critical business systems to process sensitive information in a variety of contexts, including, without limitation, third-party providers of cloud-based infrastructure, encryption and authentication technology, employee communications, and other functions. Likewise, we work with third-party research institution collaborators, CMOs, CROs, other contractors and consultants for many aspects of our business, including research and development activities and manufacturing of our approved medicines and our product candidates, and similar events relating to their computer systems or data could also have a material adverse effect on our business. 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. If the third parties with whom we work experience a security incident or other interruption, we could experience adverse consequences. 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. Similarly, supply-chain attacks have increased in frequency and severity, and we cannot guarantee that third parties and infrastructure in our supply chain or the supply chains of the third parties with whom we work have not been compromised.

While we have implemented information security measures designed to protect against security incidents, we cannot assure you that our (or the third parties with whom we work) security measures will be effective. It may be difficult and/or costly to detect, investigate, mitigate, contain, and remediate a security incident. Our efforts to do so may not be successful. Actions taken by us or the third parties with whom we work to detect, investigate, mitigate, contain, and remediate a security incident could result in outages, data losses, and disruptions of our business. Threat actors may also gain access to other networks and systems after a compromise of our networks and systems. For example, threat actors may use an initial compromise of one part of our environment to gain access to other parts of our environment, or leverage a compromise of our networks or systems to gain access to the networks or systems of third parties with whom we work, such as through phishing or supply chain attacks.

Certain of the previously identified or similar threats have in the past and could 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, in February through March of 2024, along with many others in our industry, we became aware of a security incident at Change Healthcare which impacted the ability of patient claims to be adjudicated and patient prescriptions to be filled. Our internal team had to work closely with our

specialty pharmacy and logistic providers to assess and resolve the matter. A security incident or other interruption could disrupt our ability (and that of third parties with whom we work) to provide our approved medicines and services.

We may expend significant resources or modify our business activities (including our clinical trial activities) to try to protect against security incidents. Certain data privacy and security obligations require us to implement and maintain specific security measures, industry-standard or reasonable security measures to protect our information technology systems and sensitive information. Applicable data privacy and security obligations may require us, or we may choose, to notify relevant stakeholders, including affected individuals, customers, regulators, and investors, of security incidents, or to take other actions, such as providing credit monitoring and identity theft protection services. Such disclosures and compliance with applicable requirements are costly, and the disclosure or the failure to comply with such requirements could lead to adverse consequences. If a material security incident was to occur, or we (or a third party with whom we work) are perceived to have experienced such an event, we may experience adverse consequences. These consequences may include: government enforcement actions (for example, investigations, fines, penalties, audits, and inspections), additional reporting requirements and/or oversight, restrictions on processing sensitive information (including personal data), litigation (including class claims), indemnification obligations, negative publicity, reputational harm, monetary fund diversions, interruptions in our operations (including availability of data), financial loss, and other similar harms. Security incidents and attendant consequences may negatively impact our ability to grow and operate our business. More specifically, for example, the loss of clinical trial data from completed, ongoing or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Security incidents and any unauthorized access or disclosure of our sensitive information could also compromise our intellectual property and patent portfolio, expose sensitive business information, expose the personal data of our employees, require us to incur significant remediation costs, disrupt key business operations and divert attention of management and key information technology resources.

We cannot be sure that our insurance coverage will be adequate or sufficient to protect us from or to mitigate liabilities arising out of our privacy and cybersecurity practices, that such coverage will continue to be available to us on commercially reasonable terms, or at all, or that such coverage will pay future claims. The successful assertion of one or more large claims against us that exceed available insurance coverage, or the occurrence of changes in our insurance policies, including premium increases or the imposition of large deductible or co-insurance requirements, could adversely affect our reputation, business, financial condition and results of operations. Additionally, our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our data privacy and security obligations.

In addition 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.

We are subject to U.S. and certain foreign export and import controls, sanctions, embargoes, anti-corruption laws and anti-money laundering laws and regulations. Compliance with these legal standards could impair our ability to compete in domestic and international markets. We can face criminal liability and other serious consequences for violations, which can harm our business.

We are subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations, and various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls, and anti-corruption and anti-money laundering laws and regulations, including the FCPA, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, clinical research organizations, contractors and other collaborators and partners from authorizing, promising, offering, providing, soliciting or receiving, directly or indirectly, improper payments or anything else of value to recipients in the public or private sector. We may engage third parties for clinical trials outside of the U.S., to sell our approved medicines and any then-approved product internationally once we enter a commercialization phase, and/or to obtain necessary permits, licenses, patent registrations and other regulatory approvals. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities and other organizations. We can be held liable for the corrupt or other illegal activities of our employees, agents, clinical research organizations, contractors and other collaborators and partners, even if we do not explicitly authorize or have actual knowledge of such activities. Any violations of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm and other consequences.

If we or our third-party manufacturers use hazardous and biological materials in a manner that causes injury or violates applicable law, we may be liable for damages.

Our research and development activities involve the controlled use of potentially hazardous substances, including chemical and biological materials, by our third-party manufacturers. Our manufacturers are subject to federal, state and local laws and regulations in the U.S. governing the use, manufacture, storage, handling and disposal of medical, radioactive and hazardous materials. Although we believe that our manufacturers' procedures for using, handling, storing and disposing of these materials comply with legally prescribed standards, we cannot completely eliminate the risk of contamination or injury resulting from medical, radioactive or hazardous materials. As a result of any such contamination or injury, we may incur liability or local, city, state or federal authorities may curtail the use of these materials and interrupt our business operations. In the event of an accident, we could be held liable for damages or penalized with fines, and the liability could exceed our resources. We do not have any insurance for liabilities arising from medical radioactive or hazardous materials. Compliance with applicable environmental laws and regulations is expensive, and current or future environmental regulations may impair our research, development and production efforts, which could harm our business, prospects, financial condition or results of operations.

We have identified material weaknesses in our internal control over financial reporting in the past. If we identify additional material weaknesses in the future or otherwise fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired, which could adversely impact our investors' confidence in our financial reports and our stock price could be adversely affected.

We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act and the rules and regulations of Nasdaq. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal controls over financial reporting. Each fiscal year, we must perform system and process evaluation and testing of our internal controls over financial reporting to allow management to report on the effectiveness of our internal controls over financial reporting in our Form 10-K filing for that year, as required by Section 404 of the Sarbanes-Oxley Act. This requires that we incur substantial professional fees and internal costs related to our accounting and finance functions and that we expend significant management efforts.

In connection with the audit of our consolidated financial statements as of and for the year ended December 31, 2023, management identified material weaknesses in the design of controls and level of evidence retained over the existence and valuation of inventory, including the controls over existence of inventory located at third parties and the net realizable value assessment of on-hand inventory and future purchases under firm commitments, and over the precision of management review controls and the sufficiency of control evidence related to prospective financial information used to determine the fair value of acquired developed technology. A material weakness is a deficiency, or 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.

Although we have been and are taking steps to improve our internal control over financial reporting and remediated these material weaknesses, the measures we have taken to date may not be sufficient to avoid potential future material weaknesses. Additionally, a control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we identify new material weaknesses in our internal control over financial reporting, if we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner, if we are unable to maintain proper and effective internal controls, or if our independent registered public accounting firm is unable to express an opinion that our internal control over financial reporting is effective in future periods, we may not be able to produce timely and accurate financial statements and investors may lose confidence in the accuracy and completeness of our financial reports. If that were to happen, the market price of our stock could decline and we could be subject to sanctions or investigations by Nasdaq, the SEC or comparable foreign regulatory authorities.

We have incurred and will continue to incur significant increased costs to comply with changing laws, rules, regulations and standards relating to various aspects of our business, including corporate governance, workforce initiatives and public disclosure, and failure to comply with such laws, rules, regulations and standards could adversely affect our business.

We have incurred and will continue to incur significant legal, accounting and other expenses to comply with changing laws, rules, regulations and standards relating to various aspects of our business, including corporate governance, workforce initiatives and public disclosure. Sources of these changing laws, rules, regulations and standards include new

SEC regulations, Nasdaq rules and executive orders, which are creating uncertainty for companies such as ours. These laws, rules, regulations and standards are subject to varying interpretations in some cases due to their lack of specificity, and as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies, which could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure, policies and governance practices. For example, we are subject to the reporting requirements of the Exchange Act, which require, among other things, that we file with the SEC annual, quarterly and current reports with respect to our business and financial condition. In addition, the Sarbanes-Oxley Act, as well as rules subsequently adopted by the SEC and Nasdaq to implement provisions of the Sarbanes-Oxley Act, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and changes in corporate governance practices. Further, in July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act (the "Dodd-Frank Act") was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as "say on pay" and proxy access. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new laws, rules, regulations and standards. If we fail, or are perceived to fail, to comply with these laws, rules, regulations and standards, our reputation may be harmed and we might be subject to litigation, sanctions, investigations or other regulatory proceedings by regulatory authorities, such as the SEC. Any such action could adversely affect our financial results and the market price of our common stock.

We expect the laws, rules, regulations and standards applicable to companies, especially public companies, to continue to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. If these requirements divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition and results of operations. The increased costs will decrease our net income or increase our consolidated net loss and may require us to reduce costs in other areas of our business or increase the prices of our approved medicines and any then-approved product or services. For example, we expect these laws, rules, regulations and standards to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain the same or similar coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

If securities or industry analysts publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our common stock depends in part on the research and reports that securities or industry analysts publish about us or our business. If one or more of the analysts who covers us no longer covers us, downgrades our stock or publishes inaccurate or unfavorable research about our business, our stock price may decline. If one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

Item 1B. Unresolved Staff Comments.

Not applicable.

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 our clinical trials and approved medications ("Information Systems and Data").

Our information security team, supported by our legal and compliance departments, help identify, assess and manage our cybersecurity threats and risks. Our information security team identifies and assesses risks from cybersecurity threats by monitoring and evaluating our threat environment using various methods, depending on the context, including, for example manual and automated tools, subscribing to reports and services that identify cybersecurity threats, analyzing certain reports of threats and threat actors, conducting scans of certain environments, evaluating our and our industry's risk profile, evaluating certain threats reported to us, conducting third-party threat assessments, coordinating with law

enforcement about certain threats, conducting internal audits and threat assessments, and conducting vulnerability assessments.

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 detection and response procedures and policies; disaster recovery/business continuity plans; risk assessments; implementation of certain security controls; encryption of certain data; network security, access, and physical controls for certain systems; management, tracking and disposal of certain assets; systems monitoring; employee training; penetration tests by third-party service providers; tabletop exercises conducted by third parties; and cybersecurity insurance.

Our assessment and management of material risks from cybersecurity threats are integrated into our overall risk management processes. For example, the information security team works with management to prioritize our risk management processes and mitigate cybersecurity threats that are more likely to lead to a material impact to our business and our senior management evaluates material risks from cybersecurity threats against our overall business objectives and reports to the audit committee of the board of directors, which evaluates our overall enterprise risk.

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 professional service firms such as legal counsel, threat intelligence providers, cybersecurity consultants and software providers, penetration testing services, and other third-party service providers including for identity management solutions.

We use third-party service providers to perform a variety of functions throughout our business application providers, hosting companies, contract research organizations ("CROs"), contract manufacturing organizations ("CMOs"), supply chain resources, and other business-related SaaS platforms. We have a vendor management program to manage cybersecurity risks associated with our use of certain of these providers. The program includes conducting risk assessments of certain vendors, reviewing certain vendors' security assessments, reviewing reports of certain of our vendors' security, and reviewing the written information security program of certain vendors. Depending on the nature of the services provided, the sensitivity of the Information Systems and Data at issue, and the identity of the provider, our vendor management process may involve different levels of assessment designed to help identify cybersecurity risks associated with a provider and impose contractual obligations related to cybersecurity on 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 I., Item 1A, "Risk Factors" in this Annual Report on Form 10-K, including "*Our business is subject to complex, stringent and evolving U.S. and foreign laws, regulations, and rules, contractual obligations, industry standards, policies and other obligations relating to privacy and data protection. Our actual or perceived failure to comply with such obligations could result in regulatory investigations or actions, litigations (including class claims) and mass arbitration demands, fines and penalties, disruptions of and changes to our business practices, monetary penalties, reputational harm, loss of revenue or profits, and other adverse business consequences.*"; and "*If our information technology systems, or those used by our CMOs, CROs, commercial vendors or other contractors, consultants or third parties with whom we work, or our data are or were compromised, we could experience material adverse consequences, including but not limited to regulatory investigation, actions, litigation, fines and penalties, disruptions of our business operations, reputation harm, loss of revenue or profits, and other adverse consequences.*"

Governance

Our board of directors addresses our cybersecurity risk management as part of its general oversight function. The board of directors' audit committee is responsible for overseeing our cybersecurity risk management processes, including oversight of mitigation of risks from cybersecurity threats.

Our cybersecurity risk assessment and management processes are implemented and maintained by certain Company management, including our Vice President of Information Technology and Vice President, Legal. Our Vice President of Information Technology has over fifteen years of experience in information technology and security, and previously held positions such as Vice President, Business Information Technology Strategy and Application Management, Vice President, Head of Information Technology, Executive Director, Information Technology, and similar roles at other companies.

Our Vice President of Information Technology is responsible for hiring appropriate personnel, helping to integrate cybersecurity risk considerations into the Company's overall risk management strategy, helping prepare for cybersecurity incidents, approving cybersecurity processes, reviewing security assessments and other security-related reports, and communicating key priorities to relevant personnel. Our CLO is responsible for legal and compliance risk, including related to cybersecurity.

Our incident response and vulnerability management processes are designed to escalate certain cybersecurity incidents to members of management depending on the circumstances, including the Vice President of Information Technology, Data Protection Officer, and the Vice President, Legal. These individuals work with our incident response team to help us mitigate and remediate cybersecurity incidents of which they are notified. In addition, our incident response processes include reporting to the audit committee of the board of directors for certain cybersecurity incidents.

The audit committee of the board of directors receives annual reports from our Vice President of Information Technology concerning our significant cybersecurity threats and risk and the processes we have implemented to address them. The audit committee of the board of directors also receives and has access to various presentations related to cybersecurity threats, risk and mitigation.

Item 2. Properties.

We lease 36,318 square feet of space for our headquarters in Foster City, California under an agreement that was most recently amended in 2026. Under the amended lease, we will lease an additional 19,430 square feet for a total of 55,748 square feet with lease commencement for the additional space of the later of October 1, 2026 and fifteen days after the landlord's substantial completion of the required tenant improvements ("Lease Commencement"). The expiry of the amended lease is five years from Lease Commencement.

We also lease approximately 6,000 square feet of space for an office in Basel, Switzerland under an agreement that expires in January 2031. Additionally, we lease approximately 7,000 square feet of office space in Zug, Switzerland that expires in June 2030. We believe that our facilities are adequate to meet our current needs, and that suitable additional alternative spaces will be available in the future on commercially reasonable terms, if needed.

After the closing of the Bluejay Acquisition, we also lease 11,125 square feet of office space in Redwood City, California under a lease agreement that expires in November 2027.

Item 3. Legal Proceedings.

From time to time, we may become involved in legal proceedings relating to claims arising from the ordinary course of business. Our management believes that, other than as described below, there are currently no claims or actions pending against us, the ultimate disposition of which could have a material adverse effect on our results of operations, financial condition or cash flows.

On December 19, 2025, we, along with Satiogen Pharmaceuticals, Inc. and Shire Human Genetic Therapies, Inc. as co-plaintiffs, filed four complaints against Sandoz Inc. ("Sandoz"); Annora Pharma Private Limited, Hetero Labs Limited, and Hetero USA Inc. (together, "Hetero"); Zenara Pharma Private Limited and Biophore India Pharmaceuticals Private Limited (together, "Biophore"); and Zydus Lifesciences Global FZE, Zydus Lifesciences Limited, and Zydus Pharmaceuticals (USA) Inc. (together, "Zydus") (all collectively, "Defendants") in the U.S. District Court for the District of Delaware (the "LIVMARLI Patent Litigations"), alleging infringement of certain Orange Book listed patents covering LIVMARLI (the "LIVMARLI Patents"). The LIVMARLI Patent Litigations were initiated following the submission by Defendants, in accordance with the procedures set out in the Hatch-Waxman Act, of ANDAs directed to generic versions of LIVMARLI. Defendants' ANDAs seek approval to market generic versions of LIVMARLI prior to the expiration of the LIVMARLI Patents and allege that the LIVMARLI Patents are invalid, unenforceable, and/or not infringed. We are seeking, among other relief, an order that the effective date of any FDA approval of Defendants' ANDAs be no earlier than the expiration of the asserted patents listed in the Orange Book, and such further and other relief as the court may deem appropriate. The Defendants are subject to a 30-month stay of final regulatory approval through March 29, 2029, preventing them from marketing generic versions of LIVMARLI during that time. On February 20, 2026, Sandoz asserted counterclaims against us and our co-plaintiffs seeking declaratory judgments of non-infringement and invalidity with respect to certain LIVMARLI Patents. Trial in the LIVMARLI Patent Litigations has not yet been scheduled. We cannot make any predictions about the final outcome of these matters or the timing thereof.

Item 4. Mine Safety Disclosures.

None.

PART II

Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.

Market Information

Our common stock has been listed on the Nasdaq Global Market under the symbol "MIRM" since July 18, 2019. Prior to that date, there was no public market for our common stock.

Holders of Common Stock

As of February 20, 2026 there were 60,341,617 shares of our common stock outstanding held by approximately 91 holders of record. The actual number of stockholders is greater than this number because certain stockholders who are beneficial owners hold our common stock in "street" name with brokers and other nominees.

Dividend Policy

We have never declared or paid any cash dividends on our capital stock. We intend to retain future earnings, if any, to finance the operation of our business and do not anticipate paying any cash dividends in the foreseeable future. Any future determination related to dividend policy will be made at the discretion of our board of directors and will depend on then-existing conditions, including our financial condition, operating results, contractual restrictions, capital requirements, business prospects and other factors our board of directors may deem relevant.

Securities Authorized for Issuance Under Equity Compensation Plans

See Item 12 of Part III of this Annual Report on Form 10-K for information about our equity compensation plans which is incorporated by reference herein.

Stock Performance Graph

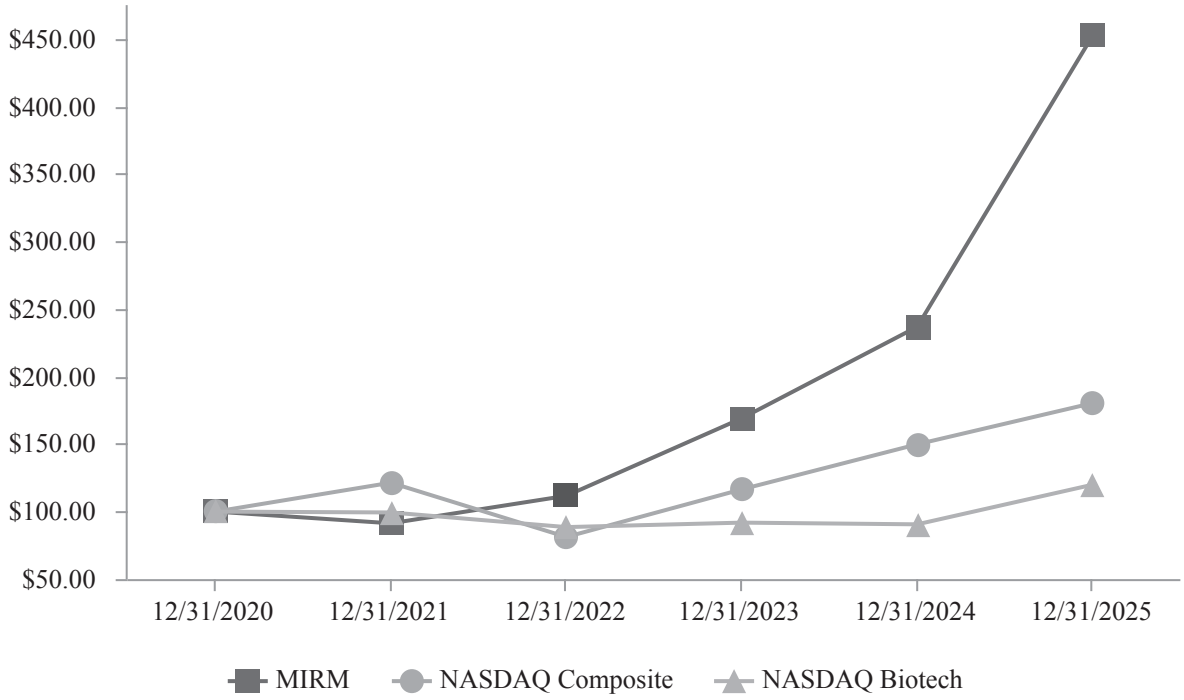
The following is not to be deemed "soliciting material" or to be "filed" with the SEC, is not subject to the liabilities of Section 18 of the Exchange Act and is not to be incorporated by reference into any filing we make under the Securities Act or the Exchange Act, whether made before or after the date hereof and irrespective of any general incorporation by reference language is such filing.

The following graph compares the cumulative total stockholder return on our common stock relative to the cumulative total returns of the Nasdaq Composite Index and the Nasdaq Biotechnology Index. An investment of $100 is assumed to have been made in our common stock and each index on December 31, 2020 and its relative performance is tracked through December 31, 2025. Pursuant to applicable SEC rules, all values assume a reinvestment of the full amount of all dividends, however no dividends have been declared on our common stock to date. The stockholder returns shown on

the graph below are based on historical results and are not necessarily indicative of future performance, and we do not make or endorse any predictions as to future stockholder returns.

COMPARISON OF CUMULATIVE TOTAL RETURN

among Mirum Pharmaceuticals, Inc., the NASDAQ Composite Index and the NASDAQ Biotech Index



$100 Investment in Stock or Index	12/31/2020	12/31/2021	12/31/2022	12/31/2023	12/31/2024	12/31/2025
Mirum Pharmaceuticals, Inc.	$ 100.00	$ 91.35	$ 111.68	$ 169.07	$ 236.83	$ 452.41
NASDAQ Composite Index	$ 100.00	$ 121.39	$ 81.21	$ 116.47	$ 149.83	$ 180.33
NASDAQ Biotechnology Index	$ 100.00	$ 99.37	$ 88.53	$ 91.84	$ 90.58	$ 119.92

Recent Sales of Unregistered Securities

There were no sales of equity securities during the period covered by this report that were not registered under the Securities Act and were not previously reported in a Quarterly Report on Form 10-Q or a Current Report on Form 8-K filed by the Company.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers

None.

Item 6. Reserved.

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and related notes included in Item 8 "Financial Statements and Supplementary Data" and included elsewhere in this Annual Report. This discussion and analysis contains forward-looking statements based upon our current beliefs, estimates, plans and expectations that involve risks, uncertainties and assumptions. Our actual results may differ materially from those contained in these forward-looking statements as a result of various factors, including those set forth under "Risk Factors" or in other parts of this Annual Report.

Overview

We are a biopharmaceutical company dedicated to transforming the treatment of rare diseases. We have three approved medicines: LIVMARLI® (maralixibat) ("Livmarli"), CHOLBAM® (cholic acid) capsules ("Cholbam"), and CTEXLI® (chenodiol) tablets ("Ctexli").

Livmarli is a novel, orally administered, minimally-absorbed ileal bile acid transporter ("IBAT") inhibitor ("IBATi") that is approved for the treatment of cholestatic pruritus in patients with Alagille syndrome ("ALGS") in the United States ("U.S."), the European Union ("EU") and various other countries around the world and for cholestatic pruritus in patients with progressive familial intrahepatic cholestasis ("PFIC") in the U.S., Canada and Japan and for the treatment of PFIC in the EU. We market and commercialize Livmarli in the U.S., Canada and certain countries in Europe through our specialized and focused commercial team. We have also entered into license and distribution agreements with several rare disease companies for the commercialization of Livmarli in additional countries. In March 2025, our partner Takeda received approval from the Japanese Ministry of Health, Labour, and Welfare for Livmarli for the treatment of cholestatic pruritus in patients with ALGS and PFIC.

In August 2023, we completed the acquisition of assets of Travere Therapeutics, Inc. ("Travere") that are primarily related to the development, manufacture (including synthesis, formulation, finishing or packaging) and commercialization of chenodiol and Cholbam (also known as Kolbam) (and together with chenodiol, the "Bile Acid Medicines") pursuant to an asset purchase agreement dated July 16, 2023 (such acquisition, the "Bile Acid Portfolio Acquisition").

The U.S. Food and Drug Administration ("FDA") approved Cholbam in March 2015, as the first FDA-approved treatment for pediatric and adult patients with bile acid synthesis disorders due to single enzyme defects, and for adjunctive treatment of patients with peroxisomal disorders, including peroxisome biogenesis disorder-Zellweger spectrum disorder ("PBD-ZSD"). Chenodiol is standard of care for the treatment of cerebrotendinous xanthomatosis ("CTX") in the U.S. with a medical necessity recognition by the FDA and was commercialized under the brand name Chenodal. We submitted a new drug application ("NDA") for chenodiol for the treatment of CTX in 2024 and received FDA approval for the treatment of adults with CTX in February 2025, which is commercialized under the brand name Ctexli. We currently market and commercialize Cholbam and Ctexli in the U.S. through our specialized and focused commercial team. We have also assumed license and distribution agreements with several rare disease companies for the commercialization of Cholbam and chenodiol in additional countries.

We are advancing our product candidate, volixibat, a novel, oral, minimally-absorbed agent designed to inhibit IBAT, for the treatment of adult patients with cholestatic liver diseases. We are developing volixibat in the setting of primary sclerosing cholangitis ("PSC") and primary biliary cholangitis ("PBC"), and in October 2024, we announced that the FDA granted Breakthrough Therapy designation for volixibat as a potential treatment for cholestatic pruritus in patients with PBC. We conducted an interim analysis of our VISTAS Phase 2b clinical trial in PSC and reported interim data from our VANTAGE Phase 2b clinical trial in PBC in June 2024. The VISTAS Phase 2b clinical trial in PSC completed enrollment in the third quarter of 2025 and topline data is expected in the second quarter of 2026. In addition, we expect to submit an NDA to the FDA for volixibat for the treatment of PSC in the second half of 2026, with both potential approval and subsequent launch of volixibat for the treatment of PSC, if approved, to occur in the first half of 2027. We expect the VANTAGE Phase 2b clinical trial in PBC to complete enrollment in the second half of 2026 with topline data expected in the first half of 2027.

In addition, we are developing Livmarli for certain rare cholestatic conditions through the Phase 3 EXPAND study, which we initiated in the fourth quarter of 2024. We expect to complete enrollment of the EXPAND study in the first half of 2026 with topline data expected in the fourth quarter of 2026.

In October 2024, we completed a license agreement with Enthorin Therapeutics, LLC and Dart Neuroscience LLC granting us the worldwide right to develop and commercialize MRM-3379, an allosteric inhibitor of Phosphodiesterase 4D ("PDE4D"). We are currently enrolling patients in the BLOOM Phase 2 clinical study of MRM-3379 in Fragile-X Syndrome ("FXS") and expect topline data in 2027.

On January 23, 2026, we completed the acquisition (the "Bluejay Acquisition") of Bluejay Therapeutics, Inc. ("Bluejay") and its lead product candidate brelovitug (BJT-778). We are advancing brelovitug for the treatment of chronic hepatitis D virus ("HDV") infection. Brelovitug is a fully human IgG1 monoclonal antibody that binds the hepatitis B surface antigen, thereby clearing virions and subviral particles and preventing HDV infection and replication. Brelovitug has been granted FDA Breakthrough Therapy designation, EMA Priority Medicines ("PRIME") scheme designation and European Commission orphan medicinal product designation. Brelovitug is currently being evaluated in the global AZURE clinical program with topline results from the AZURE-1 and AZURE-4 registration-enabling clinical trials expected in the second half of 2026 and top-line results from the AZURE-2 and AZURE-3 registration-enabling clinical trials expected by the first half of 2028. We believe that the results from the AZURE-1 and AZURE-4 trials may support a potential biologics license application submission to the FDA for brelovitug in HDV in the first half of 2027 followed by a potential approval and subsequent launch, if approved, in the second half of 2027. We believe that the results of the AZURE-2 and AZURE-3 clinical trials may support a potential EMA registration and subsequent commercial launch, if approved, in the EU.

To date, we have focused primarily on acquiring and in-licensing our product candidates, organizing and staffing our company, business planning, raising capital, advancing our product candidates through clinical development, preparing for commercialization of our product candidates, commercializing our approved medicines, and conducting business development activities relating to, among other things, portfolio expansion through collaborations and acquisitions.

Financial Overview

Our net loss was $23.4 million and $87.9 million for the years ended December 31, 2025 and 2024, respectively. As of December 31, 2025, we had an accumulated deficit of $667.5 million compared to $644.2 million as of December 31, 2024. As of December 31, 2025, we had unrestricted cash, cash equivalents and investments of $391.4 million, compared to $292.8 million as of December 31, 2024.

While we generated net income in the third quarter of 2025, we anticipate we will continue to generate net losses for the foreseeable future as we continue commercial activities for our approved medicines, conduct our ongoing and planned clinical trials, including the clinical trials for brelovitug, seek regulatory approvals for our product candidates and make potential milestone payments to the licensors and other third parties from whom we have in-licensed or acquired our product candidates. We expect that total product sales of our approved medicines will continue to increase on an annual basis; however, due to large periodic orders from Takeda and our distributors, our product revenue may experience quarterly fluctuations. Additionally, our product revenues from Takeda are based upon variable consideration estimates. If actual results vary from our estimates, we will make adjustments in the period when such variances become known. As a result, our net losses may fluctuate significantly from quarter-to-quarter and year-to-year.

We expect to satisfy future cash needs through existing capital balances, revenue from our approved medicines and through a combination of equity offerings, debt financings or other capital sources, collaborations, licenses and other similar arrangements. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Our failure to raise capital or enter into such other arrangements when needed could have a negative impact on our financial condition and on our ability to pursue our business plans and strategies. If we are unable to raise additional capital when needed, we could be forced to delay, limit, reduce or terminate the development of one or more of our product candidates or future commercialization efforts or grant rights to develop and market our product candidates even if we would otherwise prefer to develop and market such product candidates ourselves.

License agreement with Enthorin

In October 2024, we completed the in-license of MRM-3379. We paid a one-time non-refundable license fee of $7.5 million upon closing of the transaction, and up to an additional $217.5 million is payable upon achievement of certain development, regulatory and commercial milestones.

Asset Purchase Agreement with Travere Therapeutics, Inc.

In August 2023, we completed the Bile Acid Portfolio Acquisition. We paid $210.4 million upon closing of the transaction, and up to an additional $235.0 million is payable upon the achievement of certain milestones based on specified amounts of annual net sales of the Bile Acid Medicines. As of December 31, 2025, the Company accrued $25.0 million for the achievement of a commercial milestone associated with achievement of certain net product sales, which was recognized as an intangible asset in the accompanying consolidated balance sheet as of December 31, 2025 and is expected to be paid in the first quarter of 2026.

In connection with and immediately prior to the closing of the Bile Acid Portfolio Acquisition, we completed the private placement of 8,000,000 shares of our common stock at a price per share of $26.25, resulting in net proceeds of

approximately $202.2 million, which we used to finance the upfront payment at the closing of the Bile Acid Portfolio Acquisition.

Acquisition of Bluejay Therapeutics

On January 23, 2026, we completed the Bluejay Acquisition and acquired Bluejay's lead product candidate brelovitug (BJT-778). Upon the closing of the merger, we paid to the holders of Bluejay's securities an aggregate amount of $224.2 million in cash, net of cash acquired in the transaction, and 4,673,597 shares of our common stock, subject in certain cases to deduction to satisfy applicable taxes. We will also pay up to an aggregate amount of $25.8 million in cash and up to 522,375 shares of our common stock, subject to deduction for taxes and certain holdbacks pursuant to the terms and conditions of the definitive acquisition agreement. Additionally, we are obligated to pay up to an aggregate of $200.0 million upon achievement of certain commercial milestones.

Immediately following the closing of the acquisition of Bluejay, we completed the private placement of 3,385,149 shares of our common stock at a price per share of $68.48 and pre-funded warrants ("Pre-Funded Warrants") to purchase 536,412 shares of our common stock ("Warrant Shares") at a price per share of $68.4799 per Pre-Funded Warrant, which equals the purchase price per share of our common stock sold in the first private placement, less $0.0001, the exercise price of each Pre-Funded Warrant, resulting in aggregate gross proceeds of approximately $268.5 million.

Components of Results of Operations

Revenue

Product Sales, Net

We have three approved medicines: Livmarli, Cholbam and Ctexli. We expect total product sales of our approved medicines will continue to increase on an annual basis.

Our U.S. revenue from product sales, net further depends on our prescription mix of commercial payors, Medicaid and amounts of free medicines provided under our patient assistance program. We expect our prescription mix and resulting gross to net adjustment in the U.S. to remain materially consistent. Our revenue from product sales is recognized when the control of the product is transferred. Under our license agreement with Takeda as well as agreements with distributors, we may receive large periodic orders for our products. The timing of these orders can be inconsistent and can create significant quarter-to-quarter variation in product sales. In addition, we recognize our best estimate of the consideration that we expect to receive when control of the inventory is transferred to our licensed partners and distributors. Such estimates may be complex and include estimates as to if and when our distributors and licensed partner's sales in the market will occur. Estimates are reviewed and updated quarterly as additional information, including in-market pricing and sales information of our authorized distributors and licensed partners, becomes known which may cause variability of quarterly revenue particularly during periods of product launch.

Although we expect product revenues to increase as we continue commercial activities for our approved medicines, we may not achieve commercial success. Certain of our approved medicines, including the Bile Acid Medicines, are subject to immediate competition from compounded and generic entrants, as the abbreviated new drug application ("ANDA") and NDA for these drug products have no remaining or current patent exclusivity. Chenodiol is standard of care for the treatment of CTX in the U.S. and was commercialized with a medical necessity recognition by the FDA until February 2025. We submitted an NDA for chenodiol for the treatment of CTX in 2024 and received FDA approval for the treatment of adults with CTX in February 2025, which is now commercialized under the brand name Ctexli. The FDA has granted orphan exclusivity for chenodiol for the treatment of CTX.

Operating Expenses

Cost of Sales

Cost of sales consist of raw materials, third-party manufacturing costs, personnel, facility and other costs of manufacturing commercial products, transportation and freight, amortization of finite-lived intangible assets and royalty payments payable on net sales of our approved medicines under licensing agreements. Cost of sales may also include period costs related to certain manufacturing services and charges for inventory valuation reserves. In addition, we have firm commitments for the purchase of minimum order quantities for active pharmaceutical ingredients. We periodically evaluate these firm commitments to determine if these commitments are in excess of our needs. If any net loss is determined, we record a charge to cost of sales in the period identified. As of the date of our acquisition of the Bile Acid Medicines from Travere, inventory acquired was valued at its fair value. As a result, our cost of sales exceeded cost to manufacture the inventory and had a negative impact on our gross margin.

For our current approved products, we expect cost of sales to increase in the future mainly due to variable costs associated with increased product sales such as royalties payable and inventory costs, partially offset by lower unit cost of sales for the Bile Acid Medicines, as we sold the acquired inventory valued at fair value in prior periods with substantially all fair value inventory sold as of December 31, 2024. We expect cost of sales to remain approximately unchanged as a percent of product sales in the future.

Research and Development Expenses

Research and development expenses primarily relate to clinical development and manufacturing activities of our product candidates. Our research and development expenses include, among other things:

- salaries and related expenses for employee personnel, including benefits, travel and expenses related to stock-based compensation granted to personnel in development functions;
- external expenses paid to clinical trial sites, contract research organizations ("CROs") and consultants that conduct our clinical trials;
- expenses related to drug formulation development and the production of clinical trial supplies, including fees paid to contract manufacturers;
- licensing milestone payments related to development or regulatory events;
- payments made for the acquisition or licensing of in-process research and development assets with no alternative future use;
- expenses related to non-clinical studies;
- expenses related to compliance with drug development regulatory requirements; and
- other allocated expenses, which include direct and allocated expenses for rent and maintenance of facilities, depreciation of equipment, and other supplies.

We expense research and development costs as incurred. Nonrefundable advance payments for goods or 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. Upfront payments, research and development funding and milestone payments made to third parties in connection with licenses and research and development collaborations are expensed as incurred.

We expect our research and development expense may increase in the future as we continue to develop our volixibat and MRM-3379 product candidates, execute the EXPAND label expansion study for Livmarli and assume the development of brelovitug.

Selling, General and Administrative Expense

Sales and marketing expense, which is a component of selling, general and administrative expense, primarily consisted of employee-related expenses for our sales group, brand marketing, patient support groups and pre-commercialization expenses related to our product candidates. General and administrative expense, which is a component of selling, general and administrative expense, primarily consists of corporate support and other administrative expenses, including employee-related expenses.

We anticipate that our selling, general and administrative expenses will increase in the future to support our continued commercialization efforts of our current approved medicines in the U.S. and internationally as well as increased costs of operating as a global commercial stage biopharmaceutical public company. Additionally, if we receive approval for any of our future product candidates, we will incur increased selling, general and administrative expenses to support those commercialization activities. These increases will likely include increased costs related to hiring of additional personnel and fees to outside consultants to support further marketing, legal, tax, planning and accounting activities.

Interest Income

Interest income consists of interest earned on our cash equivalents and investments.

Interest Expense

We incur interest expense on our convertible notes. Interest on our convertible notes consists of a 4% per annum fixed rate of interest and amortization of debt discount and amortization costs.

Critical Accounting Estimates

The preparation of financial statements and related disclosures in conformity with U.S. generally accepted accounting principles ("GAAP") and our discussion and analysis of our financial condition and operating results require our management to make judgments, assumptions and estimates that affect the amounts reported, including the amount of assets, liabilities, expenses and the disclosure of contingent assets and liabilities. Note 2, "Summary of Significant Accounting Policies," of the Notes to Consolidated Financial Statements in Part II, Item 8 of this Annual Report describes the significant accounting policies and methods used in the preparation of our consolidated financial statements. Management bases its estimates on historical experience, known trends and events, and on various other assumptions it believes to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities. Actual results may differ materially from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in the notes to our consolidated financial statements appearing elsewhere in this Annual Report, we believe the following accounting policies are the most critical for fully understanding and evaluating our financial condition and results of operations.

Intangible Assets, Net

Intangible assets, net are measured at their fair values as of the acquisition date or, in the case of commercial milestone payments, the date they become due. Intangible assets are generally amortized on a straight-line basis over their estimated useful lives. We base the useful lives and related amortization expense on the period of time we estimate the assets will generate net product sales or otherwise be used. We also periodically review the lives assigned to our intangible assets to ensure that our initial estimates do not exceed any revised estimated periods from which we expect to realize cash flows from the technologies. If a change were to occur in any of the above-mentioned factors or estimates, the likelihood of a material change in our reported results would increase.

We evaluate our intangible assets with finite lives for indications of impairment whenever events or changes in circumstances indicate that the carrying value may not be recoverable. Factors that could trigger an impairment review include significant under-performance relative to expected historical or projected future operating results, significant changes in the manner of our use of the acquired assets or the strategy for our overall business or significant negative industry or economic trends. If this evaluation indicates that the value of the intangible asset may be impaired, we make an assessment of the recoverability of the net carrying value of the asset over its remaining useful life. If this assessment indicates that the intangible asset is not recoverable, based on the estimated undiscounted future cash flows of the technology over the remaining amortization period, we reduce the net carrying value of the related intangible asset to fair value and may adjust the remaining amortization period.

We make significant judgments in relation to the valuation of intangible assets resulting from asset acquisitions, particularly in the forecasts of future operating results that are used in the discounted cash flow valuation models. It is possible that plans may change and estimates used may prove to be inaccurate. If our actual results, or the plans and estimates used in future impairment analyses, are lower than the original estimates used to assess the recoverability of these assets, we could incur additional impairment charges.

Product Sales, Net

Revenues from direct product sales are recorded net of reserves established for applicable discounts and allowances that are offered within contracts with our customers, including amounts from payors and other third parties on behalf of our customers. For revenues from distributors and our licensed partner, Takeda, we record net product sales using the estimated variable consideration to be received. The transaction price, which may include fixed or variable consideration, may be subject to constraint and is included in the product sales price only to the extent that it is probable that a significant reversal of the amount of the cumulative revenue recognized will not occur in a future period. We recognize our best estimate of the consideration that we expect to receive when control of the inventory is transferred to our customer and revenue is recognized. For our distributor and licensed partner sales, such estimates may be more complex and include estimates as to if and when our distributors and licensed partner's sales in the market will occur. These estimates are reviewed and updated as additional information, including in-market sales information of our authorized distributors and licensed partners, becomes known. Actual amounts may ultimately differ from our estimates. If actual results vary, we adjust these estimates, which could have an effect on earnings in the period of adjustment.

We are obligated to pay rebates for mandated discounts under the Medicaid Drug Rebate Program and other foreign government programs. Our rebate calculations may require estimates based upon our actual historical experience, customer and payor mix and revenue projections. We update estimates and assumptions on a quarterly basis and record any

necessary adjustments to revenue in the period identified. Estimated rebates are recorded as a reduction of revenue in the period the related sale is recognized. To date, actual government rebates have not differed materially from our estimates.

Cost of Sales

Prior to receiving approval from the FDA or other foreign regulatory authorities for a new medicine or new formulation, we expense all costs incurred related to the manufacture of such medicines as research and development expense because of the inherent risks associated with the development of a drug candidate, the uncertainty about the regulatory approval process and our lack of history for regulatory approval of drug candidates. Subsequent to receiving FDA or other foreign regulatory authority approval, when commercialization is considered probable and the future economic benefit is expected to be realized, we begin capitalizing inventory costs as incurred.

Cost of sales consist of raw materials, manufacturing costs, transportation and freight, amortization of capitalized intangible assets, royalties and direct and indirect overhead costs associated with the manufacturing and distribution of our approved products. Cost of sales may also include period costs related to certain manufacturing services and inventory adjustment charges.

We analyze our inventory levels quarterly for obsolescence and, if required, adjust inventory to its net realizable value for quantities in excess of expected demand. The analysis uses quantitative forecast and historical demand considerations in combination with shelf life and stop sell dates in our estimates to evaluate inventory that may not be sellable. The resulting adjustments are recognized as Cost of Sales.

Accrued Research and Development Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued expenses as of each balance sheet date. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of the actual cost. We accrue and expense clinical trial activities performed by third parties based upon estimates of the proportion of work completed over the life of the individual study and patient enrollment rates in accordance with agreements established with clinical research organizations, clinical trial sites and other vendors associated with the clinical trials. We determine the estimates by reviewing contracts, vendor agreements and purchase orders and through discussions with internal clinical personnel and external service providers as to the progress or stage of completion of trials or services and the agreed-upon fee to be paid for such services.

We make estimates of accrued expenses as of each balance sheet date based on facts and circumstances known to us at that time. We periodically confirm the accuracy of our estimates with the service providers and make adjustments, if necessary. If the actual timing of the performance of services or the level of effort varies from the estimate, we will adjust the accrual accordingly. Nonrefundable advance payments for goods and services, including fees for process development or manufacturing and distribution of clinical supplies that will be used in future research and development activities, are deferred and recognized as expense in the period that the related goods are consumed or services are performed.

Although we do not expect our estimates to be materially different from amounts actually incurred, if our estimates of the status and timing of services performed differ from the actual status and timing of services performed, it could result in us reporting amounts that are too high or too low in any particular period. To date, there have been no material differences between our estimates of such expenses and the amounts actually incurred.

Recent Accounting Pronouncements

A description of recent accounting pronouncements that may potentially impact our financial position, results of operations or cash flows is disclosed in Note 2 to our consolidated financial statements included elsewhere in this Annual Report.

Results of Operations for the Years Ended December 31, 2025 and 2024

In this section, we discuss the results of our operations for the year ended December 31, 2025, compared to the year ended December 31, 2024. For a discussion of the year ended December 31, 2024 compared to the year ended December 31, 2023, please refer to Part II, Item 7, *Management's Discussion and Analysis of Financial Condition and Results of Operations*, of our Annual Report on Form 10-K filed with the SEC on February 26, 2025.

The following table summarizes our results of operations for the years ended December 31, 2025 and 2024 (in thousands):

	Year Ended December 31,		
	2025	2024	Change
Revenue:			
Product sales, net	$ 521,312	$ 336,409	$ 184,903
License and other revenue	—	479	(479)
Total revenue	521,312	336,888	184,424
Operating expenses:			
Cost of sales	100,240	81,643	18,597
Research and development	186,178	140,630	45,548
Selling, general and administrative	257,030	202,221	54,809
Total operating expenses	543,448	424,494	118,954
Loss from operations	(22,136)	(87,606)	65,470
Other income (expense):			
Interest income	12,727	13,792	(1,065)
Interest expense	(14,389)	(14,311)	(78)
Other income, net	2,371	1,213	1,158
Loss before provision for income taxes	(21,427)	(86,912)	65,485
Provision for income taxes	1,936	1,030	906
Net loss	$ (23,363)	$ (87,942)	$ 64,579

Product Sales, Net

Product sales, net was $521.3 million for the year ended December 31, 2025, compared to $336.4 million for the year ended December 31, 2024. The increase in product sales, net was a result of our continued commercialization of Livmarli in the U.S. for the treatment of ALGS and PFIC, and in certain international markets directly or through distributor and licensed partner orders and from our sales of the Bile Acid Medicines.

The following table disaggregates total Product sales, net:

	Year Ended December 31,		
	2025	2024	Change
Product sales, net:			
Livmarli	$ 360,006	$ 213,295	$ 146,711
Bile Acid Medicines	161,306	123,114	38,192
Total product sales, net	521,312	336,409	184,903

Cost of Sales

Cost of sales was $100.2 million for the year ended December 31, 2025, compared to $81.6 million for the year ended December 31, 2024. The increase in cost of sales was primarily a result of increases in royalty expenses of $17.9 million on net sales of Livmarli and the Bile Acid Medicines under licensing agreements, a $2.7 million increase primarily associated with increased PDUFA fees associated with the approval of our solid dose formulation in Livmarli and higher commercial supply chain costs of $3.5 million. These increases were partially offset by lower product cost of sales of $6.6 million primarily related to the Bile Acid Medicines, as we substantially completed the sale of acquired inventory in prior periods which had been recorded at fair value.

Research and Development Expenses

The following table summarizes the period-over-period changes in research and development expenses relating to our product candidates in development for the periods indicated (in thousands):

	Year Ended December 31,		Change
	2025	2024	
Product-specific costs:			
Livmarli	$ 14,453	$ 25,631	$ (11,178)
Volixibat	53,097	33,325	19,772
MRM-3379	12,053	7,500	4,553
Non product-specific costs:			
Stock-based compensation	24,158	15,188	8,970
Personnel	54,327	38,179	16,148
License fees (milestone payments)	5,000	—	5,000
Other	23,090	20,807	2,283
Total research and development expenses	$ 186,178	$ 140,630	$ 45,548

Research and development expenses were $186.2 million for the year ended December 31, 2025, an increase of $45.5 million compared to the year ended December 31, 2024. The net increase was primarily due to:

- for volixibat programs, an increase of $19.8 million, primarily due to increased expenses associated with conduct of the PSC and PBC trials as well as manufacturing development expenses;
- for MRM-3379, an increase of $4.6 million, primarily due to our Phase 2 study in FXS and clinical manufacturing expenses partially offset by the prior year $7.5 million upfront payment associated with the in-licensing of MRM-3379;
- for personnel related and stock-based compensation expenses, an increase of $25.1 million, primarily driven by increased employee headcount and related equity award grants to support our development pipeline; and
- for license fees, an increase of $5.0 million due to a development milestone payment associated with our Livmarli Phase 3 EXPAND label expansion study, partially offset by
- for Livmarli, a decrease of $11.2 million, primarily due to completion of clinical trials including the biliary atresia, PFIC rollover study and a safety study, and lower general clinical support costs partially offset by increased expenses associated with the Livmarli Phase 3 EXPAND label expansion study.

Selling, General and Administrative Expenses

Selling, general and administrative expenses were $257.0 million for the year ended December 31, 2025, an increase of $54.8 million compared to the year ended December 31, 2024. The increase was primarily due to increases of $34.1 million in personnel and other compensation related expenses, including an increase of $13.8 million in stock-based compensation, reflecting an increase in the number of our selling, marketing and administrative employees to support commercial activities for our approved medicines, $6.6 million in advertising, promotion and medical affairs expenses associated with commercial activities, $5.5 million associated with legal, accounting and other outside services, $5.1 million in other general administrative expenses and $3.5 million of expenses associated with post marketing studies.

Liquidity and Capital Resources

Overview

Since inception, we have funded our operations primarily through debt, equity, revenue interest financings and cash from our product sales and license and collaboration revenue. We had $391.4 million of unrestricted cash, cash equivalents and investments as of December 31, 2025, compared to unrestricted cash, cash equivalents and investments of $292.8 million as of December 31, 2024. We have incurred significant operating losses since our inception. As of December 31, 2025, we had an accumulated deficit of $667.5 million, compared to $644.2 million as of December 31, 2024.

In January 2026, immediately following the consummation of the Bluejay Acquisition, we completed the private placement of 3,385,149 shares of our common stock at a price per share of $68.48 and Pre-Funded Warrants to purchase 536,412 Warrant Shares at a price per share of $68.4799 per Pre-Funded Warrant, which equals the purchase price per share of our common stock sold in the first private placement, less $0.0001, the exercise price of each Pre-Funded Warrant, resulting in aggregate gross proceeds of approximately $268.5 million.

In August 2025, we filed an automatic shelf registration statement on Form S-3 with the SEC (the "2025 Shelf Registration"), which became effective upon filing, pursuant to which we may register for sale from time to time in one or more offerings an unlimited amount of any combination of our common stock, preferred stock, debt securities and warrants, so long as we continue to satisfy the requirements of a "well-known seasoned issuer" under SEC rules. This automatic shelf registration statement will remain in effect for up to three years from the date it became effective. As of December 31, 2025, we have not issued any securities pursuant to the 2025 Shelf Registration.

In November 2023, we entered into a Sales Agreement (the "2023 Sales Agreement") with Leerink and Cantor Fitzgerald & Co. (the "Sales Agents"), pursuant to which we may, from time to time, sell up to an aggregate amount of $200.0 million of our common stock through the Sales Agents in an "at-the-market" offering (the "ATM Offering"). We are not required to sell shares under the 2023 Sales Agreement. Sales of our common stock, if any, under the 2023 Sales Agreement may be made in any transactions that are deemed to be "at the market offerings" as defined in Rule 415 under the Securities Act. We will pay a given designated Sales Agent a commission of up to 3.0% of the aggregate gross proceeds of any shares of common stock sold through it pursuant to the 2023 Sales Agreement. As of December 31, 2025, we have not issued any securities pursuant to the 2023 Sales Agreement.

In August 2023, we completed the Bile Acid Portfolio Acquisition for an aggregate purchase price of up to $445.0 million in cash, with $210.4 million paid at the closing and up to $235.0 million upon achievement of certain milestones based on specified amounts of annual net sales (tiered from $125.0 million to $500.0 million) of the Bile Acid Medicines. In connection with and immediately prior to the closing of the Bile Acid Portfolio Acquisition, we completed the private placement of 8,000,000 shares of our common stock at a price per share of $26.25, resulting in net proceeds of approximately $202.2 million, which we used to finance the upfront payment at the closing of the Bile Acid Portfolio Acquisition.

In April 2023, we completed an offering of $316.3 million aggregate principal of the Notes, which includes the exercise of the initial purchasers' option in full. The offering resulted in net proceeds of $305.3 million after deducting the initial purchasers' discounts and commissions and offering expenses. The terms of the Notes are further described in Note 10 to our consolidated financial statements. We used a portion of the net proceeds to repurchase the Revenue Interest Purchase Agreement ("RIPA") at a call price of $192.7 million. Upon repurchase of the revenue interests from the Purchasers, the RIPA, in accordance with its terms, was terminated.

Based on our current and anticipated level of operations and cash generated from sales of our approved medicines, we believe our existing unrestricted cash, cash equivalents and investments will be sufficient to fund current operations through at least the next 12 months from the filing of this Annual Report and beyond.

While we generated net income in the third quarter of 2025, we anticipate that we will continue to incur net losses for the foreseeable future as we continue research efforts and the development of our product candidates, including development of brelovitug which we acquired in January 2026, continue commercialization activities for our approved medicines and potentially expand into additional markets, hire additional staff, including clinical, scientific, operational, financial and management personnel and pay potential development milestones. Net loss is also impacted by significant non-cash charges related to stock-based compensation and amortization of intangible assets.

Our primary use of cash is to fund operating expenses. Our cash flow from operating activities may experience material fluctuations due to a number of factors, including the timing of inventory builds, accounts receivable collections, receipt and payment of invoices, development or commercial milestone payments, as well as the magnitude and timing of cash receipts from our product revenues associated with periodic orders from Takeda and our distributors.

Our principal source of liquidity is product revenue from sales of our approved medicines. For the year ended December 31, 2025, liquidity from product revenues was sufficient to fund current operations. There can be no assurances that future revenues will continue to be sufficient to fund operations. For example, as a result of the Bluejay Acquisition, we expect a significant increase in research and development expenses over the next few years as we continue the clinical development of brelovitug. Should product revenues from our currently approved medicines, our current product candidates or any future product candidates, if approved, be insufficient to fund operations, we would expect to finance our cash needs through a combination of cash on hand, equity offerings, debt financings and potential collaboration, license or development agreements. Our primary cash needs are for day-to-day operations and to fund our working capital requirements. To the extent that we raise additional capital through the sale of equity or convertible debt securities,

ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect rights as a stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. Additionally, if the equity and credit markets deteriorate from adverse geopolitical and macroeconomic developments or otherwise, it may make any necessary debt or equity financing more difficult, more costly and more dilutive. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our drug development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Material Cash Requirements

In addition to ongoing capital needs to fund our ongoing operations, our material cash requirements include the following contractual and other obligations.

In April 2023, we completed an offering of $316.3 million aggregate principal of the Notes, which includes the exercise of the initial purchasers' option in full. The offering resulted in net proceeds of $305.3 million after deducting the initial purchasers' discounts and commissions and offering expenses. The Notes are our senior, unsecured obligations and accrue interest at a rate of 4.00% per annum, payable semi-annually in arrears on May 1 and November 1 of each year. The Notes will mature on May 1, 2029, unless earlier converted, redeemed or repurchased by us. The terms of these Notes are further described in Note 10 to our consolidated financial statements.

During the fourth quarter of 2025, the last reported sale price of our common stock exceeded 130% of the conversion price of the Notes for more than 20 trading days during the 30 consecutive trading days ended December 31, 2025. As a result, the Notes are convertible at the option of the holders of the Notes during the first quarter of 2026, the quarter immediately following the quarter when the conditions were met, as stated in the terms of the Notes. If holders of the Notes elect to convert their Notes, we may elect to settle such conversions by paying or delivering, as applicable, cash, shares of our common stock or a combination of cash and shares of our common stock. During the year ended December 31, 2025, holders of the Notes converted an immaterial amount of principal balance.

Under the Shire License Agreement, the Asset Purchase Agreement with Travere, license agreement with Enthorin Therapeutics, LLC ("Enthorin"), Bluejay's license agreement with Novartis Pharma AG, which we acquired as part of the Bluejay Acquisition in January 2026 (see below), as well as our other license and acquisition agreements, we have payment obligations that are contingent upon future events such as our achievement of specified development, regulatory and commercial milestones and are required to make royalty payments in connection with the sale of products developed under those agreements. The amount and timing of milestone obligations are unknown or uncertain as we are unable to estimate the timing or likelihood of achieving the milestone events. Additionally, the amount of royalty payments are based upon future product sales, which we are unable to predict with certainty. These potential obligations are further described in Note 7 to our consolidated financial statements.

On January 23, 2026, we completed the acquisition of Bluejay. As consideration for the transaction, we paid $224.2 million in cash, net of cash acquired in the transaction, and 4,673,597 shares of Company common stock, subject in certain cases to deduction to satisfy applicable taxes, and will pay up to an aggregate of $25.8 million in cash and 522,375 shares of Company common stock, subject to deduction for taxes and certain holdbacks pursuant to the terms and conditions of the definitive acquisition agreement. Additionally, we are obligated to pay up to an aggregate of $200 million upon achievement of certain commercial milestones.

We additionally have contractual obligations for our operating leases for our corporate headquarters. These obligations are further described in Note 9 to our consolidated financial statements.

We enter into contracts in the normal course of business with clinical research organizations and clinical sites for the conduct of clinical trials, non-clinical research studies, professional consultants for expert advice and other vendors for clinical supply manufacturing or other services. These contracts generally provide for termination on notice, and therefore are cancellable contracts.

We enter into commercial inventory supply agreements that obligate us to firm commitments for the purchase of minimum order quantities, which may be material to our financial statements.

Cash Flows

The following table provides a summary of the net cash flow activity for the periods indicated (in thousands):

	Year Ended December 31,	
	2025	2024
Net cash provided by operating activities	$ 55,827	$ 10,325
Net cash used in investing activities	(23,954)	(90,125)
Net cash provided by financing activities	40,142	17,699
Effect of exchange rate on cash, cash equivalents and restricted cash	3,222	(1,297)
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 75,237	$ (63,398)

Net Cash Provided by Operating Activities

Net cash provided by operating activities was $55.8 million for the year ended December 31, 2025, reflecting our net loss of $23.4 million partially offset by adjustments to net loss of $98.9 million. The adjustments consisted primarily of stock-based compensation expense, depreciation and amortization of our intangible assets and fixed assets and charges associated with excess and obsolete inventory and firm commitment losses. Additionally, cash provided by operating activities reflected changes in net operating assets of $19.7 million, primarily related to an increase in accounts receivable due to the growth from our product sales, payments made for the purchase of inventory and prepaid and other current assets, partially offset by an increase in accounts payable, accrued expenses and other liabilities resulting primarily from an increase in accrued sales deductions and royalties due to the growth from our product sales in the year ended December 31, 2025, and an increase in accrued expenses driven by our growth, including accrued expenses related to clinical studies and contract manufacturing activities.

Net cash provided by operating activities was $10.3 million for the year ended December 31, 2024, reflecting our net loss of $87.9 million partially offset by adjustments to net loss of $75.7 million. The adjustments consisted primarily of stock-based compensation expense, depreciation and amortization of our intangible assets and fixed assets and charges associated with excess and obsolete inventory and firm commitment losses. Additionally, cash provided by operating activities reflected changes in net operating assets of $22.6 million, primarily related to an increase in accounts payable, accrued expenses and other liabilities resulting primarily from an increase in accrued sales deductions and royalties due to the growth from our product sales in the year ended December 31, 2024, and an increase in accrued expenses driven by our growth, including accrued expenses related to clinical studies and contract manufacturing activities, partially offset by an increase in accounts receivable due to the growth from our product sales and payments made for the purchase of inventory.

Net Cash Used in Investing Activities

Net cash used in investing activities was $24.0 million for the year ended December 31, 2025, primarily due to purchases of investments partially offset by cash provided by the maturities of investments.

Net cash used in investing activities was $90.1 million for the year ended December 31, 2024, primarily due to purchases of investments resulting from the changing interest rate environment and milestone payments associated with the approval of Livmarli for cholestatic pruritus in patients with PFIC in the U.S. and for the treatment of PFIC in the EU, partially offset by proceeds from maturities of investments.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $40.1 million for the year ended December 31, 2025, due to proceeds from employee equity award exercises.

Net cash provided by financing activities was $17.7 million for the year ended December 31, 2024, due to proceeds from employee equity award exercises.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk.

Interest Rate Risk

Our cash, cash equivalents and investments as of December 31, 2025 consist of readily available checking and money market funds and investments. The primary objective of our investment activities is to preserve our capital to fund operations. We may invest in highly liquid and high-quality government and debt securities. As a result, our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the strategies we employ (including the short-term nature of the instruments in our portfolio and the low risk

profile of our investments), as of the date of this report, we do not expect anticipated changes in interest rates to have a material effect on our interest rate risk in future reporting periods. For example, a hypothetical change in interest rates of 10% would not have a material impact on the fair market value of our cash equivalents and investments as of December 31, 2025. In addition, we maintain significant amounts of cash and cash equivalents at one financial institution that is in excess of federally insured limits.

We have outstanding $316.2 million aggregate principal of the Notes as of December 31, 2025. The interest rates on these Notes are fixed and therefore they do not expose us to risk related to changing interest rates. As of December 31, 2025, the approximate fair value of our Notes was $818.5 million.

Foreign Currency Rate Risk

Our operations include activities in the U.S., the Netherlands, Switzerland and certain other countries in Europe. As a result, our financial results may be affected by factors such as changes in foreign currency exchange rates or weak economic conditions in the foreign markets in which we sell our products. Our operating results are exposed to changes in foreign currency exchange rates between the U.S. Dollar ("USD") and various foreign currencies, primarily the Euro and Swiss Franc. When the USD strengthens against these currencies, the relative value of the sales and operating expenses made in the respective foreign currency decreases. Conversely, when the USD weakens against these currencies, the relative value of such sales and operating expenses increases.

Based on our overall foreign currency denominated exposures as of December 31, 2025, we believe that a near-term 10% fluctuation of the USD exchange rate could result in a potential change in the fair value of our net assets and liabilities denominated in foreign currency by approximately $4.1 million. We expect to continue to enter into transactions based in foreign currencies that could be impacted by changes in the USD exchange rate.

Item 8. Financial Statements and Supplementary Data.

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

Reports of Independent Registered Public Accounting Firm (PCAOB ID: 42)	104
Consolidated Balance Sheets	107
Consolidated Statements of Operations	108
Consolidated Statements of Comprehensive Loss	109
Consolidated Statements of Stockholders' Equity	110
Consolidated Statements of Cash Flows	111
Notes to Consolidated Financial Statements	112

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Mirum Pharmaceuticals, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Mirum Pharmaceuticals, Inc. (the Company) as of December 31, 2025 and 2024, the related consolidated statements of operations, comprehensive loss, stockholders' equity and cash flows for each of the three years in the period ended December 31, 2025, 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, 2025 and 2024, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2025, 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, 2025, 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 25, 2026 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.

Inventory Costing

Description of the Matter	As described in Notes 2 and 5 to the consolidated financial statements, inventory, which totaled $24.9 million as of December 31, 2025, is comprised of raw materials, work-in-progress and finished goods. Inventory is valued at the lower of cost or net realizable value, with cost determined on a first-in, first-out basis. Auditing the inventory costing was complex because the nature of the process for such costing involves the use of a variety of inputs such as third-party material costs and allocated internal labor, all of which are tracked in manually-prepared spreadsheets.
How We Addressed the Matter in Our Audit	We obtained an understanding, evaluated the design and tested the operating effectiveness of internal controls over the inventory process. For example, we tested controls over management's review of inputs used in determining the costs to be capitalized into inventory. To test the completeness and accuracy of the inventory balance, our audit procedures included, among others, agreeing a sample of production costs and purchases used in the spreadsheets to actual costs incurred, agreeing a sample of capitalized labor costs to payroll records, confirming certain quantities of on-hand inventory directly with third-party service providers, and confirming amounts owed to certain third-party service providers.

/s/ Ernst & Young LLP

We have served as the Company’s auditor since 2019.

San Mateo, California
February 25, 2026

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Mirum Pharmaceuticals, Inc.

Opinion on Internal Control Over Financial Reporting

We have audited Mirum Pharmaceuticals, Inc.'s internal control over financial reporting as of December 31, 2025, 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, Mirum Pharmaceuticals, Inc. (the Company) maintained, in all material respects, effective internal control over financial reporting as of December 31, 2025, 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, 2025 and 2024, the related consolidated statements of operations, comprehensive loss, stockholders' equity and cash flows for each of the three years in the period ended December 31, 2025, and the related notes and our report dated February 25, 2026 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 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 Mateo, California
February 25, 2026

Mirum Pharmaceuticals, Inc.
Consolidated Balance Sheets
(In thousands, except share and per share data)

	December 31,	
	2025	2024
Assets		
Current assets:		
Cash and cash equivalents	$ 296,683	$ 222,503
Short-term investments	86,644	57,812
Accounts receivable	123,330	78,286
Inventory	24,887	22,403
Prepaid expenses and other current assets	18,140	11,784
Total current assets	549,684	392,788
Restricted cash	1,482	425
Long-term investments	8,105	12,526
Property and equipment, net	1,862	1,139
Operating lease right-of-use assets	8,741	8,675
Intangible assets, net	260,921	249,819
Other assets	12,018	5,382
Total assets	$ 842,813	$ 670,754
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 9,614	$ 14,618
Accrued expenses and other current liabilities	196,185	111,933
Total current liabilities	205,799	126,551
Operating lease liabilities, noncurrent	7,516	7,972
Convertible notes payable, net, noncurrent	309,797	308,082
Other liabilities	5,011	2,509
Total liabilities	528,123	445,114
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, $0.0001 par value; 10,000,000 shares authorized, and no shares issued and outstanding as of December 31, 2025 and 2024	—	—
Common stock, $0.0001 par value; 200,000,000 shares authorized; 51,896,391 and 48,338,096 shares issued and outstanding as of December 31, 2025 and 2024, respectively	5	5
Additional paid-in capital	981,878	870,189
Accumulated deficit	(667,544)	(644,181)
Accumulated other comprehensive income (loss)	351	(373)
Total stockholders' equity	314,690	225,640
Total liabilities and stockholders' equity	$ 842,813	$ 670,754

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

Mirum Pharmaceuticals, Inc.
Consolidated Statements of Operations
(In thousands, except share and per share data)

	Year Ended December 31,		
	2025	2024	2023
Revenue:			
Product sales, net	$ 521,312	$ 336,409	$ 178,874
License and other revenue	—	479	7,500
Total revenue	521,312	336,888	186,374
Operating expenses:			
Cost of sales	100,240	81,643	47,039
Research and development	186,178	140,630	102,609
Selling, general and administrative	257,030	202,221	145,880
Total operating expenses	543,448	424,494	295,528
Loss from operations	(22,136)	(87,606)	(109,154)
Other income (expense):			
Interest income	12,727	13,792	13,735
Interest expense	(14,389)	(14,311)	(15,105)
Loss from termination of revenue interest purchase agreement	—	—	(49,076)
Other income (expense), net	2,371	1,213	(2,824)
Loss before provision for income taxes	(21,427)	(86,912)	(162,424)
Provision for income taxes	1,936	1,030	991
Net loss	$ (23,363)	$ (87,942)	$ (163,415)
Net loss per share, basic and diluted	$ (0.47)	$ (1.85)	$ (4.00)
Weighted-average shares of common stock, basic and diluted	50,198,304	47,522,594	40,885,124

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

Mirum Pharmaceuticals, Inc.
Consolidated Statements of Comprehensive Loss
(In thousands)

	Year Ended December 31,		
	2025	2024	2023
Net loss	$ (23,363)	$ (87,942)	$ (163,415)
Other comprehensive income (loss):			
Unrealized gain on available-for-sale investments	10	130	230
Cumulative translation adjustments	714	(2,147)	1,631
Comprehensive loss	$ (22,639)	$ (89,959)	$ (161,554)

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

Mirum Pharmaceuticals, Inc.
Consolidated Statements of Stockholders' Equity
(In thousands, except share and per share data)

	Common Stock		Additional Paid-In Capital	Accumulated Deficit	Accumulated Other Comprehensive Income (Loss)	Total Stockholders' Equity
	Shares	Amount				
Balance as of December 31, 2022	36,956,345	$ 4	$ 535,074	$ (392,824)	$ (217)	$ 142,037
Issuance of common stock in private placement, net of issuance costs of $7,796	8,000,000	1	202,203	—	—	202,204
Issuance of common stock in at-the-market offerings, net of issuance costs of $518	658,206	—	14,480	—	—	14,480
Issuance of common stock in connection with asset acquisition	231,624	—	5,188	—	—	5,188
Issuance of common stock in connection with equity award plans	760,226	—	8,279	—	—	8,279
Issuance of common stock in connection with employee stock purchase plan	116,742	—	2,191	—	—	2,191
Stock-based compensation	—	—	35,845	—	—	35,845
Net loss	—	—	—	(163,415)	—	(163,415)
Other comprehensive income	—	—	—	—	1,861	1,861
Balance as of December 31, 2023	46,723,143	$ 5	$ 803,260	$ (556,239)	$ 1,644	$ 248,670
Issuance of common stock in connection with equity award plans	1,489,510	—	15,059	—	—	15,059
Issuance of common stock in connection with employee stock purchase plan	125,443	—	2,640	—	—	2,640
Stock-based compensation	—	—	49,230	—	—	49,230
Net loss	—	—	—	(87,942)	—	(87,942)
Other comprehensive loss	—	—	—	—	(2,017)	(2,017)
Balance as of December 31, 2024	48,338,096	$ 5	$ 870,189	$ (644,181)	$ (373)	$ 225,640
Issuance of common stock in connection with equity award plans	3,450,566	—	36,200	—	—	36,200
Issuance of common stock in connection with employee stock purchase plan	106,977		3,942			3,942
Conversion of convertible notes, net	752	—	24	—	—	24
Stock-based compensation	—	—	71,523	—	—	71,523
Net loss	—	—	—	(23,363)	—	(23,363)
Other comprehensive income	—	—	—	—	724	724
Balance as of December 31, 2025	51,896,391	$ 5	$ 981,878	$ (667,544)	$ 351	$ 314,690

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

Mirum Pharmaceuticals, Inc.
Consolidated Statements of Cash Flows
(In thousands)

	Year Ended December 31,		
	2025	2024	2023
Operating activities			
Net loss	$ (23,363)	$ (87,942)	$ (163,415)
Reconciliation of net loss to net cash provided by operating activities:			
Stock-based compensation	71,424	48,444	35,023
Depreciation and amortization	24,244	23,629	10,829
Inventory reserves and firm commitment losses	2,889	2,614	9,257
Amortization of debt discount and offering costs	1,739	1,661	1,117
Loss from termination of revenue interest purchase agreement	—	—	49,076
Non-cash interest expense related to the revenue interest liability	—	—	5,060
Unrealized foreign exchange (gain) loss	(1,720)	(1,231)	1,764
Non-cash lease expense	1,730	1,354	620
Other	(1,401)	(795)	(1,054)
Change in operating assets and liabilities:			
Accounts receivable	(48,055)	(11,426)	(43,974)
Prepaid and other current assets	(5,756)	(849)	(3,600)
Inventory	(8,448)	(3,166)	(974)
Other assets	(5,663)	(1,498)	(511)
Accounts payable, accrued expenses and other liabilities	50,103	40,312	30,778
Operating lease liabilities	(1,896)	(782)	(940)
Net cash provided by (used in) operating activities	55,827	10,325	(70,944)
Investing activities			
Purchase of investments	(97,425)	(89,026)	(27,329)
Proceeds from maturities of investments	74,425	19,894	153,000
Purchase of property and equipment	(954)	(993)	(109)
Cash paid for acquisition	—	—	(212,762)
Payments made for additions to intangible assets	—	(20,000)	(20,000)
Net cash used in investing activities	(23,954)	(90,125)	(107,200)
Financing activities			
Proceeds from issuance of common stock in private placement, net of issuance costs	—	—	202,204
Proceeds from issuance of common stock in at-the-market offerings, net of issuance costs	—	—	14,480
Proceeds from issuance of convertible notes, net	—	—	305,304
Proceeds from issuance of common stock pursuant to equity plans	40,142	17,699	10,470
Payments of deferred offering costs	—	—	(281)
Payments on revenue interest liability	—	—	(195,577)
Net cash provided by financing activities	40,142	17,699	336,600
Effect of exchange rate on cash, cash equivalents and restricted cash	3,222	(1,297)	(133)
Net increase (decrease) in cash, cash equivalents and restricted cash	75,237	(63,398)	158,323
Cash, cash equivalents and restricted cash at beginning of period	222,928	286,326	128,003
Cash, cash equivalents and restricted cash at end of period	$ 298,165	$ 222,928	$ 286,326
Supplemental disclosure of cash flow information:			
Operating cash flows paid for operating lease	$ 2,590	$ 1,274	$ 1,079
Cash paid for interest	$ 12,650	$ 12,650	$ 6,817
Cash paid for income taxes	$ 2,157	$ 1,004	$ 125
Non-cash operating, investing and financing activities:			
Accrued milestone payments classified as intangible assets, net	$ 35,000	$ —	$ —
Right-of-use assets obtained in exchange for lease liabilities	$ 2,444	$ 9,633	$ 473
Decrease in ROU assets and lease liabilities due to lease modification	$ 649	$ 723	$ —
Stock-based compensation capitalized to inventory	$ 688	$ 1,122	$ 822
Conversion of convertible notes, net into shares of common stock	$ 24	$ —	$ —
Issuance of common stock in connection with settlement of Contingent Milestone and Indemnification Holdback liabilities	$ —	$ —	$ 5,188

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

1. Organization and Description of Business

Mirum Pharmaceuticals, Inc. (the "Company") was incorporated in the State of Delaware on May 2, 2018, and is headquartered in Foster City, California. The Company is a biopharmaceutical company dedicated to transforming the treatment of rare diseases. The Company commenced significant operations in November 2018.

As of December 31, 2025, the Company had three approved medicines: LIVMARLI® (maralixibat) ("Livmarli"), CHOLBAM® (cholic acid) capsules ("Cholbam"), and CTEXLI® (chenodiol) tablets ("Ctexli"). Livmarli is approved for the treatment of cholestatic pruritus in patients with Alagille syndrome ("ALGS") in the United States ("U.S."), the European Union ("EU") and various other countries around the world and for cholestatic pruritus in patients with progressive familial intrahepatic cholestasis ("PFIC") in the U.S., Canada and Japan and for the treatment of PFIC in the EU.

On August 31, 2023, the Company completed the acquisition of assets of Travere Therapeutics, Inc. ("Travere") that are primarily related to the development, manufacture (including synthesis, formulation, finishing or packaging) and commercialization of chenodiol and Cholbam (also known as Kolbam, and together with chenodiol, the "Bile Acid Medicines"), two therapies addressing rare diseases in high-need settings (such acquisition, the "Bile Acid Portfolio Acquisition") (Note 7). Cholbam is FDA-approved for the treatment of bile acid synthesis disorders due to single enzyme deficiencies and adjunctive treatment of peroxisomal disorders in patients who show signs or symptoms of liver disease. Ctexli at the time of acquisition was approved for the treatment of radiolucent stones in the gallbladder, had received medical necessity recognition by the FDA for the treatment of cerebrotendinous xanthomatosis ("CTX") and was commercialized under the brand name Chenodal. Subsequently, in February 2025, the Company received FDA approval for chenodiol tablets for the treatment of CTX in adults, which is commercialized under the brand name Ctexli.

The Company's development pipeline consists of the clinical-stage product candidate volixibat, MRM-3379 and indication expansion opportunities for Livmarli.

On January 23, 2026, the Company acquired Bluejay Therapeutics, Inc. ("Bluejay"). With the completion of the acquisition, the Company adds worldwide rights to brelovitug, a late-stage, fully human monoclonal antibody for chronic hepatitis delta virus (HDV), a rare and severe liver disease.

The Company views its operations and manages its business as one operating segment. The Company determined its operating segment on the same basis that it uses to evaluate its performance internally. See Note 14 *Segment Reporting* for further details.

Liquidity

The Company has a limited operating history, has incurred significant operating losses since its inception, and the revenue and income potential of the Company's business and market are unproven. As of December 31, 2025, the Company had an accumulated deficit of $667.5 million and unrestricted cash, cash equivalents and investments of $391.4 million. The Company's convertible notes are convertible at the option of the holders during the first quarter of 2026. If holders of the convertible notes elect to convert, the Company may elect to settle such conversions in cash, common stock or a combination of the two. The Company believes that its unrestricted cash, cash equivalents and investments of $391.4 million as of December 31, 2025 provide sufficient capital resources to continue its operations for at least twelve months from the issuance date of the accompanying consolidated financial statements.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries. The Company translates the financial statements of its foreign subsidiaries using end-of-period exchange rates for assets and liabilities and average exchange rates during each reporting period for results of operations. All intercompany balances and transactions among the consolidated entities have been eliminated in consolidation. The accompanying consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP").

Use of Estimates

The preparation of consolidated financial statements in accordance with GAAP requires management to make estimates and assumptions that impact the reported amounts of assets, liabilities, revenues and expenses and the disclosure

of contingent assets and liabilities in the financial statements and accompanying notes. These estimates and assumptions are based upon historical experience, knowledge of current events and various other factors believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. Actual results could differ materially from those estimates.

The Company's consolidated financial statements as of and for the year ended December 31, 2025 reflect the Company's estimates of the impact of the geopolitical and macroeconomic environment, including the impact of inflation, tariffs and trade tensions and foreign exchange rate fluctuations. The duration and the scope of these conditions cannot be predicted; therefore, the extent to which these conditions will directly or indirectly impact the Company's business, results of operations and financial condition, is uncertain. The Company is not aware of any specific event or circumstance that would require an update to its estimates, judgments and assumptions or a revision of the carrying value of the Company's assets or liabilities as of the date of this filing.

Cash, Cash Equivalents and Restricted Cash

The Company considers all highly liquid investments that are readily convertible into cash without penalty and with original maturities of three months or less at the date of purchase to be cash equivalents. The carrying amounts reported in the consolidated balance sheets for cash and cash equivalents are valued at cost, which approximate their fair value.

The following table provides a reconciliation of cash, cash equivalents and restricted cash reported within the consolidated balance sheets that together reflect the same amounts shown in the consolidated statements of cash flows (in thousands):

	As of December 31,	
	2025	2024
Cash and cash equivalents	$ 296,683	$ 222,503
Restricted cash	1,482	425
Total cash, cash equivalents, and restricted cash	$ 298,165	$ 222,928

Concentrations of Credit Risk and Off-Balance Sheet Risk

Financial instruments that potentially subject the Company to a concentration of credit risk consist of cash and cash equivalents, accounts receivable and investments. The Company limits the amount of credit exposure by investing cash that is not required for immediate operating needs in money market funds, government obligations, corporate debt securities and/or commercial paper. Additionally, the Company has established guidelines regarding diversification of its investments and their maturities, which are designed to maintain principal and maximize liquidity. To date, the Company has not experienced any losses associated with this credit risk and continues to believe that this exposure is not significant.

The Company relies on a single third-party logistics provider ("3PL") in each major market and a single specialty pharmacy for all of the Company's sales of its approved medicines in the United States.

The Company sources materials and services through several vendors. Certain materials are sourced from a single vendor. The loss of certain vendors could result in a temporary disruption of the Company's commercialization efforts.

As of December 31, 2025 and as of December 31, 2024, the Company did not have any customers that individually accounted for more than 10% of accounts receivable. For the years ended December 31, 2025, 2024 and 2023, the Company did not have revenue attributable to any one customer in excess of 10% of sales.

Investments

The Company classifies all investments in securities as available-for-sale. Management determines the appropriate classification of its investments in securities at the time of purchase. Investments with original maturities beyond three months at the date of purchase and which mature at, or less than twelve months from the balance sheet date, are classified as a current asset.

Investments are recorded at fair value, with unrealized gains and losses reported as accumulated other comprehensive income (loss) until realized, with the exception of any declines in fair value below the cost basis that are a result of a credit loss, which, if any, are reported in other income (expense), net in the current period through an allowance for credit losses. Each reporting period, the Company evaluates whether declines in fair values of its available-for-sale

securities below their cost basis are a result of credit loss or other factors and whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. This evaluation consists of several qualitative and quantitative factors regarding the severity and duration of the unrealized loss, the creditworthiness of the security issuers, as well as the Company's ability and intent to hold the available-for-sale security until a forecasted recovery occurs. Additionally, the Company assesses whether it has plans to sell the security or it is more likely than not it will be required to sell any available-for-sale securities before recovery of its amortized cost basis. The cost of debt securities is adjusted for amortization of premiums and accretion of discounts to maturity. Such amortization and accretion, as well as interest and dividends, are included in interest income. Realized gains and losses from the sale of available-for-sale securities, if any, are determined on a specific identification basis and are also included in other income (expense). To date, the Company has not identified any declines in fair value of its investments related to credit loss.

Fair Value of Financial Instruments

Fair value is defined as an exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As such, fair value is a market-based measurement determined based on assumptions that market participants would use in pricing an asset or liability. The following fair value hierarchy distinguishes between observable and unobservable inputs, which are categorized in one of the following levels:

Level 1: Observable inputs (unadjusted) such as quoted prices in active markets for identical assets or liabilities;

Level 2: Inputs, other than the quoted prices in active markets, that are observable either directly or indirectly for similar assets or liabilities; and

Level 3: Unobservable inputs in which there is little or no market data, which require the reporting entity to develop its own assumptions.

Accounts Receivable

The Company has accounts receivable amounts due from product sales. The Company also has accounts receivable amounts due from license agreements for milestones achieved, but not yet paid. Amounts payable to the Company are recorded as accounts receivable when the Company's right to consideration is unconditional. The Company estimates the allowance for credit losses using the current expected credit loss model. Under this model, the allowance for credit losses reflects the Company's estimate of lifetime expected credit losses. The Company evaluates the collectability of the cash flows based on the risk of loss over the contractual life, even when that risk is remote, based on judgments about the creditworthiness of its customers, historical experience and other relevant information that is available to the Company. There was no allowance for credit losses as of December 31, 2025. There was no bad debt expense for the years ended December 31, 2025, 2024 and 2023.

Inventory

Inventory is valued at the lower of cost or net realizable value, with cost determined on a first-in, first-out (FIFO) basis. The Company periodically reviews the composition of inventory to identify excess, obsolete, slow-moving or otherwise unsaleable items. If unsaleable items are observed and there are no alternate uses for the inventory, the Company will record a write-down to net realizable value in the period that the decline in value is recognized through a charge to cost of sales. Furthermore, the Company periodically reviews its firm commitments for the purchase of minimum order quantities. If the minimum order quantities exceed the Company's future demand, a net loss is accrued in cost of sales for such future inventory purchases. The determination of whether inventory costs will be realizable requires estimates by management. If actual market conditions are less favorable than projected by management, additional write-downs of inventory may be required.

Property and Equipment

Property and equipment are recorded at cost less accumulated depreciation. Depreciation is computed on a straight-line basis over the estimated useful lives of the related assets, ranging from three to five years. Leasehold improvements are amortized over the shorter of their useful lives or the related lease term. As of December 31, 2025, property and equipment consisted primarily of leasehold improvements of $0.8 million and furniture and equipment of $2.1 million. As of December 31, 2024, property and equipment consisted primarily of leasehold improvements of $0.2 million and furniture and equipment of $1.6 million. Accumulated depreciation as of December 31, 2025 and 2024 was $1.1 million and $0.7 million, respectively. Depreciation expense was $0.3 million, $0.5 million and $0.3 million for the years ended December 31, 2025, 2024 and 2023, respectively.

Impairment of Long-Lived Assets

Long-lived assets are reviewed for indications of possible impairment whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. Recoverability is measured by comparison of the carrying amounts to the future undiscounted cash flows attributable to these assets. An impairment loss is recognized to the extent an asset group is not recoverable, and the carrying amount exceeds the projected discounted future cash flows arising from these assets. There were no impairments of long-lived assets for any of the periods presented.

Intangible Assets, Net

The Company accounts for asset acquisitions that do not meet the definition of a business using the cost accumulation method, whereby the cost of the acquisition, including certain transaction costs, is allocated to the asset (or assets) acquired on the basis of its (or their) relative fair value(s) on the measurement date. No goodwill is recognized in an asset acquisition.

Intangible assets are measured at their fair values as of the acquisition date or, in the case of commercial milestone payments, the date they become due. The evaluation of intangible assets includes assessing the amortization period for which the asset is expected to contribute to the future cash flows of the Company. Intangible assets with finite useful lives are amortized over their estimated useful lives on a straight-line basis. The Company tests its finite lived intangible assets for impairment annually or if events or changes in circumstances indicate that it is more likely than not that the asset is impaired. If it is determined that the asset is impaired, the carrying value is written down to its estimated fair value, with the related impairment charge recognized in the consolidated statements of operations in the period in which the impairment occurs. The Company has not recorded any impairments to its intangible assets for any of the periods presented.

Leases

The Company determines if a contractual arrangement is or contains a lease at inception. Operating lease right-of-use ("ROU") assets represent the Company's right to use an underlying asset during the lease term, and operating lease liabilities represent the Company's obligation to make lease payments arising from the lease. Operating leases are included in ROU assets, current operating lease liabilities, and long-term operating lease liabilities on the accompanying consolidated balance sheets. Operating lease ROU assets and lease liabilities are initially recognized based on the present value of the future minimum lease payments over the lease term at commencement date calculated using the Company's incremental borrowing rate applicable to the leased asset, unless the implicit rate is readily determinable. Operating lease ROU assets also include any lease payments made at or before lease commencement and exclude any lease incentives received. The Company determines the lease term as the noncancelable period of the lease and may include options to extend or terminate the lease when it is reasonably certain that the Company will exercise that option. Leases with a term of 12 months or less are not recognized on the consolidated balance sheets. The Company's leases do not contain any residual value guarantees. Lease expense for minimum lease payments is recognized as rent expense on a straight-line basis over the lease term. Variable lease payments include lease operating expenses.

Accrued Research and Development Expenses

The Company accrues and expenses clinical trial activities performed by third parties based upon estimates of the proportion of work completed over the life of the individual study and patient enrollment rates in accordance with agreements established with clinical research organizations and clinical trial sites. The Company determines the estimates by reviewing contracts, vendor agreements and purchase orders and through discussions with internal clinical personnel and external service providers as to the progress or stage of completion of trials or services and the agreed-upon fee to be paid for such services.

The Company makes estimates of accrued expenses as of each balance sheet date based on facts and circumstances known to the Company at that time. If the actual timing of the performance of services or the level of effort varies from the estimate, the Company will adjust the accrual accordingly. The Company has not experienced any material differences between accrued costs and actual costs incurred for the periods presented. Nonrefundable advance payments for goods and services are deferred and recognized as expense in the period that the related goods are consumed or services are performed.

Convertible Notes

The Company evaluates all conversion, repurchase and redemption features contained in a debt instrument to determine if there are any embedded features that require bifurcation as a derivative. The Company accounts for its

convertible notes (refer to Note 10) as a liability equal to the proceeds received from issuance, including any embedded conversion features, net of the unamortized debt discount and offering costs in the accompanying consolidated balance sheets. The debt issuance and offering costs are amortized over the contractual term of the convertible notes, using the effective interest method, as interest expense in the accompanying consolidated statements of operations.

Revenue Interest Liability, Net

In relation to the revenue interest liability, net, associated with the Revenue Interest Purchase Agreement ("RIPA") with Mulholland SA LLC, an affiliate of Oberland Capital LLC, as agent for the purchasers party thereto (the "Purchasers"), and the Purchasers that the Company entered into in December 2020, as amended in September 2021, the Company imputed interest expense using the effective interest rate method. The effective interest rate was calculated based on the rate that would enable the debt to be repaid in full over the anticipated life of the arrangement. The interest rate on the liability varied during the term of the agreement depending on a number of factors, including the level of actual and forecasted product sales, net. The Company evaluated the interest rate quarterly based on actual product sales, net and forecast product sales, net, utilizing the prospective method. In April 2023, the Company repurchased all future revenue interests (the "Revenue Interests") and as a result, the RIPA was terminated in accordance with its terms (refer to Note 6).

Derivative Liability

The RIPA contained certain features that met the definition of being an embedded derivative requiring bifurcation as a separate compound financial instrument apart from the RIPA. The derivative liability was initially measured at fair value on issuance and was subject to remeasurement at each reporting period. In April 2023, the derivative liability was extinguished in connection with the termination of the RIPA (refer to Note 6).

Revenue Recognition

The Company recognizes revenue when a customer obtains control of promised goods or services, in an amount that reflects the consideration the Company expects to receive in exchange for those goods or services.

Product Sales, Net

The Company recognizes product sales, net when the customer obtains control of its product, which occurs at a point in time, typically upon delivery of the Company's product to the customer.

Revenues from product sales are recorded at the net sales price, or the transaction price, which may include fixed or variable consideration for discounts, government rebates, co-pay assistance, returns and other allowances that are offered within contracts with a customer relating to the sale of the Company's approved medicines. Estimates of variable consideration are calculated using the actual product sales each reporting period and the nature of the variable consideration related to those sales. Overall, these estimates reflect the Company's best estimate of the amount of consideration to which the Company expects to be entitled based on the terms of the contract. The amount of variable consideration that is included in the transaction price may be constrained and is included in product sales, net only to the extent that it is considered probable that a significant reversal in the amount of the cumulative revenue recognized will not occur when the uncertainty associated with the variable consideration is subsequently resolved. Estimates are reviewed and updated as additional information becomes known. Actual amounts of consideration ultimately received may differ materially from estimates. If actual results in the future vary from estimates, the Company will adjust these estimates, which would affect product sales, net and earnings in the period such variances are adjusted. Significant categories of sales discounts and allowances are as follows:

Government Rebates: The Company records rebates payable under Medicaid and other government programs as a reduction of revenue at the time product revenues are generated. The Company's rebate calculations may require estimates, including estimates of customer mix, to determine which sales will be subject to rebates and the amount of such rebates. The Company updates its estimates and assumptions on a quarterly basis and records any necessary adjustments to revenue in the period identified. The liability for unpaid rebates is included in accrued expenses on the accompanying consolidated balance sheets. To date, actual government rebates have not differed materially from the Company's estimates.

Other Incentives: Other incentives include a branded co-pay assistance program for eligible patients with commercial insurance in the United States. The branded co-pay assistance program assists commercially insured patients who have coverage for the Company's approved medicines and is intended to reduce each participating patient's portion of the financial responsibility of the purchase price up to a specified dollar amount of assistance. The calculation of the accrual for co-pay assistance is based upon an identification of claims and the cost per claims associated with product that has been recognized as revenue. The Company records amounts paid under the brand specific co-pay assistance program

for each patient as a reduction of revenue from product sales. To date, actual other incentives have not differed materially from the Company's estimates.

Product Returns: The Company records revenue for product sales, net of estimated product returns. Customers have limited return rights related only to the product's damage or defect identified upon delivery of the product. The Company estimates the amount of product sales that may be returned and records the estimate as a reduction of revenue and a refund liability in the period the related product revenue is recognized. To date, actual returns have not differed materially from the Company's estimates.

The following table represents total revenues and disaggregates Product sales, net by approved medicine (in thousands):

	Year Ended December 31,		
	2025	2024	2023
Product sales, net:			
Livmarli	$ 360,006	$ 213,295	$ 141,795
Bile Acid Medicines	161,306	123,114	37,079
Total product sales, net	521,312	336,409	178,874
License and other revenue	—	479	7,500
Total revenues	$ 521,312	$ 336,888	$ 186,374

The following table sets forth Product sales, net by geographic area based on the ship-to location (in thousands):

	Year Ended December 31,		
	2025	2024	2023
United States	$ 401,547	$ 274,173	$ 146,699
Rest of the world	119,765	62,236	32,175
Total product sales, net	$ 521,312	$ 336,409	$ 178,874

License and Collaboration Arrangements

The Company enters into collaborative arrangements with partners and analyzes the collaboration arrangements to assess whether they are within the scope of *Collaborative Arrangements (Topic 808)* ("Topic 808") and determines whether such arrangements involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards dependent on the commercial success of such activities. This assessment is performed throughout the life of the arrangement based on changes in the responsibilities of all parties in the arrangement. The accounting for some of the activities under collaboration arrangements may be subject to *Revenue from Contracts with Customers (Topic 606)* ("Topic 606") for distinct units of account that are reflective of a vendor-customer relationship. For other elements of collaboration arrangements, such as reimbursements of certain development costs, the Company generally records reimbursements received as a reduction of research and development expenses.

In determining the appropriate amount of revenue to be recognized under Topic 606 as the Company fulfills its obligations under each of its agreements, the Company performs the following steps: (i) identification of the contracts with customers; (ii) determination of whether the promised goods or services are performance obligations including whether they are distinct in the context of the contract; (iii) determination and measurement of the transaction price, including any constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations in the contract; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation.

The terms of the Company's license and collaborative research and development agreements include upfront license fees, research, development and other funding or reimbursements, milestone and other contingent payments for the achievement of defined collaboration objectives and certain development, regulatory and sales-based events, as well as royalties on sales of commercialized products. Arrangements that include upfront payments may require deferral of revenue recognition to a future period until the Company satisfies performance obligations under these arrangements.

A performance obligation is a promise in a contract to transfer a distinct good or service and is the unit of accounting in Topic 606. A contract's transaction price is allocated among each distinct performance obligation based on relative standalone selling price and recognized as revenue when, or as, the applicable performance obligation is satisfied.

If a license to the Company's intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes revenues attributed to the license when the license is transferred to the customer and the customer is able to use and benefit from the license.

At the inception of each arrangement that includes development milestone payments, the Company evaluates whether the milestones are considered probable of being achieved and estimates the amount to be included in the transaction price as variable consideration using the most likely amount method or expected value method, depending on the nature of the contingency and the variable payments. If it is probable that a significant reversal of cumulative revenue recognized for the contract would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the control of the Company or the licensee, such as regulatory approvals, are not generally considered probable of being achieved until those approvals are received. Given the high degree of uncertainty around the occurrence of these events, the Company generally determines the milestone and other contingent amounts to be fully constrained until the uncertainty associated with these payments is resolved. At the end of each reporting period, the Company re-evaluates the probability of achievement of any development milestones, and if necessary, adjusts its estimate of the transaction price. Any such adjustments would be recorded on a cumulative catch-up basis, which would affect revenues and earnings in the period of adjustment.

For arrangements that include sales-based royalties, including milestone payments based on the level of sales, the Company recognizes revenue at the later of (i) when or as the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied).

Accounting for these arrangements requires the Company to develop assumptions that require judgment to determine the stand-alone selling price of each performance obligation identified in the contract. The Company has never sold the performance obligations in its collaborative arrangements separately; therefore, an observable stand-alone selling price does not exist. Accordingly, the Company estimates a stand-alone selling price through maximizing the use of observable inputs such as market data, project cost estimates, and targeted margins.

Cost of Sales

Prior to receiving approval from the U.S. Food and Drug Administration ("FDA") or other foreign regulatory authorities for a new medicine or new formulation, the Company expenses all costs incurred related to the manufacture of such medicines as research and development expense because of the inherent risks associated with the development of a drug candidate, the uncertainty about the regulatory approval process and the lack of history for the Company of regulatory approval of drug candidates. Subsequent to receiving FDA or other foreign regulatory authority approval, when commercialization is considered probable and the future economic benefit is expected to be realized, the Company begins capitalizing inventory costs incurred.

Cost of sales consist of manufacturing costs, transportation and freight, amortization of capitalized intangible assets, royalties and direct and indirect overhead costs associated with the manufacturing and distribution of the Company's approved products. Cost of sales may also include period costs related to certain manufacturing services and inventory adjustment charges.

Research and Development Expenses

Research and development expenses consists primarily of fees paid to contract research organizations and other vendors for clinical, non-clinical and manufacturing services, salaries and employee benefits, including stock-based compensation, consultant expenses, costs related to acquiring manufacturing materials, costs related to compliance with regulatory requirements and license payments related to acquiring intellectual property rights for the Company's product candidates. Research and development expenses are expensed as incurred.

Selling, General and Administrative Expenses

Selling, general and administrative ("SG&A") expenses relate to sales and marketing, finance, human resources, legal and other administrative activities. SG&A expenses consist primarily of personnel costs, facilities and overhead costs, outside marketing, advertising and legal expenses, and other general and administrative costs.

The Company expenses the costs of advertising, including promotional expenses, as incurred. Advertising expenses were $10.9 million, $11.5 million and $6.3 million for the years ended December 31, 2025, 2024 and 2023, respectively.

Stock-Based Compensation

The Company recognizes stock-based compensation for all stock-based awards based on the grant date fair value of the award granted to employees and nonemployees, including members of its board of directors. For stock-based awards with service conditions, the fair value of the awards is recognized on a straight-line basis over the requisite service period in which the awards are expected to vest. For stock-based awards with performance vesting conditions, stock-based compensation is recognized when it is considered probable that the performance conditions will be satisfied. At each reporting period, the Company reassesses the probability of the achievement of the performance vesting conditions. Any change in stock-based compensation resulting from an adjustment in the vesting is treated as a cumulative catch-up in the period of adjustment. For stock-based awards with market conditions, stock-based compensation is recognized over the appropriate requisite service period. The Company accounts for forfeitures as they occur.

Foreign Currency

The consolidated financial statements are presented in U.S. dollars. The functional currency for most of the Company's foreign subsidiaries is their local currency. Balance sheet accounts of international subsidiaries are translated at the current exchange rates as of the end of each accounting period. Income statement items are translated at average exchange rates for the period. The resulting translation adjustments are recorded as a separate component of stockholders' equity.

Foreign currency transaction gains and losses are included in other income (expense), net in the consolidated statements of operations. Transaction gains and losses result primarily from fluctuations in exchange rates when intercompany receivables and payables are denominated in currencies other than the functional currency of its subsidiary that recorded the transaction. Unrealized foreign exchange gains and losses amounted to a $1.7 million and a $1.2 million gain for the years ended December 31, 2025 and 2024, respectively, and $1.8 million loss for the year ended December 31, 2023. Realized foreign exchange losses amounted to a $1.0 million loss for the year ended December 31, 2025 and were insignificant for the years ended December 31, 2024 and 2023.

Income Taxes

Income taxes are recorded using the liability method, under which deferred tax assets and liabilities are determined based on differences between the financial reporting and tax basis of assets and liabilities and are measured using the enacted tax rates and laws that will be in effect when the differences are expected to reverse. Valuation allowances are recorded against deferred tax assets, including net operating losses and tax credits, when it is determined it is more-likely-than-not that some or all of the tax benefits will not be realized.

The Company accounts for uncertain tax positions in accordance with the provisions of Financial Accounting Standards Board ("FASB") Accounting Standards Codification No. 740, *Income Taxes* ("ASC 740"). When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit would more likely than not be realized assuming examination by the taxing authority. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances.

Interest and penalties related to unrecognized tax benefits, if any, are recorded as a component of income tax expense.

Net Loss Per Share

Basic net loss per share is computed by dividing net loss by the weighted-average shares of common stock outstanding for the period, without consideration for potentially dilutive securities. Diluted net loss per share is computed by dividing the net loss by the weighted-average shares of common stock and potentially dilutive securities outstanding for the period determined using the treasury-stock and if-converted methods. Diluted net loss per share excludes the potential impact of the Company's common stock subject to repurchase, common stock options, restricted stock units, contingently issuable employee stock purchase plan shares and common stock issuable upon conversion of convertible notes because their effect would be anti-dilutive due to the Company's net loss.

The following outstanding potential dilutive shares have been excluded from the calculation of diluted net loss per share for the periods presented due to their anti-dilutive effect:

	As of December 31,		
	2025	2024	2023
Options to purchase common stock and restricted stock units	10,728,153	11,709,003	10,909,831
Common stock issuable upon conversion of convertible notes	9,963,490	9,964,247	9,964,247
Employee stock purchase plan contingently issuable	17,257	19,580	23,054
Total	20,708,900	21,692,830	20,897,132

Recently Adopted Accounting Pronouncements

In December 2023, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2023-09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures* ("ASU 2023-09"). This new guidance is designed to enhance the transparency and decision usefulness of income tax disclosures. The amendments of this update are related to the rate reconciliation and income taxes paid. ASU 2023-09 is effective for fiscal years beginning after December 15, 2024. The Company adopted ASU 2023-09 in 2025 using a prospective method. For further information, refer to Note 13 *Income Taxes*.

Recent Accounting Pronouncements Not Yet Adopted

From time to time, new accounting pronouncements are issued by the FASB or other standard setting bodies and adopted by the Company as of the specified effective date. 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 the accompanying consolidated financial statements and disclosures.

In November 2024, the FASB issued ASU 2024-03, *Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses* ("ASU 2024-03"), which requires disclosure of disaggregated information about specific categories underlying certain income statement expense line items in the footnotes to the financial statements for both annual and interim periods. Subsequently in January 2025, the FASB issued ASU 2025-01, *Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Clarifying the Effective Date* ("ASU 2025-01") to clarify the effective date of ASU 2024-03. ASU 2024-03 is effective for fiscal years beginning after December 15, 2026, and interim periods within annual reporting periods beginning after December 15, 2027. Early adoption is permitted. The Company is currently assessing the impact that adopting this new accounting standard will have on its consolidated financial statements.

In July 2025, the FASB issued ASU 2025-05, *Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses for Accounts Receivable and Contract Assets* ("ASU 2025-05"). The new guidance provides a practical expedient related to the estimation of expected credit losses for current accounts receivable and current contract assets that arise from transactions accounted for under FASB Accounting Standards Codification 606. ASU 2025-05 is effective for annual reporting periods beginning after December 15, 2025, and interim reporting periods within those annual reporting periods. Early adoption is permitted. The Company does not expect that adoption of this new accounting standard will have a material impact on its consolidated financial statements.

In September 2025, the FASB issued ASU 2025-06, *Intangibles - Goodwill and Other - Internal-Use Software (Subtopic 350-40): Targeted Improvements to the Accounting for Internal-Use Software* ("ASU 2025-06"), to modernize the accounting guidance for the costs to develop software for internal use by aligning it with current development practices, especially agile and iterative methods. The guidance removes references to development stages and clarifies when capitalization of software costs should occur. The new guidance also clarifies disclosure requirements for all capitalized internal-use software costs, ASU 2025-06 is effective for annual reporting periods beginning after December 15, 2027, and interim reporting periods within those annual reporting periods. Early adoption is permitted as of the beginning of an annual reporting period. The Company is currently assessing the impact that adopting this new accounting standard will have on its consolidated financial statements.

3. Fair Value Measurements

Financial assets and liabilities subject to fair value measurements on a recurring basis and the level of inputs used in such measurements by major security type are presented in the following table (in thousands):

	December 31, 2025			
	Level 1	Level 2	Level 3	Total
Financial assets:				
Money market funds	$ 254,376	$ —	$ —	$ 254,376
Corporate debt securities	—	72,277	—	72,277
U.S. government bonds	—	17,016	—	17,016
Commercial paper	—	5,456	—	5,456
Total financial assets	$ 254,376	$ 94,749	$ —	$ 349,125

	December 31, 2024			
	Level 1	Level 2	Level 3	Total
Financial assets:				
Money market funds	$ 202,965	$ —	$ —	$ 202,965
U.S. treasury bills	$ 2,964	$ —	$ —	$ 2,964
Corporate debt securities	$ —	$ 37,978	$ —	$ 37,978
U.S. government bonds	$ —	$ 26,904	$ —	$ 26,904
Agency bonds	$ —	$ 2,492	$ —	$ 2,492
Total financial assets	$ 205,929	$ 67,374	$ —	$ 273,303

The carrying amounts of certain financial instruments such as cash and cash equivalents, restricted cash, accounts receivable, prepaid expenses, other current assets, accounts payable and accrued expenses as of December 31, 2025 and 2024 approximate their related fair values due to their short-term nature.

Money market funds and U.S. treasury bills are highly liquid investments and are actively traded. The pricing information on these investment instruments is readily available and can be independently validated as of the measurement date. This approach results in the classification of these securities as Level 1 of the fair value hierarchy.

Certain financial instruments classified within Level 2 of the fair value hierarchy include the types of instruments that trade in markets that are not considered to be active, but are valued based on quoted market prices, broker or dealer quotations, or alternative pricing sources with reasonable levels of price transparency. The Company reviews trading activity and pricing for these investments as of each measurement date.

4. Financial Instruments

The fair value and amortized cost of cash equivalents and available-for-sale investments by major security type are presented in the following table (in thousands):

	December 31, 2025			
	Amortized Cost	Unrealized Gain	Unrealized Loss	Estimated Fair Value
Cash equivalents and investments:				
Money market funds	$ 254,376	$ —	$ —	$ 254,376
Corporate debt securities	72,170	107	—	72,277
U.S. government bonds	16,984	32	—	17,016
Commercial paper	5,455	1	—	5,456
Total cash equivalents and investments	$ 348,985	$ 140	$ —	$ 349,125
Classified as:				
Cash equivalents				$ 254,376
Short-term investments				86,644
Long-term investments				8,105
Total cash equivalents and investments				$ 349,125

	December 31, 2024			
	Amortized Cost	Unrealized Gain	Unrealized Loss	Estimated Fair Value
Cash equivalents:				
Money market funds	$ 202,965	$ —	$ —	$ 202,965
U.S. treasury bills	$ 2,957	$ 7	$ —	$ 2,964
Corporate debt securities	$ 37,942	$ 50	$ (14)	$ 37,978
U.S. government bonds	$ 26,819	$ 90	$ (5)	$ 26,904
Agency bonds	$ 2,490	$ 2	$ —	$ 2,492
Total cash equivalents and investments	$ 273,173	$ 149	$ (19)	$ 273,303
Classified as:				
Cash equivalents				$ 202,965
Short-term investments				57,812
Long-term investments				12,526
Total cash equivalents and investments				$ 273,303

As of December 31, 2025, the remaining contractual maturities of available-for-sale debt securities were as follows (in thousands):

	Estimated Fair Value
Due within one year	$ 86,644
One to two years	8,105
Total	$ 94,749

During the years ended December 31, 2025 and 2024, there have been no significant realized gains or losses on available-for-sale investments, no investments have been in a continuous unrealized loss position for more than 12 months, and the Company did not recognize any credit losses on these securities.

5. Balance Sheet Components

Inventory

Inventory consists of the following (in thousands):

	December 31,	
	2025	2024
Raw materials	$ 2,898	$ 3,030
Work in progress	18,473	16,089
Finished goods	3,516	3,284
Total inventory	$ 24,887	$ 22,403

Intangible Assets, Net

The components of the Company's intangible assets were as follows (in thousands, except for weighted-average remaining amortization period):

	December 31, 2025			
	Gross Carrying Value	Accumulated Amortization	Net Carrying Amount	Weighted-Average Remaining Amortization Period (Years)
Commercial milestones	$ 94,000	$ (11,660)	$ 82,340	11.5
Developed technology	226,620	(48,255)	178,365	9.7
Assembled workforce	970	(754)	216	0.7
Total intangible assets	$ 321,590	$ (60,669)	$ 260,921	10.3

	December 31, 2024			
	Gross Carrying Value	Accumulated Amortization	Net Carrying Amount	Weighted-Average Remaining Amortization Period (Years)
Commercial milestones	$ 59,000	$ (7,092)	$ 51,908	12.6
Developed technology	226,620	(29,248)	197,372	10.7
Assembled workforce	970	(431)	539	1.7
Total intangible assets	$ 286,590	$ (36,771)	$ 249,819	11.1

Amortization expense was $23.9 million, $23.1 million and $10.5 million for the years ended December 31, 2025, 2024 and 2023, respectively, and was included in cost of sales on the accompanying consolidated statements of operations.

The following table summarizes the estimated future amortization expense associated with the Company's intangible assets as of December 31, 2025 (in thousands):

	Amount
2026	$ 26,958
2027	26,742
2028	26,742
2029	26,742
2030	26,742
Thereafter	126,995
	$ 260,921

Accrued Expenses

Accrued expenses consist of the following (in thousands):

	December 31,	
	2025	2024
Accrued sales deductions	$ 51,375	$ 37,361
Accrued compensation and related benefits	47,276	32,248
Accrued royalties payable	16,208	9,361
Accrued professional service fees	17,292	12,545
Accrued clinical trials	13,959	7,148
Accrued contract manufacturing and non-clinical costs	8,721	7,555
Accrued loss on firm purchase commitments	2,107	1,898
Operating lease liabilities, current	2,139	1,709
Accrued interest	2,108	2,108
Accrued milestone payment	35,000	—
Total accrued expenses and other current liabilities	$ 196,185	$ 111,933

Other liabilities

Other liabilities consist of the following (in thousands):

	December 31,	
	2025	2024
Noncurrent accrued sales deductions and other	$ 3,586	$ —
Noncurrent accrued loss on firm purchase commitments	1,425	2,509
Total other liabilities	$ 5,011	$ 2,509

6. Revenue Interest Purchase Agreement

In December 2020, the Company entered into the RIPA, as amended in September 2021, with Oberland as agent for the Purchasers, and the Purchasers to obtain financing for the commercialization and further development of Livmarli and other working capital needs. Pursuant to the RIPA, the Company received a total of $115.0 million.

As consideration for such payments, the Purchasers had the right to receive the Revenue Interests from the Company based on annual product sales, net of Livmarli.

Under the RIPA, the Company had an option (the "Call Option") to terminate the RIPA and repurchase future Revenue Interests at any time upon advance written notice. If the Call Option was exercised, the required repurchase price is 175.0% of the Cumulative Purchaser Payments (minus all payments the Company has made to the Purchasers in connection with the Revenue Interests), if such option was exercised prior to the third anniversary of the closing date, and 195.0% of the Cumulative Purchaser Payments (minus all payments Company has made to the Purchasers in connection with the Revenue Interests) if such option was exercised thereafter.

The Company recorded interest expense related to this arrangement of $5.1 million for the year ended December 31, 2023. No interest expense related to this arrangement was recorded for the years ended December 31, 2025 and 2024.

In April 2023, the Company exercised the Call Option and repurchased all future Revenue Interests. In connection with such repurchase, the Company made a payment of $192.7 million, or 175.0% of the Cumulative Purchaser Payments (minus all payments that were made to the Purchasers in connection with the Revenue Interests). As a result, the RIPA terminated in accordance with its terms.

The net loss from termination of the RIPA of $49.1 million, comprised of the $50.2 million loss related to the settlement of the revenue interest liability and the $1.1 million gain on the derecognition of the related derivative liability, was recorded in the accompanying consolidated statements of operations. As of December 31, 2025 and 2024, there were no outstanding balances related to the revenue interest liability and the derivative liability.

7. Asset Acquisitions

License Agreement with Enthorin Therapeutics, LLC

In October 2024, the Company entered into a license agreement with Enthorin Therapeutics, LLC and Dart Neuroscience LLC ("License Agreement"), under which the Company acquired the worldwide rights to develop, manufacture and commercialize a compound designated as ENT-3379, renamed MRM-3379, in exchange for an upfront payment of $7.5 million and up to an additional $217.5 million upon the achievement of regulatory and sales-based milestones as well as mid-single digit percent royalties on any future sales of MRM-3379.

The Company accounted for the transaction as an asset acquisition as the set of acquired assets did not constitute a business and substantially all the fair value of the gross assets acquired was concentrated in a group of similar identifiable assets, namely, the intellectual property related to MRM-3379. The Company determined that the intellectual property assets acquired did not have an alternate future use and were not deemed to be commercially viable. Therefore, the upfront payment of $7.5 million was recorded as research and development expense in the year ended December 31, 2024.

Through December 31, 2025, no milestones have been achieved.

Asset Purchase Agreement with Travere Therapeutics, Inc.

In August 2023, the Company completed the Bile Acid Portfolio Acquisition. In accordance with the terms and conditions of the Asset Purchase Agreement entered into with Travere, the Company purchased from Travere substantially all of the assets related to its business of development, manufacturing (including synthesis, formulation, finishing or packaging) and commercialization of the Bile Acid Medicines. The Company paid $210.4 million upon closing of the transaction, and up to an additional $235.0 million is payable upon the achievement of certain milestones based on specified amounts of annual net sales of the Bile Acid Medicines. In connection with the Bile Acid Portfolio Acquisition, the Company recorded $198.5 million of developed technology intangible assets on the consolidated balance sheet at the time of the acquisition. As of December 31, 2025, the Company accrued $25.0 million for the achievement of product sales milestones associated with achievement of certain net product sales, which was recognized as an intangible asset in the accompanying consolidated balance sheet as of December 31, 2025. Through December 31, 2025, the Company paid or accrued $25.0 million for the achievement of product sales milestones associated with this agreement.

The Company is obligated to pay tiered royalties, based on licensing agreements acquired with the Bile Acid Medicines, with rates ranging from high single digit to mid-teens based on net sales of the Bile Acid Medicines.

Assignment and License Agreement with Shire International GmbH (Takeda)

In November 2018, the Company entered into an Assignment and License Agreement (the "Shire Agreement") with Shire International GmbH ("Shire"), which was subsequently acquired by Takeda Pharmaceutical Company Limited ("Takeda"). Under the terms of the Shire Agreement, Shire granted the Company an exclusive, royalty bearing worldwide license to develop and commercialize its two product candidates, Livmarli and volixibat. As part of the Shire Agreement, the Company was assigned license agreements held by Shire with Satiogen, Pfizer Inc. ("Pfizer") and Sanofi-Aventis Deutschland GmbH ("Sanofi"). The Company has the right to sublicense under the Shire Agreement and additionally has the right to sublicense under the Satiogen, Pfizer and Sanofi licenses subject to the terms of those license agreements.

The Company is obligated to pay Shire up to an aggregate of $109.5 million upon the achievement of certain clinical development and regulatory milestones for Livmarli in certain indications and an additional $25.0 million upon regulatory approval of Livmarli for each and every other indication. In addition, the Company is required to pay up to an aggregate of $30.0 million upon the achievement of certain clinical development and regulatory milestones for volixibat solely for the first indication sought. Upon commercialization, the Company is obligated to pay Shire commercial milestones on total licensed products up to an aggregate of $30.0 million. Through December 31, 2025 under this agreement, the Company paid or accrued $101.5 million for the achievement of various clinical development, regulatory and commercial milestones.

The Company is also obligated to pay tiered royalties with rates ranging from low double-digits to mid-teens based upon annual worldwide net sales for all licensed products; however, these royalties are reduced in part by royalties due under the Satiogen and Sanofi licenses, as discussed below, related to Livmarli and volixibat, as applicable. The Company's royalty obligations will continue on a licensed product-by-licensed product and country-by-country basis until the later to occur of the expiration of the last valid claim in a licensed patent covering the applicable licensed product in such country, expiration of any regulatory exclusivity for the licensed product in a country and ten years after the first commercial sale of a licensed product in such country.

In April 2024, the Company paid a $10.0 million milestone associated with the approval of Livmarli for the treatment of cholestatic pruritus in patients with PFIC five years of age and older (now twelve months of age and older) by the FDA. In July 2024, the Company paid a $10.0 million milestone associated with the approval of Livmarli for the treatment of PFIC in patients three months and older by the European Medicines Agency. In February 2025, the Company achieved a $5.0 million development milestone related to maralixibat, which was recognized in research and development expense in the accompanying consolidated statements of operations. In addition, as of December 31, 2025, the Company accrued $10.0 million for the achievement of a commercial milestone associated with product sales, which was recognized as an intangible asset in the accompanying consolidated balance sheet as of December 31, 2025. There were no volixibat development and regulatory milestones achieved during the years ended December 31, 2025, 2024 and 2023.

Pfizer License

Through the Shire Agreement, the Company was assigned a license agreement with Pfizer pursuant to which the Company obtained an exclusive, worldwide license to certain Pfizer know-how with a right to sublicense. Upon commercialization of any product utilizing the licensed product, the Company is required to pay to Pfizer a low single-digit royalty on net sales of product sold by the Company, its affiliates or sublicensees. The Company's royalty obligations continue on a licensed product-by-licensed product basis until the eighth anniversary of the first commercial sale of such licensed product anywhere in the world.

Sanofi License

Through the Shire Agreement, the Company was assigned a license agreement with Sanofi pursuant to which the Company obtained an exclusive, worldwide license to certain patents and know-how with the right to sublicense to a third party subject to certain financial considerations. The Company is obligated to pay up to an aggregate of $36.0 million upon the achievement of certain regulatory, commercialization and product sales milestones. Additionally, upon commercialization, the Company is required to pay tiered royalties in the mid to high single-digit range based upon net sales of licensed products sold by the Company and sublicensees in a calendar year, subject to adjustments in certain circumstances. The Company's royalty obligations continue on a licensed product-by-licensed product and country-by-country basis until the later to occur of the expiration of the last valid claim in a licensed patent covering the applicable licensed product in such country and ten years after the first commercial sale of a licensed product in such country. Royalty obligations under the Sanofi license are creditable against the royalty obligations to Shire under the Shire Agreement. The Company has not paid milestone payments pursuant to this agreement for the periods presented. Through December 31, 2025, no milestones have been achieved.

8. Collaboration and License Agreements

Licensing Agreement with Takeda

In September 2021, the Company entered into an exclusive licensing agreement with Takeda for the development and commercialization of Livmarli in Japan for ALGS, PFIC, and BA. Further, in October 2024, the parties entered into a commercial supply agreement. Under the terms of the agreements, Takeda is responsible for development and commercialization of Livmarli for licensed indications in Japan, while the Company is responsible for commercial supply to Takeda. In accordance with the agreements, in exchange for commercial inventory supply, the Company is eligible to receive a percentage of Takeda's net sales, which range from high double digits declining to mid double digits over the first four years from commercial launch and thereafter remains at mid double digits. The Company records net product sales at the time control of the product is transferred, based on the estimated variable consideration.

9. Leases

The Company has operating leases for office spaces in various global locations, including its headquarters in Foster City, California, the lease for which was entered into in January 2024 with an initial lease term of approximately five years (the "Initial Lease"). In January 2026, the Company amended the Initial Lease (the "Amended Agreement") to expand its headquarters office space by approximately 19,400 square feet (the "Expanded Space") with a lease term of approximately five years from the commencement date. The Amended Agreement extends the lease term of the Initial Lease to be coterminous with the Expanded Space. The addition of the expanded space will result in approximately $10.6 million of additional base rent compared to the remaining payments of the Initial Lease.

In March 2025, the Company entered into an operating lease agreement for office space at an international location. The lease commenced in the second quarter of 2025 and has a lease term of approximately five years. In December 2025, the Company amended the terms of a lease at another international location to include additional space with a five-year lease term expiring January 2031 and a commencement date of December 2025. In addition, the amendment extended the lease term of the existing space to the same term.

The following tables contain a summary of other information and the undiscounted future minimum payments pertaining to the Company's operating leases that had commenced as of the end of the periods presented:

	December 31, 2025
Weighted-average incremental borrowing rate	7.4%
Weighted-average remaining lease term (in years)	4.0 years

Years Ended December 31,	Undiscounted Rent Payments (in thousands)
2026	$ 2,771
2027	2,856
2028	2,922
2029	2,201
2030	351
Thereafter	21
Total undiscounted lease payments	11,122
Less: imputed interest	(1,467)
Total lease liability	$ 9,655

Rent expense was $2.4 million, $1.8 million and $0.8 million for the years ended December 31, 2025, 2024 and 2023, respectively. Variable lease payments for the years ended December 31, 2025, 2024 and 2023 were insignificant.

10. Convertible Notes

In April 2023, the Company issued $316.3 million aggregate principal amount of its 4.00% Convertible Senior Notes due 2029 (the "Notes") in a private placement to qualified institutional buyers pursuant to Rule 144A under the Securities Act of 1933, as amended. The Notes are subject to the terms and conditions of an Indenture (the "Indenture") between the Company and U.S. Bank Trust Company, National Association, as trustee.

The net proceeds from the issuance of the Notes were $305.3 million, after deducting the initial purchasers' discounts and commissions and offering expenses.

The Notes are the Company's senior, unsecured obligations and are (i) senior in right of payment to any of the Company's indebtedness that is expressly subordinated to the Notes in right of payment; (ii) equal in right of payment to any of the Company's indebtedness that is not so subordinated; (iii) effectively subordinated to any of the Company's secured indebtedness, to the extent of the value of the collateral securing that indebtedness; and (iv) structurally subordinated to all indebtedness and other liabilities, including trade payables, and (to the extent the Company is not a holder thereof) preferred equity, if any, of the Company's subsidiaries.

The Notes accrue interest at a rate of 4.00% per annum, payable semi-annually in arrears on May 1 and November 1 of each year, beginning on November 1, 2023. The Notes will mature on May 1, 2029, unless earlier converted, redeemed or repurchased by the Company. Before January 2, 2029, noteholders will have the right to convert their Notes only in the following circumstances:

(i) during any calendar quarter (and only during such calendar quarter) commencing after the calendar quarter ending on June 30, 2023, if the last reported sale price per share of the Company's common stock exceeds 130% of the conversion price for the Notes for each of at least 20 trading days, whether or not consecutive, during the 30 consecutive trading days ending on, and including, the last trading day of the immediately preceding calendar quarter;

(ii) 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 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 common stock on such trading day and the conversion rate on such trading day;

(iii) upon the occurrence of certain corporate events or distributions on the common stock, as described in the Indenture; and

(iv) if the Company calls such Notes for redemption.

Additionally, noteholders will have the right to convert their Notes at any time from January 2, 2029 until the close of business on the scheduled trading day immediately before the maturity date. The Company may settle conversions by paying or delivering, as applicable, cash, shares of its common stock or a combination of cash and shares of its common stock, at the Company's election. The initial conversion rate for the Notes is 31.5075 shares of common stock per $1,000 principal amount of Notes, which represents an initial conversion price of approximately $31.74 per share of common stock. The conversion rate and conversion price will be subject to customary adjustments upon the occurrence of certain events. In addition, if certain corporate events that constitute a "Make-Whole Fundamental Change" (as defined in the Indenture) occur, then the conversion rate will, in certain circumstances, be increased for a specified period of time.

During the fourth quarter of 2025, the last reported sale price of the Company's common stock exceeded 130% of the conversion price of the Notes for more than 20 trading days during the 30 consecutive trading days ended December 31, 2025. As a result, the Notes are convertible at the option of the holders of the Notes during the first quarter of 2026, the quarter immediately following the quarter when the conditions were met, as stated in the terms of the Notes.

The Company may not redeem the Notes at its option at any time before May 5, 2026. The Notes are redeemable, in whole or in part (subject to the partial redemption limitation described below), at the Company's option at any time, and from time to time, on or after May 5, 2026 and, in the case of a partial redemption, on or before the 50th scheduled trading day immediately before the maturity date, at a cash redemption price equal to the principal amount of the 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 (i) 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 (ii) the trading day immediately before the date the Company sends such notice. In addition, calling any Note for redemption will constitute a Make-Whole Fundamental Change with respect to that Note, in which case the conversion rate applicable to the conversion of that Note will be increased in certain circumstances if it is converted after it is called for redemption. Pursuant to the partial redemption limitation, the Company may not elect to redeem less than all of the outstanding Notes unless at least $75.0 million aggregate principal amount of Notes are outstanding and not called for redemption as of the time the Company sends the related redemption notice.

If a "Fundamental Change" (as defined in the Indenture) occurs, then, subject to a limited exception for certain cash mergers, noteholders may require the Company to repurchase their Notes at a cash repurchase price equal to the principal amount of the Notes to be repurchased, plus accrued and unpaid interest, if any, to, but excluding, the fundamental change repurchase date. The definition of Fundamental Change includes certain business combination transactions involving the Company and certain de-listing events with respect to the Company's common stock.

The Indenture contains customary events of default with respect to the Notes and provides that upon certain events of default occurring and continuing, the Trustee may, and the Trustee at the request of holders of at least 25% in principal amount of the Notes shall, declare all principal and accrued and unpaid interest, if any, of the Notes to be due and payable. In case of certain events of bankruptcy, insolvency or reorganization, involving the Company or a significant subsidiary, all of the principal of and accrued and unpaid interest on the Notes will automatically become due and payable.

As of December 31, 2025 and 2024, the Notes consisted of the following (in thousands):

	December 31,	
	2025	2024
Principal amount	$ 316,226	$ 316,250
Unamortized debt discount and issuance costs	(6,429)	(8,168)
Net carrying amount	$ 309,797	$ 308,082

The Company incurred $10.9 million of transaction costs related to the issuance of the Notes, which are being amortized to interest expense over the term of the Notes using the effective interest method. As of December 31, 2025, the

remaining amortization period of the debt discount was approximately 3.3 years and the effective interest on the Notes was 4.6%. The following table sets forth interest expense recognized related to the Notes (in thousands):

	Year Ended December 31,		
	2025	2024	2023
Coupon interest expense	$ 12,650	$ 12,650	$ 8,925
Amortization of debt discount and issuance costs	1,739	1,661	1,120
Total interest expense on convertible notes	$ 14,389	$ 14,311	$ 10,045

As of December 31, 2025 and 2024, the estimated fair value of the Notes was $818.5 million and $481.9 million, respectively. The fair values were determined based on the quoted price of the convertible notes in an inactive market on the last trading day of the reporting period and has been classified as Level 2 in the fair value hierarchy.

11. Stockholders' Equity

Common Stock

In August 2025, the Company filed an automatic shelf registration statement on Form S-3 with the SEC (the "2025 Shelf Registration"), which became effective upon filing, pursuant to which the Company may register for sale from time to time in one or more offerings an unlimited amount of any combination of the Company's common stock, preferred stock, debt securities and warrants, so long as the Company continues to satisfy the requirements of a "well-known seasoned issuer" under SEC rules. This automatic shelf registration statement will remain in effect for up to three years from the date it became effective. As of December 31, 2025, the Company had not issued any securities pursuant to the 2025 Shelf Registration.

In November 2023, the Company entered into a Sales Agreement (the "2023 Sales Agreement") with Leerink and Cantor Fitzgerald & Co. (the "Sales Agents"), pursuant to which the Company may, from time to time, sell up to an aggregate amount of $200.0 million of its common stock through the Sales Agents in an "at-the-market" offering (the "ATM Offering"). The Company is not required to sell shares under the 2023 Sales Agreement. Sales of the Company's common stock, if any, under the 2023 Sales Agreement may be made in any transactions that are deemed to be "at the market offerings" as defined in Rule 415 under the Securities Act. The Company will pay a given designated Sales Agent a commission of up to 3.0% of the aggregate gross proceeds of any shares of common stock sold through such Sales Agent pursuant to the 2023 Sales Agreement. As of December 31, 2025, the Company had not issued any securities pursuant to the 2023 Sales Agreement.

In August 2023, in connection with and immediately prior to the closing of the Bile Acid Portfolio Acquisition, the Company completed the private placement of 8,000,000 shares of the Company's common stock at a price per share of $26.25, resulting in net proceeds of approximately $202.2 million, which the Company used to finance the upfront payment at the closing of the Bile Acid Portfolio Acquisition.

Common Stock Reserved for Issuance

Common stock reserved for issuance is as follows:

	Year Ended December 31,	
	2025	2024
Stock options, restricted stock units and performance stock units issued and outstanding	10,728,153	11,709,003
Reserved for future stock awards or option grants	3,724,927	2,277,739
Reserved for employee stock purchase plan	1,759,019	1,382,616
Common stock issuable upon conversion of convertible notes	9,963,490	9,964,247
	26,175,589	25,333,605

12. Stock-Based Compensation

Equity Incentive Plans

In November 2018, the Company adopted the 2018 Equity Incentive Plan (the "2018 Plan"), which permits the granting of stock awards and incentive and nonstatutory stock options to employees, directors and consultants of the Company.

In July 2019, the Company's board of directors and stockholders approved and adopted the 2019 Equity Incentive Plan (the "2019 Plan"). The 2019 Plan became effective on July 17, 2019. Under the 2019 Plan, the Company may grant stock options, stock appreciation rights, restricted stock, restricted stock units and other stock or cash-based awards to individuals who are then employees, officers, directors or consultants of the Company. Shares subject to outstanding awards under the 2018 Plan as of the effective date of the 2019 Plan that are subsequently canceled, forfeited or repurchased by the Company will be added to the shares reserved under the 2019 Plan. In addition, the number of shares of common stock available for issuance under the 2019 Plan will be automatically increased on the first day of each calendar year during the ten-year term of the 2019 Plan, beginning with January 1, 2020 and ending with January 1, 2029, by an amount equal to 5% of the outstanding number of shares of the Company's common stock on December 31st of the preceding calendar year or such lesser amount as determined by the Company's board of directors. As of December 31, 2025, 2,285,219 shares of common stock were available for issuance under the 2019 Plan.

In March 2020, the compensation committee of the Company's board of directors approved and adopted the 2020 Inducement Plan (the "2020 Inducement Plan"). Under the 2020 Inducement Plan, the Company may grant nonstatutory stock options, stock appreciation rights, restricted stock and restricted stock units to new employees entering into employment with the Company in accordance with Nasdaq Listing Rule 5635(c)(4). Through December 31, 2025, the Company's board of directors authorized 5,500,000 shares of the Company's common stock for future issuance. As of December 31, 2025, 1,439,708 shares of common stock were available for issuance under the 2020 Inducement Plan.

Stock Options

The following table summarizes stock option activity during the year ended December 31, 2025 (in thousands, except share and per share data):

	Number of Awards	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life (in Years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2024	10,031,486	$ 18.12	6.5	$ 233,161
Granted	1,859,835	$ 49.25		
Exercised	(2,658,854)	$ 13.62		
Canceled and forfeited	(273,976)	$ 34.93		
Outstanding as of December 31, 2025	8,958,491	$ 25.41	6.5	$ 479,984
Vested and exercisable as of December 31, 2025	5,747,724	$ 17.61	5.4	$ 352,815

Intrinsic value is calculated as the difference between the exercise price of the underlying options and the fair value of the common stock at December 31, 2025 or on the date of exercise, for the options that had exercise prices that were lower than the per share fair value of the common stock on the date of exercise. The weighted-average grant date fair value per share of stock options granted during the years ended December 31, 2025, 2024 and 2023 was $32.71, $20.02 and $18.25 per share, respectively. The total intrinsic value of options exercised during the years ended December 31, 2025, 2024 and 2023 was $123.7 million, $23.1 million and $5.7 million, respectively. As of December 31, 2025, the total unrecognized stock-based compensation related to unvested stock option awards granted was $70.3 million, which the Company expects to recognize over a weighted-average period of approximately 2.7 years.

The fair value of each employee and non-employee stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. Due to the Company's limited operating history and a lack of company specific historical and implied volatility data, the expected stock price volatility was based upon the weighting of the Company's historical volatility and the historical volatility of a peer group of publicly traded companies. The historical volatility data was computed using the daily closing prices for the Company's and its peer companies' shares during the equivalent period of the calculated expected term of the stock-based awards. Due to the lack of historical exercise history, the expected term of the Company's stock options for employees has been determined utilizing the "simplified" method for awards. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is zero based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

The following assumptions were used to estimate the fair value of stock option awards granted during the following periods:

	Year Ended December 31,		
	2025	2024	2023
Expected term (in years)	5.5-6.1	5.5-6.1	5.3-6.1
Expected volatility	67.82%-71.31%	71.79%-80.36%	80.17%-85.24%
Risk-free interest rate	3.69%-4.65%	3.48%-4.62%	3.35%-4.67%
Expected dividend yield	—	—	—

Restricted Stock Units

The following table summarizes the activity under the Company's restricted stock units for the year ended December 31, 2025:

	Number of Awards	Weighted-Average Grant Date Fair Value per Award
Unvested and outstanding as of December 31, 2024	1,392,562	$ 26.63
Granted	787,765	$ 49.47
Vested	(644,037)	$ 25.29
Cancelled/Forfeited	(120,371)	$ 36.84
Unvested and outstanding as of December 31, 2025	1,415,919	$ 39.08

The fair value of restricted stock unit ("RSU") awards granted to employees and nonemployees is equal to the closing market price of the Company's common stock on the grant date.

As of December 31, 2025, the total unrecognized stock-based compensation related to restricted stock unit awards granted was $35.6 million, which the Company expects to recognize over a weighted-average period of approximately 1.9 years.

Performance Stock Units

The fair value of performance stock units ("PSUs") granted to employees is equal to the closing market price of the Company's common stock on the grant date. PSUs are subject to vest only if certain specified sales-based criteria are achieved and the employees' continued service with the Company. As of December 31, 2025, certain specified sales-based criteria were deemed probable of achievement or already achieved. Stock-based compensation for PSUs is recognized over the service period beginning in the period the Company determines it is probable that the performance criteria will be achieved. PSUs generally vest over a three-year service period. The number of shares earned is adjusted based on the specified sales-based criteria achievement.

The following table summarizes the activity under the Company's performance stock units in base units for the year ended December 31, 2025:

	Number of Awards	Weighted-Average Grant Date Fair Value per Award
Unvested and outstanding as of December 31, 2024	284,955	$ 25.15
Granted	217,547	$ 40.18
Vested	(147,675)	$ 23.92
Cancelled/Forfeited	(1,084)	$ 25.39
Unvested and outstanding as of December 31, 2025	353,743	$ 34.91

As of December 31, 2025, the total unrecognized stock-based compensation related to performance stock units granted was $9.0 million, which the Company expects to recognize over a weighted-average period of approximately 1.7 years.

2019 Employee Stock Purchase Plan

In July 2019, the Company's board of directors and stockholders approved and adopted the 2019 Employee Stock Purchase Plan ("ESPP"). During the year ended December 31, 2025, 106,977 shares were issued under the ESPP. As of December 31, 2025, the Company had 1,759,019 shares available for future issuance under the ESPP.

Stock-Based Compensation Expense

Total stock-based compensation is reflected in the accompanying consolidated statements of operations as follows (in thousands):

	Year Ended December 31,		
	2025	2024	2023
Selling, general and administrative	$ 46,094	$ 32,308	$ 24,131
Research and development	24,158	15,188	10,892
Cost of sales	1,172	948	—
Total	$ 71,424	$ 48,444	$ 35,023

Stock-based compensation capitalized into inventory was $0.7 million, $1.1 million and $0.8 million for the years ended December 31, 2025, 2024, 2023, respectively.

13. Income Taxes

The provision for (benefit from) income taxes was based upon income (loss) before income taxes as follows (in thousands):

	Year Ended December 31,		
	2025	2024	2023
U.S. source	$ (24,877)	$ (89,937)	$ (164,953)
Non-U.S. source	3,450	3,025	2,529
Loss before income taxes	$ (21,427)	$ (86,912)	$ (162,424)

The components of the Company's income tax provision for (benefit from) were as follows (in thousands):

	Year Ended December 31,		
	2025	2024	2023
Provision for income taxes:			
Federal	$ (80)	$ 32	$ 37
State	819	249	67
Foreign	1,197	749	887
	1,936	1,030	991
Benefit from deferred income taxes:			
Federal	—	—	—
State	—	—	—
	—	—	—
Provision for (benefit from) income taxes	$ 1,936	$ 1,030	$ 991

Beginning in 2025 annual reporting, the Company adopted ASU 2023-09 prospectively. See Note 2 for additional details on the adoption of ASU 2023-09. A reconciliation of the U.S. federal statutory income tax rate to the

effective tax rate pursuant to the disclosure requirements of ASU 2023-09 for the year ended December 31, 2025 is as follows (in thousands, except percentages):

	Year Ended December 31, 2025	
Loss before provision of income taxes	$ (21,427)	
Provision for income taxes at U.S. Federal statutory rate	(4,500)	21.00 %
State income taxes, net of Federal effect [1]	641	(2.99)%
Foreign tax effects		
Switzerland		
Foreign rate differential	(285)	1.33 %
Cantonal tax impacts	106	(0.49)%
Other	(42)	0.20 %
Other foreign jurisdictions	695	(3.24)%
Effects of cross-border tax laws		
Subpart F	527	(2.46)%
Change in valuation allowance	11,778	(54.97)%
Nontaxable or nondeductible items		
Stock-based compensation	(5,167)	24.11 %
Executive compensation	4,189	(19.55)%
Intercompany sale	2,943	(13.74)%
Transaction costs	440	(2.05)%
Other	83	(0.39)%
Tax credits	(12,629)	58.94 %
Changes in unrecognized tax benefits	3,157	(14.74)%
Total tax provision	$ 1,936	(9.04)%

(1) State taxes in Florida, Texas and Michigan made up majority (greater than 50%) of the tax effect in this category.

A reconciliation of the federal statutory income tax rate to the Company's effective income tax rate for the years ended December 31, 2024 and 2023 is as follows:

	Year Ended December 31,	
	2024	2023
Federal statutory income tax rate	21.00 %	21.00 %
State tax	3.95	3.57
Permanent differences	0.03	(0.50)
Other	(0.38)	1.12
Section 162(m) limitation	(3.29)	(1.33)
Foreign source income subject to U.S. tax	(0.45)	(1.69)
Orphan Drug and General Business Credit	7.50	1.91
Change in valuation allowance	(29.54)	(24.69)
Total tax provision	(1.18)%	(0.61)%

The amounts of income taxes paid, net of refunds received by the Company are as follows (in thousands):

	Year Ended December 31, 2025
Federal	$ 53
State and Local	
New York	206
Texas	118
Other	762
Foreign	
Netherlands	256
Canada	201
Germany	166
Spain	183
Switzerland	142
Other	70
Income taxes paid, net of amounts refunded	$ 2,157

The significant components of the Company's deferred taxes are as follows (in thousands):

	December 31,	
	2025	2024
Deferred tax assets:		
Net operating loss carryforwards	$ 45,302	$ 38,404
Capitalized research and development expenses	48,776	51,694
Tax credit carryforwards	49,303	37,260
Interest limitation attributes	10,610	14,008
Stock-based compensation	9,875	9,949
Intangible assets	8,976	6,664
Accrued expenses	7,074	5,140
Inventory	1,769	4,097
Lease liabilities	1,762	2,144
Total deferred tax assets	183,447	169,360
Deferred tax liabilities:		
Operating lease right-of-use assets	(1,531)	(1,885)
Fixed assets	(94)	(83)
Total deferred tax liabilities	(1,625)	(1,968)
Valuation allowance	(181,822)	(167,392)
Net deferred tax assets	$ —	$ —

The valuation allowance increased by $14.4 million, $24.2 million and $40.2 million for the years ended December 31, 2025, 2024, 2023, respectively. The tax benefit of deductible temporary differences or carryforwards is recorded as a deferred tax asset to the extent that management assesses the realization is "more likely than not." Future realization of the tax benefit ultimately depends on the existence of sufficient taxable income within the period available under the tax law. At December 31, 2025 and 2024, the Company has set up valuation allowances against all federal and state net deferred tax assets, because based on all available evidence, these deferred tax assets are not more than likely to be realizable.

As of December 31, 2025, the Company had the following net operating loss and tax credit carryforwards, which if not utilized, will expire as follows (in millions, except for expiry):

Type	Amount	Year
Federal net operating loss carryforwards	$185.9	Indefinite
Federal R&D and orphan drug credit carryforwards	$57.3	2039
State net operating loss carryforwards	$105.1	2038
State R&D and orphan drug credit carryforwards	$10.8	Indefinite

In general, if the Company experiences a greater than 50 percentage point aggregate change in ownership of certain significant stockholders over a three-year period (a "Section 382 ownership change"), utilization of its pre-change NOL carryforwards and the research and development credit carryforwards is subject to an annual limitation under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, and similar state laws. The annual limitation generally is determined by multiplying the value of the Company's stock at the time of such ownership change, subject to certain adjustments, by the applicable long-term tax-exempt rate. Such limitations may result in expiration of a portion of the NOL carryforwards and research and development credit carryforwards before utilization and may be material. As of December 31, 2025, the Company determined that it has not experienced an ownership change and determined that NOLs and tax credits are not subject to a limitation pursuant to Section 382.

The Company recognizes the financial statements effects of a tax position when it is more likely than not, based on technical merits, that the position will be sustained upon examination.

A reconciliation of the Company's unrecognized tax benefits is as follows (in thousands):

	Year Ended December 31,	
	2025	2024
Balance at beginning of year	$ 12,965	$ 10,454
(Decreases)/increases related to prior year tax positions	(110)	337
Increases related to current year tax positions	4,269	2,174
Balance at end of year	$ 17,124	$ 12,965

The Company has considered the amounts and probabilities of the outcomes that can be realized upon ultimate settlement with the tax authorities and determined unrecognized tax benefits primarily related to credits should be established as noted in the summary roll-forward above. The Company's effective income tax rate would not be impacted if the unrecognized tax benefits were recognized in 2025 and 2024, as the Company is in a full valuation allowance position.

The Company files federal, state and foreign income tax returns in jurisdictions with varying statutes of limitations. All of the Company's tax returns in all jurisdictions remain open to examination since inception. The Company's policy is to recognize interest expense and penalties related to income tax matters as tax expense. As of December 31, 2025 and 2024, there were no significant accruals for interest related to unrecognized tax benefits or tax penalties.

The Company has not provided U.S. income or foreign withholding taxes on the undistributed earnings of its foreign subsidiaries as of December 31, 2025 and 2024, because it intends to permanently reinvest such earnings outside of the U.S. If these foreign earnings were to be repatriated in the future, the related U.S. tax liability will be immaterial, due to the participation exemption put in place in the Tax Act.

14. Segment Reporting

The Company's chief operating decision maker ("CODM"), the Chief Executive Officer, manages the Company's operations and business as one operating segment and allocates resources to operations of the Company on an entity-wide basis. The CODM assesses performance of the Company and determines resource allocation primarily based on net product sales and loss from operations on a consolidated basis. The CODM uses loss from operations to monitor budget versus actual results and considers any adjustments and actions required for good fiscal management.

The Company's CODM is regularly provided with entity-wide expense categories similar to those found in the consolidated statements of operations, as well as the following (in thousands):

	Year Ended December 31,		
	2025	2024	2023
Revenue:			
Product sales, net	$ 521,312	$ 336,409	$ 178,874
License and other revenue	—	479	7,500
Total revenue	521,312	336,888	186,374
Less:			
Cost of sales (excluding intangible amortization and other non-cash expenses)	76,665	50,672	33,215
General and administrative expenses (excluding stock-based compensation)	54,912	45,617	36,902
Commercialization and Medical Affairs expenses (excluding stock-based compensation)	156,024	124,296	84,847
Research and development expenses (excluding stock-based compensation)	162,020	125,442	91,717
Stock-based compensation	70,252	47,496	35,023
Intangible amortization and other non-cash expenses	23,575	30,971	13,824
Loss from operations	$ (22,136)	$ (87,606)	$ (109,154)

15. Commitments and Contingencies

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

The Company is subject to potential liabilities under government regulations and various claims and legal actions that are pending or may be asserted from time-to-time. These matters arise in the ordinary course and conduct of the Company's business and may include, for example, commercial, intellectual property, and employment matters. The Company intends to defend itself vigorously in such matters and when warranted, take legal action against others. Furthermore, the Company regularly assesses contingencies to determine the degree of probability and range of possible loss for potential accrual in its consolidated financial statements.

An estimated loss contingency is accrued in the Company's consolidated financial statements if it is probable that a liability has been incurred and the amount of the loss can be reasonably estimated. The Company does not accrue amounts for liabilities that it does not believe are probable. Litigation is inherently unpredictable, and unfavorable resolutions could occur. As a result, assessing contingencies is highly subjective and requires judgment about future events. During the periods presented, the Company has not recorded any accruals for loss contingencies.

On December 19, 2025, the Company, along with Satiogen Pharmaceuticals, Inc. and Shire Human Genetic Therapies, Inc. as co-plaintiffs, filed four complaints against Sandoz Inc. ("Sandoz"); Annora Pharma Private Limited, Hetero Labs Limited, and Hetero USA Inc. (together, "Hetero"); Zenara Pharma Private Limited and Biophore India Pharmaceuticals Private Limited (together, "Biophore"); and Zydus Lifesciences Global FZE, Zydus Lifesciences Limited, and Zydus Pharmaceuticals (USA) Inc. (together, "Zydus") (all collectively, "Defendants") in the U.S. District Court for the District of Delaware (the "LIVMARLI Patent Litigations"), alleging infringement of certain Orange Book listed patents covering LIVMARLI (the "LIVMARLI Patents"). The LIVMARLI Patent Litigations were initiated following the submission by Defendants, in accordance with the procedures set out in the Hatch-Waxman Act, of ANDAs directed to generic versions of LIVMARLI. Defendants' ANDAs seek approval to market generic versions of LIVMARLI prior to the expiration of the LIVMARLI Patents and allege that the LIVMARLI Patents are invalid, unenforceable, and/or not infringed. The Company is seeking, among other relief, an order that the effective date of any FDA approval of Defendants' ANDAs be no earlier than the expiration of the asserted patents listed in the Orange Book, and such further and other relief as the court may deem appropriate. The Defendants are subject to a 30-month stay of final regulatory approval through March 29, 2029, preventing them from marketing generic versions of LIVMARLI during that time. On February 20, 2026, Sandoz asserted counterclaims against the Company and its co-plaintiffs seeking declaratory judgments of non-infringement and invalidity with respect to certain LIVMARLI Patents. Trial in the LIVMARLI Patent Litigations

has not yet been scheduled. The Company cannot make any predictions about the final outcome of these matters or the timing thereof.

16. Subsequent Events

On January 23, 2026, the Company completed the acquisition of Bluejay. As consideration for the transaction, the Company paid to the holders of Bluejay's securities an aggregate amount of $224.2 million in cash, net of cash acquired in the transaction, and 4,673,597 shares of Company common stock, subject in certain cases to deduction to satisfy applicable taxes, and will pay up to an aggregate amount of $25.8 million in cash and up to 522,375 shares of Company common stock, subject to certain conditions and deduction to satisfy applicable taxes. Additionally, the Company is obligated to pay up to an aggregate amount of $200.0 million upon the achievement of certain commercial milestones.

Entities affiliated with Frazier Life Sciences, which are associated with a member of the Company's board of directors, were Bluejay security holders.

Immediately following the completion of the acquisition, the Company completed the private placement of 3,385,149 shares of the Company's common stock ("PIPE Shares") and pre-funded warrants (the "Pre-Funded Warrants") to purchase 536,412 shares of Company common stock (the "Warrant Shares") for an aggregate gross proceeds of approximately $268.5 million. The purchase price per PIPE Share was $68.48 and the purchase price per Pre-Funded Warrant was $68.4799 (which equals the purchase price of a PIPE Share, less $0.0001, the exercise price of each Warrant Share).

Item 9. Changes in and Disagreements With Accountants on Accounting and Financial Disclosure.

None.

Item 9A. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation and supervision of our Chief Executive Officer and our Chief Financial Officer (our principal executive officer and principal financial officer, respectively), has evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) as of the end of the period covered by this Annual Report. Based on that evaluation, our Chief Executive Officer and our Chief Financial Officer have concluded that our disclosure controls and procedures were effective as of December 31, 2025.

Management's Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting, as defined in Exchange Act Rules 13a-15(f) and 15d-15(f). We maintain internal control over financial reporting designed by, or under the supervision of, our Chief Executive Officer and Chief Financial Officer (our principal executive officer and principal financial officer, respectively), 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.

Under the supervision and with the participation of our Chief Executive Officer and our Chief Financial Officer, our management has assessed the effectiveness of our internal control over financial reporting as defined in Rule 13a-15(f) and 15d-15(f) under the Exchange Act as of December 31, 2025. Our management's assessment was based on criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO), Internal Control-Integrated Framework (2013). Based on this assessment, our management concluded that our internal control over financial reporting was effective at the reasonable assurance level as of December 31, 2025.

The effectiveness of our internal control over financial reporting as of December 31, 2025 has been audited by Ernst & Young LLP, an independent registered public accounting firm, as stated in its report which is included in Item 8 of this Annual Report on Form 10-K.

Changes in Internal Control over Financial Reporting

There have been no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the quarter ended December 31, 2025 that materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information

There are no disclosures required by this Item 9B, including those relating to "Rule 10b5-1 trading arrangements" and "non-Rule 10b5-1 trading arrangements," as those terms are defined in Item 408 of Regulation S-K.

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections.

Not applicable.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

The information required by this item will be included,in our definitive proxy statement to be filed with the Securities and Exchange Commission in connection with our 2026 Annual Meeting of Stockholders ("Definitive Proxy Statement"), which is expected to be filed not later than 120 days after the end of our fiscal year ended December 31, 2025 and is incorporated herein by reference.

Code of Business Conduct and Ethics

We maintain a Code of Conduct that applies to all our employees, officers and directors. This includes our principal executive officer, principal financial officer and principal accounting officer or controller, or persons performing similar functions. The full text of our Code of Conduct is posted on our website at www.mirumpharma.com. If we make any amendments to the Code of Conduct or grant any waiver from a provision of the Code of Conduct to any principal executive officer, principal financial officer, principal accounting officer or controller, or any person performing similar functions that are required to be disclosed pursuant to SEC rules, we will promptly disclose the nature of the amendment or waiver on our website or in a current report on Form 8-K. Information contained in, or that can be accessed through, our website is not incorporated by reference herein, and you should not consider information on our website to be part of this Annual Report.

Item 11. Executive Compensation.

The information required by this item will be included in the Definitive Proxy Statement and is incorporated herein by reference.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

The information required by this item will be included in the Definitive Proxy Statement and is incorporated herein by reference.

Item 13. Certain Relationships and Related Transactions, and Director Independence.

The information required by this item will be included in the Definitive Proxy Statement and is incorporated herein by reference.

Item 14. Principal Accountant Fees and Services.

The information required by this item will be included in the Definitive Proxy Statement and is incorporated herein by reference.

PART IV

Item 15. Exhibits and Financial Statement Schedules.

(a) The following documents are filed as part of this Annual Report:

(1) Financial statements

The financial statements filed as part of this Annual Report are included in Part II, Item 8 of this Annual Report.

(2) Financial statement schedules

Financial statement schedules have been omitted in this Annual Report because they are not applicable, not required under the instructions, or the information requested is set forth in the financial statements or related notes thereto.

(3) Exhibits

The exhibits listed in the accompanying Exhibit Index are filed as part of, or incorporated by reference into, this Annual Report.

Item 16. Form 10-K Summary.

None.

Exhibit Index

Exhibit Number	Description
2.1#¥	Asset Purchase Agreement, dated July 16, 2023, by and between Mirum Pharmaceuticals, Inc. and Travere Therapeutics, Inc. (incorporated by reference to Exhibit 2.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on July 17, 2023, and incorporated by reference herein).
2.2#¥	Agreement and Plan of Merger and Reorganization, dated December 6, 2025, by and among Mirum Pharmaceuticals, Inc., Bjork Merger Sub I, Inc., Bjork Merger Sub II, LLC, Bluejay Therapeutics, Inc. and Fortis Advisors LLC (incorporated by reference to Exhibit 2.1 to the Registrant's Current Report on Form 8-K, filed with the Commission on December 8, 2025).
3.1	Amended and Restated Certificate of Incorporation, as currently in effect (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on July 25, 2019, and incorporated by reference herein).
3.2	Amended and Restated Bylaws, as currently in effect (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed with the SEC on July 25, 2019, and incorporated by reference herein).
4.1	Form of Common Stock Certificate (incorporated by reference to Exhibit 4.1 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-232251), filed with the SEC on July 8, 2019, and incorporated by reference herein).
4.2	Description of Common Stock of the Registrant (incorporated by reference to Exhibit 4.3 to the Registrant's Annual Report on Form 10-K, filed with the SEC on March 15, 2024, and incorporated by reference herein).
4.3	Indenture, dated as of April 17, 2023, between Mirum Pharmaceuticals, Inc. and U.S. Bank Trust Company, National Association, as trustee (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on April 17, 2023, and incorporated by reference herein).
4.4	Form of certificate representing the 4.00% Convertible Senior Notes due 2029 (included as Exhibit A to Exhibit 4.3 above) (incorporated by reference to Exhibit 4.2 to the Registrant's Current Report on Form 8-K, filed with the SEC on April 17, 2023, and incorporated by reference herein).
4.5	Form of Pre-Funded Warrant (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on December 8, 2025, and incorporated by reference herein).
4.6	Registration Rights Agreement by and among the Registrant and the parties thereto, dated December 7, 2025 (incorporated by reference to Exhibit 10.2 to the Registrant's Current Report on Form 8-K, filed with the SEC on December 8, 2025, and incorporated by reference herein).
4.7	Registration Rights Agreement by and among the Registrant and the parties thereto, dated December 18, 2025 (incorporated by reference to Exhibit 10.2 to the Registrant's Current Report on Form 8-K, filed with the SEC on December 19, 2025, and incorporated by reference herein).
10.1+	Mirum Pharmaceuticals, Inc. 2018 Equity Incentive Plan (incorporated by reference to Exhibit 10.1 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-232251), filed with the SEC on June 21, 2019, and incorporated by reference herein).
10.1A+	Forms of grant notice, stock option agreement and notice of exercise under the Mirum Pharmaceuticals, Inc. 2018 Equity Incentive Plan (incorporated by reference to Exhibit 10.2 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-232251), filed with the SEC on June 21, 2019, and incorporated by reference herein).
10.2+	Mirum Pharmaceuticals, Inc. 2019 Equity Incentive Plan (incorporated by reference to Exhibit 10.3 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-232251), filed with the SEC on July 8, 2019, and incorporated by reference herein).
10.2A+	Forms of grant notice, stock option agreement and notice of exercise under the Mirum Pharmaceuticals, Inc. 2019 Equity Incentive Plan (incorporated by reference to Exhibit 10.4 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-232251), filed with the SEC on July 8, 2019, and incorporated by reference herein).
10.2B+	Forms of restricted stock unit grant notice and award agreement under the Mirum Pharmaceuticals, Inc. 2019 Equity Incentive Plan (incorporated by reference to Exhibit 10.5 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-232251), filed with the SEC on July 8, 2019, and incorporated by reference herein).

10.2C+	Forms of international grant notice, stock option agreement and notice of exercise under the Mirum Pharmaceuticals, Inc. 2019 Equity Incentive Plan (incorporated by reference to Exhibit 10.6 to the Registrant's Annual Report on Form 10-K, as filed with the SEC on March 9, 2022, and incorporated by reference herein).
10.2D+	Forms of international director grant notice, stock option agreement and notice of exercise under the Mirum Pharmaceuticals, Inc. 2019 Equity Incentive Plan (incorporated by reference to Exhibit 10.7 to the Registrant's Annual Report on Form 10-K, as filed with the SEC on March 9, 2022, and incorporated by reference herein).
10.2E+	Forms of international restricted stock unit grant notice and award agreement under the Mirum Pharmaceuticals, Inc. 2019 Equity Incentive Plan (incorporated by reference to Exhibit 10.8 to the Registrant's Annual Report on Form 10-K, as filed with the SEC on March 9, 2022, and incorporated by reference herein).
10.2F+	Forms of restricted stock unit grant notice and award agreement for non-employee directors electing to defer settlement of restricted stock units under the Mirum Pharmaceuticals, Inc. 2019 Equity Incentive Plan (incorporated by reference to Exhibit 10.2 to the Registrant's Quarterly Report on Form 10-Q, as filed with the SEC on May 8, 2024, and incorporated by reference herein).
10.3+	Mirum Pharmaceuticals, Inc. 2019 Employee Stock Purchase Plan (incorporated by reference to Exhibit 10.6 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-232251), filed with the SEC on July 8, 2019, and incorporated by reference herein).
10.3A+	Mirum Pharmaceuticals, Inc. 2019 Employee Stock Purchase Plan Terms and conditions – Non-U.S. Participants (incorporated by reference to Exhibit 10.10 to the Registrant's Annual Report on Form 10-K, as filed with the SEC on March 9, 2022, and incorporated by reference herein).
10.4+	Mirum Pharmaceuticals, Inc. 2020 Inducement Plan, as amended September 4, 2025 (incorporated by reference to Exhibit 99.1 to the Registrant's Registration Statement on Form S-8 (File No. 333-290137), as filed with the SEC on September 9, 2025, and incorporated by reference herein).
10.4A+	Forms of grant notice, stock option agreement and notice of exercise under the Mirum Pharmaceuticals, Inc. 2020 Inducement Plan (incorporated by reference to Exhibit 10.2 to the Registrant's From 10-Q, filed with the SEC on May 7, 2020, and incorporated by reference herein).
10.4B+	Forms of restricted stock unit grant notice and award agreement under the Mirum Pharmaceuticals, Inc. 2020 Inducement Plan (incorporated by reference to Exhibit 10.3 to the Registrant's From 10-Q, filed with the SEC on May 7, 2020, and incorporated by reference herein).
10.4C+	Forms of international grant notice, stock option agreement and notice of exercise under the Mirum Pharmaceuticals, Inc. 2020 Inducement Plan (incorporated by reference to Exhibit 10.14 to the Registrant's Annual Report on Form 10-K, as filed with the SEC on March 9, 2022, and incorporated by reference herein).
10.4D+	Forms of international restricted stock unit grant notice and award agreement under the Mirum Pharmaceuticals, Inc. 2020 Inducement Plan (incorporated by reference to Exhibit 10.15 to the Registrant's Annual Report on Form 10-K, as filed with the SEC on March 9, 2022, and incorporated by reference herein).
10.5+	Form of Indemnification Agreement by and between the Registrant and each director and executive officer (filed as Exhibit 10.7 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-232251), filed with the SEC on July 8, 2019, and incorporated by reference herein).
10.6+	Mirum Pharmaceuticals, Inc. Amended and Restated Severance Benefit Plan and form of Participation Agreement thereunder (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on August 30, 2023, and incorporated by reference herein).
10.7+	Amended and Restated Offer Letter by and between the Registrant and Christopher Peetz, dated May 15, 2019 (filed as Exhibit 10.10 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-232251), filed with the SEC on June 21, 2019, and incorporated by reference herein).
10.8+	Offer Letter by and between the Company and Eric Bjerkholt, dated August 8, 2023 (filed as Exhibit 10.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on September 11, 2023, and incorporated by reference herein).
10.9+	Offer Letter by and between the Registrant and Peter Radovich, dated April 28, 2020 (incorporated by reference to Exhibit 10.18 to the Registrant's Annual Report on Form 10-K, filed with the SEC on March 9, 2021, and incorporated by reference herein).

10.10+	Offer Letter by and between the Registrant and Joanne Quan, dated January 11, 2024 (incorporated by reference to Exhibit 10.5 to the Registrant's Quarterly Report on Form 10-Q, filed with the SEC on May 8, 2024, and incorporated by reference herein)
10.11#¥	License Agreement by and between Lumena Pharmaceuticals, Inc. and Satiogen Pharmaceuticals, Inc., dated February 8, 2011 (filed as Exhibit 10.15 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-232251), filed with the SEC on June 21, 2019, and incorporated by reference herein).
10.11A#	Amendment to License Agreement by and between Lumena Pharmaceuticals, Inc. and Satiogen Pharmaceuticals, Inc., dated February 8, 2011 (filed as Exhibit 10.16 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-232251), filed with the SEC on June 21, 2019, and incorporated by reference herein).
10.12#¥	License Agreement by and between Lumena Pharmaceuticals, Inc. and Pfizer Inc., dated June 1, 2012 (filed as Exhibit 10.17 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-232251), filed with the SEC on June 21, 2019, and incorporated by reference herein).
10.13#¥	License Agreement by and between Lumena Pharmaceuticals, Inc. and Sanofi-Aventis Deutschland GmbH, dated September 27, 2012 (filed as Exhibit 10.18 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-232251), filed with the SEC on June 21, 2019, and incorporated by reference herein).
10.13A#	Amendment No. 1 to License Agreement by and between Shire Orphan and Rare Disease GmbH (successor in interest of Lumena Pharmaceuticals, Inc.) and Sanofi-Aventis Deutschland GmbH, dated June 26, 2015 (filed as Exhibit 10.19 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-232251), filed with the SEC on June 21, 2019, and incorporated by reference herein).
10.14#¥	Assignment and License Agreement by and between the Registrant and Shire International GmbH, dated November 5, 2018 (filed as Exhibit 10.20 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-232251), filed with the SEC on June 21, 2019, and incorporated by reference herein).
10.15¥	Office Lease, dated January 17, 2024, by and between the Registrant and Hudson Metro Center, LLC (incorporated by reference to Exhibit 10.17 to the Registrant's Annual Report on Form 10-K, filed with the SEC on March 15, 2024, and incorporated by reference herein).
10.15A¥*	First Amendment to Office Lease, dated December 10, 2025, by and between the Registrant and Hudson Metro Center, LLC.
10.15B¥*	Second Amendment to Office Lease, dated January 13, 2026, by and between the Registrant and Hudson Metro Center, LLC.
10.16	Sales Agreement by and between the Registrant, Leerink Partners LLC and Cantor Fitzgerald & Co., dated November 2, 2023 (incorporated by reference to Exhibit 10.3 to the Registrant's Quarterly Report on Form 10-Q, filed with the SEC on November 2, 2023).
10.17+	Mirum Pharmaceuticals, Inc. Non-Employee Director Compensation Policy, as amended April 4, 2024 (incorporated by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q, filed with the SEC on August 7, 2024, and incorporated by reference herein).
10.18	Subscription Agreement by and among the Registrant and the parties thereto, dated December 7, 2025 (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on December 8, 2025, and incorporated by reference herein).
10.19	Subscription Agreement by and among the Registrant and the parties thereto, dated December 18, 2025 (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on December 19, 2025, and incorporated by reference herein).
19.1	Mirum Pharmaceuticals, Inc. Insider Trading and Window Period Policy, as amended.
21.1	Subsidiaries of the Registrant (incorporated by reference to Exhibit 21.1 to the Registrant's Annual Report on Form 10-K, as filed with the SEC on March 8, 2023, and incorporated by reference herein).
23.1*	Consent of Independent Registered Public Accounting Firm.
24.1	Power of Attorney. Reference is made to the signature page hereto.
31.1*	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.

31.2*	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1*†	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2*†	Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
97.1	Mirum Pharmaceuticals Inc. Incentive Compensation Recoupment Policy (incorporated by reference to Exhibit 97.1 to the Registrant's Annual Report on Form 10-K, filed with the SEC on March 15, 2024, and incorporated by reference herein).
101.INS*	Inline XBRL Instance Document - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL 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*	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104*	Cover Page Interactive Data File (embedded within the Inline XBRL document)

---

* Filed herewith.

\+ Indicates management contract or compensatory plan.

\# Pursuant to Item 601(b)(2) and Item 601(b)(10) of Regulation S-K, as applicable, certain portions of this exhibit have been omitted (indicated by "[*]" or "[…***…]") because the Registrant has determined that the information is not material and is the type that the Registrant treats as private or confidential.

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

† The information in Exhibits 32.1 and 32.2 shall not be deemed "filed" for purposes of Section 18 of the Exchange Act, or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act or the Exchange Act (including this Annual Report), unless the Registrant specifically incorporates the foregoing information into those documents by reference.

SIGNATURES

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

MIRUM PHARMACEUTICALS, INC.

Date: February 25, 2026

By: /s/ Christopher Peetz

Christopher Peetz

Chief Executive Officer and Director
(Principal Executive Officer)

POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Christopher Peetz, Eric Bjerkholt and Douglas Sheehy and each of them, as his or her true and lawful attorneys-in-fact and agents, each with the full power of substitution, for him or her and in his or her name, place or stead, in any and all capacities, to sign any and all amendments to this Annual Report on Form 10-K, and to file the same, with all exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in and about the premises, as fully to all intents and purposes as he or she might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents, or their or his substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, this Report has been signed below by the following persons on behalf of the Registrant in the capacities and on the dates indicated.

Name	Title	Date
/s/ Christopher Peetz Christopher Peetz	Chief Executive Officer and Director (Principal Executive Officer)	February 25, 2026
/s/ Eric Bjerkholt Eric Bjerkholt	Chief Financial Officer (Principal Financial Officer)	February 25, 2026
/s/ Jody Howe Jody Howe	Senior Vice President, Global Controller (Principal Accounting Officer)	February 25, 2026
/s/ Laura Brege Laura Brege	Director	February 25, 2026
/s/ Lon Cardon, Ph.D. Lon Cardon, Ph.D.	Director	February 25, 2026
/s/ William C. Fairey William C. Fairey	Director	February 25, 2026
/s/ Laurent Fischer, M.D. Laurent Fischer, M.D.	Director	February 25, 2026
/s/ Michael Grey Michael Grey	Director	February 25, 2026
/s/ Patrick Heron Patrick Heron	Director	February 25, 2026
/s/ Saira Ramasastry, M.S., M.Phil Saira Ramasastry, M.S., M.Phil	Director	February 25, 2026
/s/ Timothy Walbert Timothy Walbert	Director	February 25, 2026

[This page intentionally left blank]

[This page intentionally left blank]