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, 2021

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.)**
950 Tower Lane, Suite 1050, 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. ☐

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, 2021, the last business day of the Registrant's most recently completed second fiscal quarter, was approximately $0.3 million, based on the closing price of the Registrant's common stock on the Nasdaq Global Market of $17.63 per share.

The number of shares of Registrant's common stock, par value $0.0001 per share, outstanding as of March 4, 2022 was 31,730,420.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the Registrant's definitive proxy statement for its 2022 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, 2021, are incorporated by reference into Part III of this Annual Report on Form 10-K.

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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:

- our ability to obtain and maintain regulatory approval for 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;
- the commercialization of Livmarli and our other product candidates, if approved;
- 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 of Livmarli and volixibat;
- our expectations regarding the size of target patient populations for Livmarli and our other 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 product candidates, and our ability to serve those markets;
- the rate and degree of market acceptance of our product candidates, as well as third-party payor coverage and reimbursement for 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 product candidates;
- regulatory and legal developments in the United States and foreign countries;
- the performance of our third-party 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 estimates regarding the impact of the ongoing coronavirus (together with its variants, COVID-19) pandemic, including political or social movements arising in relation to the COVID-19 pandemic on our business and operations, the business and operations of our collaborators and on the global economy;

•our ability to obtain funding for our operations; 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.

- LIVMARLI® oral solution ("Livmarli") is our only U.S. Food and Drug Administration ("FDA") -approved product and the success of our business depends, in part, on its continued successful commercialization.
- As a company we currently have limited marketing and sales experience. If we are unable to adequately maintain and scale our marketing and sales capabilities or enter into or lose rights pursuant to agreements with third parties to market and sell our products, we may not be able to generate viable

product revenues. Even if we adequately establish such capabilities, market acceptance or reimbursement of our products may be lower than expected.

•Livmarli may fail to achieve the broad degree of physician and patient adoption and use necessary for commercial success.

•We rely completely on third parties to supply, manufacture and distribute drug supplies for Livmarli, including certain sole-source suppliers and manufacturers.

•We have a very limited operating history, and we have incurred significant losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future.

•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 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.

•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 applications for marketing authorization with regulatory authorities may not be accepted or may require additional studies, regulatory actions, or manufacturing requirements to be completed before marketing authorization is granted.

•Even if we obtain regulatory approval for our product candidates, our product candidates may not gain market acceptance among physicians, patients, tertiary care centers, transplant centers and others in the medical community.

•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 will need substantial additional financing to continue our commercialization efforts for Livmarli, develop our product candidates and implement our operating plans. If we fail to obtain additional financing, we may be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

•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.

•If we are unable to obtain and maintain sufficient intellectual property protection for Livmarli 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 Livmarli and our other product candidates, if approved, may be adversely affected.

PART I

Item 1. Business.

Overview

We are a biopharmaceutical company focused on the identification, acquisition, development and commercialization of novel therapies for rare and orphan diseases. We focus on diseases for which the unmet medical need is high and the biology for treatment is clear. Our current focus is the development and commercialization of LIVMARLI® (maralixibat) oral solution ("Livmarli") and volixibat.

Livmarli is approved for the treatment of cholestatic pruritus in patients with Alagille syndrome ("ALGS") one year of age and older in the United States. We market and commercialize Livmarli in the United States through our specialized and focused commercial team. We have also submitted a marketing authorization application ("MAA") to the European Medicines Agency ("EMA") for Livmarli for the treatment of cholestatic liver disease in patients with ALGS one year of age and older and plan to commercialize Livmarli in Western Europe by the end of 2022 with our international team based in Switzerland, if approved. We have entered into license and distribution agreements with several rare disease companies for the commercialization of Livmarli in several additional countries, including South Korea, Greater China and Israel.

Our pipeline consists of rare pediatric indications for Livmarli and orphan adult indications for our second product candidate, volixibat.

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 and advance regulatory approvals of Livmarli for the treatment of ALGS internationally.*** In September 2021 Livmarli was approved by the U.S. Food and Drug Administration ("FDA") for the treatment of cholestatic pruritus in patients with ALGS one year of age and older. Subsequently we commenced marketing and sales of Livmarli in the United States. We have also submitted an MAA to the EMA for Livmarli for the treatment of cholestatic liver disease in patients with ALGS and, if approved, plan to commercialize Livmarli in Western Europe by the end of 2022. Additionally, we have entered into several distributor and licensing agreements to advance Livmarli in numerous territories outside of the United States and Europe.

- ***Advance Livmarli through clinical development and seek regulatory approvals for the treatment of progressive familial intrahepatic cholestasis ("PFIC") and biliary atresia ("BA").*** Livmarli has been granted breakthrough designation for PFIC type 2 and we are conducting the MARCH-PFIC Phase 3 clinical trial to evaluate Livmarli in PFIC at a higher dose than in the previous Phase 2 clinical trials. We are also evaluating the potential of Livmarli in the EMBARK Phase 2b clinical trial in BA. We plan to commercialize Livmarli for these indications with our existing commercial teams, if approved.

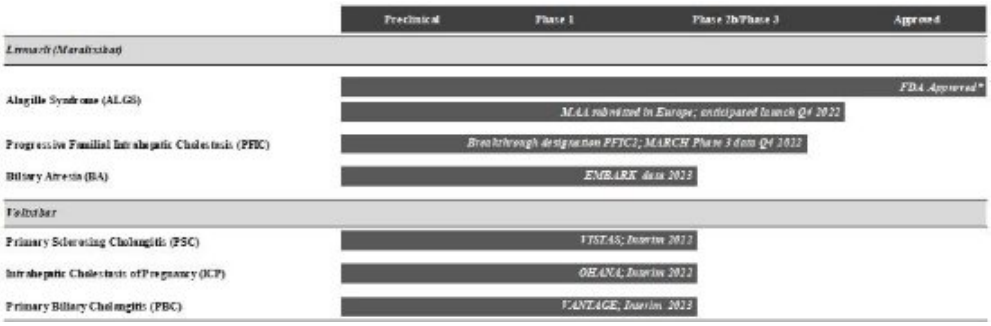
- ***Develop and commercialize volixibat for the treatment of adults with primary sclerosing cholangitis ("PSC"), intrahepatic cholestasis of pregnancy ("ICP") and primary biliary cholangitis ("PBC").*** We plan to further leverage our understanding of cholestatic liver disease with volixibat in adult settings. We are conducting adaptive, potentially registrational, Phase 2b clinical trials of volixibat in PSC, ICP and PBC.

- ***Actively manage our product portfolio and expand our pipeline of novel product candidates.*** 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.

Our Product Pipeline

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



*Approved by the FDA for pruritus in patients one year of age and older.

Commercial Product

Livmarli for cholestatic pruritus in patients with ALGS one year of age and older

Livmarli is an oral solution approved as a once daily medicine for cholestatic pruritus in patients with ALGS one year of age and older. Livmarli is an ileal bile acid transporter ("IBAT") inhibitor that prevents absorption of bile acids in the ileum, thereby lowering serum bile acid 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. In patients with ALGS, multiple organ systems may be affected by the mutation, including the liver, heart, kidneys and central nervous system. The accumulation of bile acids prevents the liver from working properly to eliminate waste from the bloodstream and leads to 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. Furthermore, it is reported that the majority of liver transplants in ALGS are due to cholestatic symptoms rather than progressive liver damage. 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. A study to assess the health-related quality of life in ALGS patients indicated a significant burden in physical, psychological and social health accompanies the disease.

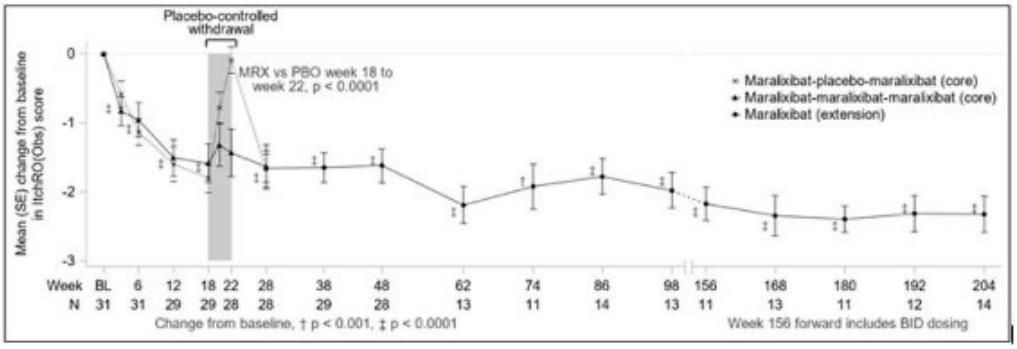
We believe the initial addressable patient population in the United States is approximately 2,000-2,500 pediatric ALGS patients. We believe this addressable number may grow with the availability of an oral therapy as an alternative to transplant for the treatment of pruritus due to ALGS as well as increased disease awareness and diagnosis.

As depicted in the figure below, patients treated with Livmarli in our registrational study experienced an approximately 60% mean reduction from baseline to week 18 in pruritus, as measured by ItchRO(Obs) score ($p<0.0001$). The predefined analysis to assess changes in pruritus during the randomized drug withdrawal period showed that patients who were randomized to placebo experienced a recurrence of their pruritus, whereas those who

continued on Livmarli maintained their reduction in pruritus (p<0.0001). After the randomized drug withdrawal phase during which placebo patients received Livmarli for a second time, a decrease in ItchRO(Obs) score similar to those who had received continuous Livmarli treatment was observed. The results during the randomized drug withdrawal period demonstrate a treatment effect with Livmarli. Pruritus improvements were maintained through week 48 and were statistically significant as compared to baseline (p<0.0001) and were sustained through week 204.

The ItchRO(Obs) score is a caregiver-reported outcome assessment developed for use in cholestatic liver diseases, which measures severity of itch on a 0-4 scale. The ItchRO(Obs) score is a tool developed for evaluating pruritus in pediatric cholestatic settings. Over the course of the Livmarli program, ItchRO(Obs) has been developed and tested to support an outcome assessment for regulatory purposes. We incorporated FDA input into the use of the tool in our clinical trials. Our validation work with ItchRO(Obs) supports that a change of 1.0 is clinically meaningful.

Observer-reported pruritus severity ItchRO(Obs) weekly average morning score change from baseline



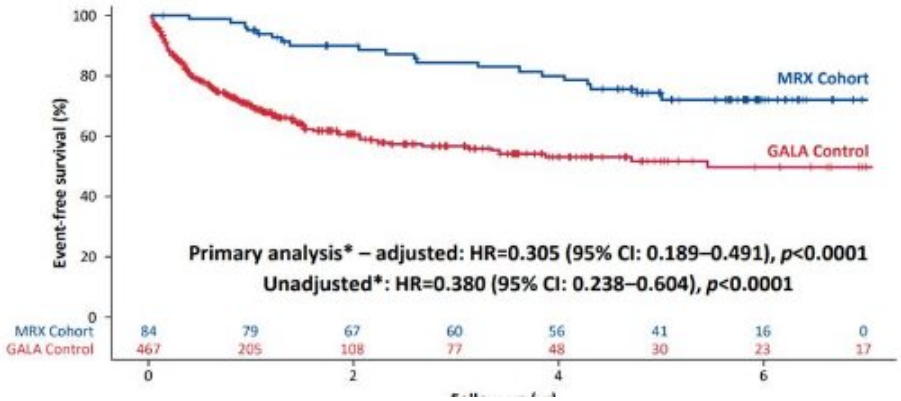
Natural History Comparisons

In a six-year event-free survival analysis in ALGS of a natural history cohort (the GALA clinical research database) and Livmarli treated patients, it was demonstrated that those treated with Livmarli had a 70% reduction for clinical outcomes versus the natural history cohort with similar baseline characteristics.

The analysis evaluated six years of follow-up from pooled Livmarli studies (n=84) in ALGS and compared it against an external natural history control cohort from the GALA database that was selected to have similar disease severity. The objective of the analysis was to compare the time to first clinical event in Livmarli-treated patients with ALGS versus a natural history cohort, with events defined as liver transplantation, biliary diversion surgery, decompensation events (ascites requiring therapy or variceal bleeding), or death. Additional analyses included transplant-free survival as well as several sensitivity and subgroup analyses to ensure robustness of the findings.

The analysis demonstrated a highly significant improvement in six-year event-free survival with a p-value of <0.0001 (Hazard Ratio (“HR”): 0.305, 95% Confidence Interval (“CI”): 0.189-0.491) translating to a 70% overall reduction of clinical outcomes in the Livmarli group. The analysis also showed statistically significant improvements in transplant-free survival with a p-value of <0.0001 (HR: 0.332, 95% CI: 0.197-0.559). These data underscore Livmarli’s potential to have long-term and sustained impact for patients with ALGS.

Livmarli data shows significant improvement in Event Free Survival



HR: Hazard Ratio; CI: Confidence Interval

The results of the natural history comparison analysis were included in our MAA to the EMA for Livmarli in the treatment of cholestatic liver disease in patients with ALGS. The MAA is currently under review and we are preparing for a launch of Livmarli in Western Europe in the fourth quarter of 2022, if approved.

Safety and Tolerability Data for Livmarli in ALGS

In the ALGS clinical development program, which includes five clinical studies comprising 86 patients, patients received doses of Livmarli up to 760 mcg/kg per day with a median duration of exposure of 32.3 months (range: 0.03 - 60.9 months). The majority of Livmarli exposure in the development program occurred without a placebo control in open-label trial extensions. The most common adverse reactions (≥5%) for ALGS patients treated with Livmarli are presented in the table below, regardless of causality. Treatment interruptions or dose reductions occurred in five (6%) patients due to diarrhea, abdominal pain, or vomiting.

Events observed over 5% of patients	Number of events per 100 person-years
Diarrhea	41.6
Abdominal pain	38.6
Vomiting	19.8
Nausea	2.9
Fat-soluble vitamin deficiency	11.1
Transaminase increased	6.9
GI bleeding	3.8
Bone fractures	3.3

In a pooled analysis of patients with ALGS (n=86) administered Livmarli, increases in hepatic transaminases (“ALT”) were observed. Seven (8.1%) patients discontinued Livmarli due to ALT increases. Three (3.5%) patients had a decrease in dose or interruption of Livmarli in response to ALT increases. In the majority of cases, the elevations resolved or improved after discontinuation or dose modification of Livmarli. In some cases, the elevations resolved or improved without change in Livmarli dosing. Increases to more than three times baseline in ALT occurred in 24% of patients treated with Livmarli and increases to more than five times baseline occurred in 2%.

AST increases to more than three times baseline occurred in 14% of patients treated with Livmarli, and an increase to more than five times baseline occurred in one patient. Elevations in transaminases were asymptomatic and not associated with bilirubin elevations or other laboratory abnormalities.

Our Clinical Product Candidates

Livmarli in PFIC

PFIC affects approximately one in 50,000 to 100,000 births in the United States and Europe and typically presents within the first three to six months of life. Surgical options such as PEBD and liver transplantation can be effective, but are expensive, may require life-long administration of immuno-suppressants and have potential for significant comorbidities. Only a portion of the patients who require a liver transplant are able to match with a suitable donor organ, and there can be serious complications, for example, rapid re-occurrence of disease and steatosis. Odevixibat was recently approved for the treatment of pruritus in patients with PFIC, though we believe there is still unmet medical need for new therapies for PFIC.

Livmarli has been granted breakthrough designation for PFIC2 based on the response observed in the Phase 2 INDIGO clinical trial. We are conducting the MARCH-PFIC Phase 3 clinical trial of Livmarli in PFIC. The MARCH-PFIC clinical trial and the open-label long-term extension MARCH-ON are evaluating Livmarli at higher doses in multiple PFIC subtypes. We expect to report topline data in the fourth quarter of 2022.

Livmarli in BA

BA is a rare liver disorder in which there is a blockage or absence of large bile ducts that leads to bile accumulation in the liver and results in progressive cholestasis and liver damage. BA occurs in infants and is estimated to affect one in every 10,000 to 15,000 live births in the United States. BA is the most common reason for liver transplantation in children. There remains a substantial unmet medical need for therapeutic interventions as initial surgical treatment is often unsuccessful. The standard of care for BA is the Kasai procedure, a surgery in which a segment of the small intestine is used to attach the small intestine directly to the liver where bile is expected to drain and is most successful if conducted in the first eight weeks of life. The success rate for the Kasai procedure is approximately 30% to 40%, while the remaining patients are at risk of progressive liver disease requiring liver transplantation. Prognosis after Kasai procedure has been shown to be most promising in patients with lower levels of bilirubin. Further, a strong association between sBA control post-Kasai procedure with long-term outcomes has been observed, supporting the hypothesis that pharmacologically reducing sBA may improve outcomes in BA. We believe IBAT inhibition has therapeutic potential in this patient population through its impact on sBA. We are conducting the EMBARK Phase 2b clinical trial of Livmarli in BA. Livmarli has been granted orphan drug designation for BA by the FDA and the EMA.

Volixibat

We are advancing our second 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 ICP, 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.

ICP is diagnosed in pregnant women typically during the second or third trimester and increases the risk of stillbirth, preterm labor and neonatal complications. Additionally, ICP is associated with severe pruritus. In ICP, sBA levels have been shown to correlate with worsened risk of pre-term labor and stillbirth. We estimate that each year there are approximately 40,000 cases of ICP in the United States and approximately 100,000 cases of ICP in Europe. We have initiated recruitment for the OHANA clinical trial of volixibat in ICP. OHANA is an adaptive, randomized Phase 2b clinical trial evaluating the effect of volixibat on sBA, pruritus and perinatal outcomes in patients with ICP.

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 29,000 people in the United States and approximately 50,000 people in Europe suffer from PSC. Up to 70% 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 immuno-suppressants 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.

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. We are conducting the VANTAGE Phase 2b clinical trial of volixibat in patients with pruritus and PBC.

There are no approved therapies for PSC or ICP 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, peroxisome proliferator activator receptors like bezafibrate and fenofibrate, and others. However, the few therapeutic options available to manage PBC associated pruritus are temporary and/or suboptimal.

License, Finance and Royalty Agreements

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 Pfizer Agreement, Satiogen Agreement and Sanofi Agreement, each of which is defined below.

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, ALGS and BA 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.

As consideration for the rights granted to us under the Shire License Agreement, we made a one-time upfront payment to Shire of $7.5 million and issued Shire 1,859,151 shares of our common stock pursuant to a common stock issuance agreement that we entered into concurrently with the Shire License Agreement.

We are also required to pay Shire up to an aggregate of $107.0 million upon the achievement of certain other clinical development and regulatory milestones for Livmarli in the PFIC, ALGS and BA 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 paid aggregate development and regulatory milestones of $51.0 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. 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"). 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. Similarly, if we actually make royalty payments to Satiogen, which is defined below, under the Satiogen Agreement, the Shire royalties will be reduced by a low single digit percentage of net sales.

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 apical sodium bile acid transporter ("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. After November 5, 2021, 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, or 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 will be 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 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 after the second anniversary of the Sanofi Agreement. 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 Satiogen Pharmaceuticals, Inc.

Through the Shire License Agreement, we were assigned the rights to the license agreement, as amended ("Satiogen Agreement"), with Satiogen Pharmaceuticals, Inc ("Satiogen"), 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"). Under the Satiogen Agreement, we are permitted to develop, manufacture and commercialize products utilizing the ASBTi Technology or TGR5 Technology for the diagnosis, treatment, prevention, mitigation and cure of human diseases and disorders, other than diabetes, obesity or a combination thereof, and to sublicense such rights.

We have sole responsibility and control over development and commercialization activities for products utilizing the ASBTi Technology or TGR5 Technology under the Satiogen Agreement and we are required to use commercially reasonable efforts to develop and commercialize such licensed products.

Ownership of inventions and discoveries conceived or reduced to practice under the Satiogen Agreement will be determined in accordance with the rules of inventorship under United States patent laws. We will own any and all inventions made by us or jointly with Satiogen under the Satiogen Agreement and we will be responsible for filing, prosecuting and maintaining any patents for such inventions. Satiogen will own any and all inventions that are solely invented by Satiogen under the Satiogen Agreement and will be responsible for preparing, filing, prosecuting and maintaining any patents for such inventions. Satiogen will be responsible for filing, prosecuting and maintaining patents related to the ASBTi Technology and TGR5 Technology controlled by Satiogen as of the effective date or during the term of the Satiogen Agreement. Additionally, prior to certain events specified in the Satiogen Agreement, Satiogen will have the sole right, but not the obligation, to enforce patents related to the ASBTi Technology and the TGR5 Technology; after which, we will have the sole right, but not the obligation, to enforce patents related to the ASBTi Technology and the TGR5 Technology. In March 2017, the Satiogen Agreement was amended to terminate the license of certain patents related to the ASBTi Technology and TGR5 Technology as each relates to diabetes and obesity.

We are required to pay to Satiogen up to an aggregate of $10.5 million upon the achievement of certain milestones, of which $0.5 million relates to the initiation of certain development activities, $5.0 million relates to the completion of regulatory approvals and $5.0 million relates to commercialization activities. We will be required to pay to Satiogen a low single-digit royalty on net sales of products utilizing the ASBTi Technology or TGR5 Technology sold by us and our affiliates. Our royalty obligations continue on a licensed product-by-licensed product and country-by-country basis until the expiration of the last valid claim in a licensed patent or patent application covering the applicable licensed product in such country. The term of the last patent or patent application licensed under the Satiogen Agreement ends on August 30, 2031, absent patent term adjustment or extension.

In the event we sublicense any of our rights under the ASBTi Technology or TGR5 Technology to a third party, we are obligated under the Satiogen Agreement to pay to Satiogen a fee based on a percentage of sublicense revenue received by us, which percentage ranges from the mid-teens to mid-twenties, depending on whether the right granted is in connection with the ASBTi Technology or TGR5 Technology, and the stage of development of such sublicensed technology. In addition, we are obligated under the Satiogen Agreement to pay to Satiogen a percentage of royalties we receive in consideration for the grant of such sublicense based on a percentage of revenue generated by such sublicensee for sales of products utilizing the ASBTi Technology or TGR5 Technology, which percentage is in the low-fifties and is subject to adjustment in certain circumstances. This payment will not exceed an amount that is one-half of our low single-digit royalty obligation to Satiogen.

We may unilaterally terminate the Satiogen Agreement for any reason or no reason upon 90 days' written notice to Satiogen. If we cease all research, development and commercialization efforts with respect to all licensed products related to the ASBTi Technology or the TGR5 Technology for over one year, or we determine to cease all such efforts, Satiogen may elect to terminate the Satiogen Agreement with respect to the license under the ASBTi Technology or the TGR5 Technology, respectively. Either party may terminate the Satiogen Agreement for the other party's material breach of the Satiogen Agreement which remains uncured after 90 days of receiving written notice of such breach. Absent early termination, the Satiogen Agreement will automatically terminate upon the expiration of our royalty obligations.

Revenue Interest Purchase Agreement with Oberland

In December 2020, we entered into a 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. Pursuant to the RIPA, the Purchasers paid us $50.0 million on closing and $65.0 million in April 2021, less certain transaction expenses. We may also be entitled to receive up to approximately $50.0 million at the option of the Purchasers to finance in-licenses or other acquisitions on or prior to December 31, 2022.

As consideration for such payments, the Purchasers will have a right to receive certain revenue interests (the "Revenue Interests") from us based on annual net sales of Livmarli, if approved, which will be tiered payments (the "Revenue Interest Payments") based on whether such annual net sales are (i) less than or equal to $350.0 million ("Tier 1"), (ii) exceeding $350.0 million and less than or equal to $1.1 billion ("Tier 2"), or (iii) exceeding $1.1 billion ("Tier 3"). The Revenue Interest Payments will initially be 9.75% (at Tier 1) and 2.00% (at Tier 2 and Tier 3) of such annual net sales in a specified territory (the "Covered Territory"); provided that (i) if the Purchasers have received Revenue Interest Payments in an amount equal to or greater than 110.0% of the total payments actually made by the Purchasers to us, exclusive of transaction expenses (the "Cumulative Purchaser Payments"), on or prior to December 31, 2026, the Revenue Interests shall be reduced to 2.00% at Tier 1 and 0.00% at Tier 3 for all subsequent calendar years beginning on January 1, 2027 and (ii) if the Purchasers have not received Revenue Interest Payments in an amount equal to or greater than 110.0% of the Cumulative Purchaser Payments on or prior to December 31, 2026, the Revenue Interests shall be increased for all subsequent calendar years beginning on January 1, 2027 to a single defined rate (with no separate tiers) that would have provided the Purchasers with an amount equal to 110.0% of the Cumulative Purchaser Payments on or prior to December 31, 2026 had such rate applied to Tier 1 of initial Revenue Interest Payments. The Purchasers' rights to receive the Revenue Interest Payments shall terminate on the date on which the Purchasers have received Revenue Interest Payments of 195.0% of the Cumulative Purchaser Payments, unless the RIPA is terminated earlier.

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 and volixibat. We have rights to pending patent applications in the United States, Europe, South Korea, Hong Kong, China, Japan, Mexico 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 two issued patents in the United Stated directed to treating or ameliorating PFIC2 and 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 Livmarli, both of which expire in October 2032. We have rights to an issued United States Patent No. 11,229,647 in the United States directed to treating ALGS in a pediatric subject comprising administering to the subject about 400 ug/kg/day to about 800 ug/kg/day maralixibat chloride expiring in February 2040. This patent is listed in the FDA's Orange Book: Approved Drug Products with Therapeutic Equivalence Evaluations ("Orange Book") for Livmarli. We have rights to granted and/or issued 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 IBAT inhibitors ("IBATis") that have limited systemic exposure, which expire in October 2032. We also have rights to pending patent applications in United States, Europe, Australia, Japan, Eurasia, South Korea, 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 and/or issued 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 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 modulating a dosage of an IBATi and to methods for using patient genotype to predict response to IBATi administration in patients with BSEP deficiency. 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 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 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 licensed patent applications in the United States and a pending application in 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 recently issued as United States Patent No. 10,251,880 and 11,260,053, the latter being Orange-Book listable 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, 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, upon approval of a commercial use of the corresponding product by a regulatory agency in the jurisdiction where the patent was granted and/or issued. Similar to the patent

term-extensions in the United States, Supplementary Protection Certificates ("SPCs") serve as an extension to a patent right in the European Union for up to five years. Unlike patent term extensions, SPCs apply to extend the protection afforded by the relevant patent solely in relation to the product covered by the marketing authorization.

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.

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 licensed know-how related to Livmarli in the United States, Europe and other countries from Pfizer. We have licensed know-how related to ASBTi Technology and TGR5 Technology from Satiogen. We have licensed know-how related to volixibat from Sanofi. Our existing license agreements as related to Livmarli and volixibat impose various development, regulatory and/ commercial diligence obligations, payment of milestones and/or royalties and other obligations.

In addition, we currently have orphan drug designation for Livmarli for the treatment of ALGS, PFIC, PSC and PBC in the United States and the European Union, 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 European Union, which can be extended to 12 years in the European Union 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 currently with its seven years of orphan drug exclusivity if we obtain orphan drug designation for its approved uses.

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 and Marketing

As a company, we currently have a limited commercial experience. We are building the commercial infrastructure necessary to effectively support the commercialization of Livmarli, and volixibat, if approved, in North America and Europe and are using strategic partners, distributors, or contract management organizations to assist in the commercialization of Livmarli and volixibat in other markets. We believe that our commercial organization can be targeted to the relatively small number of specialists who treat patients with pediatric cholestasis, such as ALGS, PFIC and BA.

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 believe that we can effectively target the relevant audience for Livmarli and volixibat in North America and Europe primarily through an internal sales force. To develop the appropriate commercial infrastructure, we have invested and expect to continue to invest significant amounts of financial and management resources.

In addition, we are building 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 liver disease community, focusing on providing accurate disease state and balanced product information for appropriate management of patients with rare liver disorders. Medical affairs is comprised of medical information, patient advocacy, patient diagnosis, medical science liaisons, research and educational grants.

In October 2020, we entered into a services agreement with Eversana, a leading provider of commercial support services to the life science industry, to provide integrated nationwide distribution, specialty pharmacy, medical information, patient services and hub support for Livmarli in the United States.

Manufacturing

We do not own or operate manufacturing facilities for the production of Livmarli and volixibat or other 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. Over the course of the development of our IBATis we have used and continue to use multiple third-party contract manufacturers. We do not have any current contractual arrangements for the manufacture of commercial supplies of Livmarli or volixibat. We have entered into and expect to continue to enter into, prior to our receipt of any approval from the FDA, if at all, agreements for commercial production of Livmarli and our product candidates with third party suppliers. 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.

Outside of surgery and Livmarli, there are no other approved therapies for the treatment of ALGS in the United States. There are no FDA-approved therapies for the treatment of BA, PSC or ICP in the United States. Symptomatic treatment with antipruritics, such as cholestyramine, typically provides only modest relief. Bristol Myers Squibb Company has discontinued its brand name cholestyramine, but generic versions of the drug are marketed by Upsher-Smith Laboratories, Inc., Par Pharmaceutical Companies, Inc., Sandoz Inc., the generic pharmaceuticals division of Novartis AG and others. UDCA, as ursodiol, is marketed by a number of generic pharmaceutical companies such as Mylan Inc., Actavis Inc., Lannett Company, Inc. and Par Pharmaceutical Companies, Inc.

A number of drugs, including UDCA, rifampin and naltrexone, are used off-label to treat patients suffering from cholestatic liver diseases. 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.

We are aware of two other companies pursuing clinical development of therapies that reduce sBA levels via the IBAT pathway. GlaxoSmithKline plc and Albireo Pharma, Inc. ("Albireo") have IBATis in clinical development for cholestatic liver diseases. We are aware that Albireo has received approval for odevixibat for the treatment of pruritus in patients with PFIC in the United States and for the treatment of PFIC in Europe. Albireo has been granted orphan designation for PFIC in Europe and if Livmarli is deemed similar, it could prevent the approval of Livmarli in PFIC in Europe. Albireo has also announced the initiation of studies of odevixibat in BA and ALGS and plans to pursue other cholestatic liver diseases. We are aware that GlaxoSmithKline plc has completed a Phase 2 trial of its IBATi in PBC patients and has initiated a Phase 3 trial in PBC. We are also aware that Intercept Pharmaceuticals, Inc. is exploring BA as an indication for obeticholic acid. Further, we may compete with companies that are developing gene therapy for the treatment of PFIC. In adult settings of cholestasis, similar to pediatric settings, cholestyramine, UDCA, rifampin and naltrexone are commonly used agents. We are not aware of FDA approved therapeutics for the treatment of ICP or PSC. We are aware of several agents in clinical development for the treatment of PSC, including Cymabay's seladelpar, DURECT Corporation's DUR928, Gilead Sciences Inc.'s GS-9674, HighTide Biopharmaceutical Inc.'s HTD1801, Immunic Therapeutics' IMU-838, Intercept's Ocaliva, or obeticholic acid, NGM Biopharmaceuticals Inc.'s NGM282 and Pliant Therapeutics' PLN-74809. Intercept Pharmaceuticals, Inc.'s Ocaliva is approved as a second-line treatment for PBC. We are aware of several agents in clinical development for the treatment of PBC including Cymabay's seladelpar, Genfit's elafibranor and NGM Biopharmaceuticals, Inc.'s NGM282, Calliditas' setanaxib and COUR Pharmaceuticals' CNP-104. We are also aware that Cara Therapeutics' Korsuva is in Phase 2 development for the treatment of moderate-to-severe pruritus associated with chronic liver disease.

Government Regulation and Product Approval

As a pharmaceutical company that operates in the United States, 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 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 Europe are addressed in a centralized way, but country-specific regulation remains essential in many respects.

U.S. Drug Development Process

In the United States, the FDA regulates drugs under the Federal Food, Drug and Cosmetic Act ("FDCA"), and implementing regulations. Drugs 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 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;

•satisfactory completion of an FDA advisory committee review, if applicable;

•a determination by the FDA within 60 days of its receipt of an NDA to file the NDA 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; and

•FDA review and approval of the NDA prior to any commercial marketing or sale of the drug in the United States.

Before testing any compounds with potential therapeutic value in humans, the drug 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 drug 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 on a drug candidate at any time before or during clinical trials due to safety concerns or non-compliance.

Clinical trials involve the administration of the drug 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 drug 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 drug 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.

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 drug 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 drug 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 drug candidate and, among other things, must develop methods for testing the identity, strength, quality and purity of the final drug. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the drug candidate 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 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 drug product to the satisfaction of the FDA. The submission of an NDA 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 NDAs 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 drug 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 submitted before it accepts them for filing and may request additional information rather than accepting an NDA for filing. The FDA must make a decision on accepting an NDA for filing within 60 days of receipt. Once the submission is accepted for filing, the FDA begins an in-depth review of the NDA. Under the Prescription Drug User Fee Act ("PDUFA"), guidelines that are currently in effect, the FDA has a goal of ten months from the date of "filing" of a standard NDA for a new molecular entity to review and act on the submission. This review typically takes twelve months from the date the NDA 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 NDA to determine, among other things, whether the proposed product is safe and effective for its intended use 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 drug products or drug 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, 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, 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 NDA 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 NDA, 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 NDA 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. 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. 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 European Union has similar but not identical benefits in that jurisdiction.

Rare Pediatric Disease Priority Review Voucher Program

In 2012, Congress authorized the FDA to award priority review vouchers to sponsors of certain rare pediatric disease product applications. This provision is designed to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. Specifically, under this program, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor of a rare pediatric disease drug product receiving a priority review voucher may transfer (including by sale) the voucher to another sponsor. The voucher may be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted the application. The FDA may also revoke any priority review voucher if the rare pediatric disease drug for which the voucher was awarded is not marketed in the U.S. within one year following the date of approval.

For the purposes of this program, a "rare pediatric disease" is a (a) serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents; and (b) rare disease or conditions within the meaning of the Orphan Drug Act. A sponsor may choose to request Rare Pediatric Disease Designation, but the designation process is entirely voluntary; requesting designation is not a prerequisite to requesting or receiving a priority review voucher. In addition, sponsors who choose not to submit a Rare Pediatric Disease Designation request may nonetheless receive a priority review voucher if they request such a voucher in their original marketing application and meet all of the eligibility criteria.

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 products that meet certain criteria. Specifically, new drugs are eligible for Fast Track designation if they 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 on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA, the FDA agrees to accept sections of the NDA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the NDA.

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 drug’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 drugs 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. Drug manufacturers and other entities involved in the manufacture and distribution of approved drugs 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, 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 between the effective date of an IND and the submission date of an NDA plus the time between the submission date of an NDA 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.

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 or non-infringement to one of the patents listed with the FDA by the innovator NDA holder. 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.

Orphan drug exclusivity, as described above, may offer a seven-year period of marketing exclusivity, 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 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 European Union 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 European Union rules with respect to cross-border transfers of personal data out of the European Union and European Economic Area. We are subject to the supervision of local data protection authorities in those European Union 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, California recently enacted the California Consumer Privacy Act ("CCPA"), which requires covered companies to provide new disclosures to California residents and honor their requests to access, delete and opt-out of certain sharing of their personal data. The CCPA, which took effect on January 1, 2020, provides for civil penalties for violations and private right of action for certain data breaches. The CCPA will expand substantially as a result of California voters approving a November 2020 ballot measure that adopted the California Privacy Rights Act of 2020, which will, among other things, create a new administrative agency to implement and enforce California's privacy laws effective January 1, 2023. While certain clinical trial activities are exempt from the CCPA's requirements, other personal data that we handle may be subject to the CCPA, which 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. 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. 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 European Union, 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.

Among the Affordable Care Act's provisions of importance to the pharmaceutical industry, in addition to those otherwise described above, are the following:

- an annual, nondeductible fee on any entity that manufactures or imports certain specified branded prescription drugs and biologic agents apportioned among these entities according to their market share in some government healthcare programs;

•an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program to 23.1% and 13% of the average manufacturer price for most branded and generic drugs, respectively, and a cap on the total rebate amount for innovator drugs at 100% of the Average Manufacturer Price ("AMP");

•a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 70% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturers' outpatient drugs to be covered under Medicare Part D;

•extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;

•expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals, including individuals with income at or below 133% of the federal poverty level, thereby potentially increasing manufacturers' Medicaid rebate liability;

•expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program; and

•a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

There have been executive, judicial and Congressional challenges to certain aspects of the Affordable Care Act. For example, the Tax Act was enacted, which included a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the Affordable Care Act on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." On June 17, 2021, the U.S. Supreme Court dismissed a challenge on procedural grounds that argued the Affordable Care Act is unconstitutional in its entirety because the "individual mandate" was repealed by Congress. Thus, the Affordable Care Act will remain in effect in its current form. Prior to the U.S. Supreme Court ruling, on January 28, 2021, the current administration issued an executive order to initiate a special enrollment period from February 15, 2021 through August 15, 2021 for purposes of obtaining health insurance coverage through the Affordable Care Act marketplace. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the Affordable Care Act. It is possible that the Affordable Care Act will be subject to judicial or Congressional challenges in the future. It is unclear how such challenges and the healthcare reform measures of the current administration will impact the Affordable Care Act and our business.

Other legislative changes have also been proposed and adopted in the United States since the Affordable Care Act was enacted. On August 2, 2011, the Budget Control Act of 2011, among other things, included aggregate reductions to Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2031, except for a temporary suspension from May 1, 2020 through March 31, 2022, unless additional Congressional action is taken.. Under current legislation, the actual reduction in Medicare payments will vary from 1% in 2022 to up to 3% in the final fiscal year of this sequester. In January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Additionally, on March 11, 2021, President Biden signed the American Rescue Plan Act of 2021 into law, which eliminates the statutory Medicaid drug rebate cap, currently set at 100% of a drug's average manufacturer price, for single source and innovator multiple source drugs, beginning January 1, 2024. In addition, Congress is considering additional health reform measures.

There has been heightened governmental scrutiny recently over the manner in which pharmaceutical companies set prices for their marketed products, which has resulted in several Congressional inquiries and proposed federal legislation, as well as state efforts, designed to, among other things, bring more transparency to product pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products

At the federal level, the previous administration used several means to propose or implement drug pricing reform, including through federal budget proposals, executive orders and policy initiatives. For example, on July 24, 2020 and September 13, 2020, the previous administration announced several executive orders related to prescription drug pricing that attempt to implement several of the administration's proposals. The FDA also concurrently released a final rule and guidance in September 2020 implementing a portion of the importation executive order providing pathways for states to build and submit importation plans for drugs from Canada. Further, on November 20, 2020, the U.S. Department of Health and Human Services ("HHS") finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The implementation of the rule has been delayed by the current administration from January 1, 2022 to January 1, 2023 in response to ongoing litigation. The rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a new safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufacturers, the implementation of which have also been delayed pending review by the current administration until January 1, 2023. On November 20, 2020, Centers for Medicare & Medicaid Services ("CMS") issued an interim final rule implementing the previous administration's Most Favored Nation executive order, which would tie Medicare Part B payments for certain physician-administered drugs to the lowest price paid in other economically advanced countries, effective January 1, 2021. As a result of litigation challenging the Most Favored Nation model, on December 27, 2021, CMS published a final rule that rescinds the Most Favored Nation model interim final rule. Further, in July 2021 the current administration released an executive order, "Promoting Competition in the American Economy," with multiple provisions aimed at prescription drugs. In response to the current administration's executive order, on September 9, 2021, HHS released a Comprehensive Plan for Addressing High Drug Prices that outlines principles for drug pricing reform and sets out a variety of potential legislative policies that Congress could pursue as well as potential administrative actions HHS can take to advance these principles. No legislation or administrative actions have been finalized to implement these principles. It is unclear whether these or similar policy initiatives will be implemented in the future.

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. 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. Further, it is possible that additional governmental action will be taken in response to the COVID-19 pandemic.

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 will be 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.

Previously, in the European Union, pursuant to the EU Clinical Trials Directive 2001/20/EC, a CTA had to be submitted to each country's national regulatory authority in which the clinical trial was to take place, together with an independent ethics committee, much like the FDA and IRB, respectively. Although the Directive had sought to harmonize the EU clinical trials regulatory framework, EU Member States transposed and applied the provisions of the Directive differently, leading to significant variation in the regulatory regimes of the member states. In 2014, a new Clinical Trials Regulation 536/2014, replacing the current Directive, was adopted. The new Regulation is directly applicable in all EU Member States (without national implementation) and entered into application on 31 January 2022. The new Regulation seeks to simplify and streamline the approval of clinical trials in the European Union. Pursuant to the Regulation, the sponsor shall submit a single CTA via the EMA's Clinical Trials Information System, or CTIS, which will cover all regulatory and ethics assessments from the member states concerned.

Any submissions made from January 31, 2023 onwards must be made through CTIS and all trials authorized pursuant to the Directive that are still ongoing on January 31, 2025 must be made through CTIS. Once the CTA is approved in accordance with a member state's requirements, clinical trial development may proceed. Approval and monitoring of clinical trials in the European Union is, as it was under the Directive, the responsibility of individual member states, but compared to the position prior to the applicability of the Clinical Trials Regulation there is likely to be more collaboration, information-sharing, and decision-making between member states. The new Regulation also aims to streamline and simplify the rules on safety reporting and introduces enhanced transparency requirements, such as mandatory submission of a summary of the clinical trial results to a new E.U. Database.

The requirements and process governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, 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.

To obtain regulatory approval of an investigational drug or biological product under European Union regulatory systems, we must submit an MAA either under the so-called centralized or national authorization procedures.

Centralized procedure. The centralized procedure provides for the grant of a single marketing authorization by the European Commission (following the EMA's Committee for Medical Products for Human Use adopting a positive opinion) that is valid in all European Union member states, as well as Iceland, Liechtenstein and Norway. The centralized procedure is compulsory for medicines produced by specified biotechnological processes, products designated as orphan medicinal products, and products with a new active substance indicated for the treatment of specified diseases, such as HIV/AIDS, cancer, diabetes, neurodegenerative disorders or autoimmune diseases, other immune dysfunctions and viral diseases. The centralized procedure is optional for other products that represent a significant therapeutic, scientific or technical innovation, or whose authorization would be in the interest of public health or which contain a new active substance for indications other than those specified to be compulsory.

National authorization procedures. There are also two other possible routes to authorize medicinal products in several European Union countries, which are available for investigational medicinal products that fall outside the scope of the centralized procedure:

- *Decentralized procedure.* Using the decentralized procedure, an applicant may apply for simultaneous authorizations in more than one European Union Member State of medicinal products that have not yet been authorized in any European Union Member State and that do not fall within the mandatory scope of the centralized procedure.

- *Mutual recognition procedure.* In the mutual recognition procedure, a medicine is first authorized in one European Union Member State, in accordance with the national procedures of that country. Following this, further marketing authorizations can be sought from other European Union countries in a procedure whereby the countries concerned agree to recognize the validity of the original, national marketing authorization.

The EMA grants orphan drug designation to promote the development of products for the treatment, prevention or diagnosis of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 people in the European Union. In addition, orphan drug designation can be granted if the drug is intended for a life threatening or chronically debilitating condition in the European Union and without incentives it is unlikely that sales of the drug in the European Union would be sufficient to justify the investment required to develop the drug. Orphan drug designation is only available if there is no other satisfactory method approved in the European Union of diagnosing, preventing or treating the condition, or if such a method exists, the proposed orphan drug will be of significant benefit to patients. Orphan drug designation provides opportunities for free or reduced-fee protocol assistance, fee reductions for MAAs and other post-authorization activities and ten years of market exclusivity following drug approval, which can be extended to 12 years if trials are conducted in accordance with an agreed-upon pediatric investigational plan. During the exclusivity period, the EMA and the relevant regulatory authorities of EU Member States may not accept another application for, or grant, a marketing authorization for the same indication in respect of a similar product. The exclusivity period may be reduced to six years if the designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity.

For other countries outside of the European Union, 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.

If we or our potential collaborators fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Human Capital Management

As of December 31, 2021, we employed 137 employees, all of whom are full-time, consisting of clinical, research, operations, finance and business development personnel. Twenty-six of our employees hold Ph.D. or M.D. degrees. Further, 119 of our employees are located in the United Sates, and 18 are located in Switzerland. As of December 31, 2021, 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 2022, 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. In response to COVID-19, we have implemented and continue to enhance safety measures in all our facilities, including establishing clear and regular COVID-19 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 team of employees and are committed to equality, inclusion and workplace diversity. 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 consisting of four 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 to ensure we meet our diversity objectives. We were certified as a Great Place to Work® in December 2021 after 92% of our employees participated in their 2021 Global Employee Engagement Study.

Corporate Information

We were incorporated in Delaware in May 2018. Our principal executive offices are located at 950 Tower Lane, Suite 1050, Foster City, California 94404, and our telephone number is (650) 667-4085. Our corporate website address is www.mirumpharma.com. 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.

Emerging Growth Company

We are an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012 ("JOBS Act"). We may take advantage of certain exemptions from various public company reporting requirements, including not being required to have our internal control over financial reporting audited by our independent registered public accounting firm under Section 404 of the Sarbanes-Oxley Act of 2002 ("Sarbanes-Oxley Act"), reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and any golden parachute payments. We may take advantage of these exemptions until December 31, 2024 or until we are no longer an "emerging growth company," whichever is earlier. We will cease to be an emerging growth company prior to the end of such period if certain earlier events occur, including if we become a "large accelerated filer" as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended ("Exchange Act"), our annual gross revenues exceed $1.07 billion or we issue more than $1.0 billion of non-convertible debt in any three-year period.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This provision allows an emerging growth company to delay the adoption of accounting standards that have different effective dates for public and private companies until those standards would otherwise apply to private companies. We have not elected to avail ourselves of this exemption and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not "emerging growth companies."

We are also a "smaller reporting company" as defined in the Exchange Act. We may continue to be a smaller reporting company even after we are no longer an emerging growth company. We may take advantage of certain of the scaled disclosures available to smaller reporting companies and will be able to take advantage of these scaled disclosures for so long as our voting and non-voting common stock held by non-affiliates is less than $250.0 million measured on the last business day of our second fiscal quarter, or our annual revenue is less than $100.0 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is less than $700.0 million measured on the last business day of our second fiscal quarter.

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 Products

Livmarli is our only FDA-approved product and the success of our business depends, in part, on its continued successful commercialization.

Livmarli is approved by the FDA for the treatment of cholestatic pruritus in patients with ALGS one year of age and older. The success of our business will depend, in part, on the continued successful commercialization of Livmarli. The successful commercialization of Livmarli depends on a number of factors, including, among others, the following:

•our ability to maintain our sales team and scale our distribution capabilities;

•the availability of adequate reimbursement for Livmarli;

•acceptance by physicians, payors and patients of the benefits, safety and efficacy of Livmarli, including relative to alternative and competing treatments;

•a continued acceptable safety profile of Livmarli;

•our ability to successfully obtain the substances and materials used in manufacturing Livmarli 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 Livmarli and avoid third-party patent interference or intellectual property infringement claims; and

•sufficient patient population that would benefit from Livmarli as ALGS is a rare disease and the patient population is small.

If one or more of the factors for successful commercialization 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 successfully commercialize Livmarli, which would harm our business, financial condition, operating results and prospects.

As a company we currently have limited marketing and sales experience. If we are unable to adequately maintain and scale our marketing and sales capabilities or enter into or lose rights pursuant to agreements with third parties to market and sell our products, we may not be able to generate viable product revenues. Even if we adequately establish such capabilities, market acceptance or reimbursement of our products may be lower than expected.

To successfully commercialize Livmarli we must 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 expect that the majority of pediatric cholestatic patients will be treated at tertiary care centers and transplant centers and therefore can be addressed with a targeted sales force. We have established our own commercial capabilities in North America to target these centers. We are also establishing a team to target similar centers in Western Europe, if we receive approval of Livmarli in ALGS from the EMA. However, our projections of

the commercial and sales needs to target these centers may not be accurate given our limited marketing and sales experience. If we are materially off from our projections, our business and operating results would be harmed.

We are in the process of establishing our capabilities in major European markets and have entered into a limited number of partner and distributor agreements in other select geographies. We will evaluate opportunities to partner with pharmaceutical companies that have established sales and marketing capabilities to commercialize Livmarli and our other product candidates, if approved, outside of these geographies.

The growth and maintenance of our own sales force to market Livmarli are 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 products. To the extent we also rely on third parties to commercialize Livmarli 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 or termination of rights pursuant to these agreements could delay or hinder our commercialization efforts. In the event we are unable to successfully develop, maintain and grow our own marketing and sales force or collaborate with a third-party marketing and sales organization, we would not be able to commercialize Livmarli and our other product candidates, if approved.

Our commercial success may be severely hindered if we are unable to obtain adequate coverage and reimbursement for Livmarli and any 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, is critical to market acceptance and commercial success of Livmarli, which is available in the United States only by prescription. Timely coverage and acceptable patient cost-sharing tiers for Livmarli 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 or limit changes in list price;
- changes in federal, state or foreign government regulations or private third-party payors' reimbursement policies;
- consolidation and increasing assertiveness of commercial payors seeking net price reduction via drug rebates and other forms of discounts linked to the placement of Livmarli 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 therapy requirements or formulary exclusion) 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. 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.

Although private third-party payors in the United States tend to follow Medicare reimbursement policies for products which are administered in a hospital or physician office setting, no uniform policy of coverage and reimbursement for drug products exists among third-party payors. Therefore, 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 Livmarli to

each third-party payor separately, with no assurance that coverage will be obtained. Additionally, coverage policies and third-party reimbursement rates may change at any time and from time to time. Even if favorable coverage and reimbursement status is attained for one or more products for which we or our collaborators receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

In addition, third-party payors may limit coverage to specific product candidates on an approved list, known as a formulary, which might not include all FDA-approved drugs for a particular indication. The market for Livmarli will depend significantly on access to third-party payors' drug formularies, or lists of medications for which third-party payors provide coverage and impose patient out-of-pocket cost sharing limits. The industry competition to be included in such formularies often leads to downward pricing pressures on pharmaceutical companies. Also, third-party payors may refuse to include a particular branded drug in their formularies or otherwise restrict patient access to a branded drug when a less costly generic equivalent or other therapeutically similar alternative is available.

Patients who are prescribed medicine for the treatment of their conditions generally rely on third-party payors to reimburse all or part of the costs of their prescription drugs. Even if we obtain favorable coverage for Livmarli, the patient may be required to pay co-payments or co-insurance they find unacceptably high. Patients may be unlikely to use Livmarli unless coverage is established and reimbursement for their medicine from their insurer adequately covers a significant portion of the cost of Livmarli.

Our inability to promptly obtain insurance coverage, profitable reimbursement rates or access to third-party payors drug formularies from private payors and government-funded health insurance for Livmarli could have a material adverse effect on our business, financial condition, operating results and prospects.

Livmarli may fail to achieve the broad degree of physician and patient adoption and use necessary for commercial success.

The commercial success of Livmarli depends significantly on the broad adoption and use of the product by physicians and patients for the approved indication. The degree and rate of physician and patient adoption of Livmarli depends on a number of factors, including, among other things:

- patient demand for Livmarli;
- our ability to successfully compete with currently available off-label therapies, future approved therapies, and existing therapies in development and available for use through expanded access programs, including demonstrating that the relative cost, safety and efficacy of Livmarli provides an attractive alternative to such existing therapies;
- the availability of adequate reimbursement from private third-party payors for Livmarli;
- the cost of treatment with Livmarli in relation to alternative treatments and patients willingness to pay for Livmarli;
- acceptance by physicians, tertiary care centers and transplant centers and patients of Livmarli as a safe and effective treatment;
- physician and patient willingness to adopt a new therapy over other available therapies to treat cholestatic pruritus in patients with ALGS;
- patients and physicians perception of cholestatic pruritus as a condition for which medical treatment may be appropriate;
- overcoming any biases physicians or patients may have toward particular therapies for the treatment of cholestatic pruritus;

•Livmarli patients and caregivers properly using Livmarli as instructed;

•patient satisfaction with the results and administration of Livmarli and overall treatment experience;

•patient satisfaction leading to a high percentage of patients deriving clinical benefit and staying on Livmarli chronically in the real world setting, as has been seen in clinical trials;

•the willingness of patients to pay for Livmarli relative to discretionary items;

•the prevalence and severity of side effects from the use or potential misuse of Livmarli;

•limitations or warnings contained in the FDA-approved labeling of Livmarli;

•the effectiveness of our sales, marketing and distribution efforts;

•adverse publicity about Livmarli or favorable publicity about competitive products;

•potential product liability claims;

•the ability of specialty pharmacies we contract with to process prescriptions and dispense Livmarli and the processes required to place orders with those pharmacies;

•the ability of our patient services hub to provide adequate support for patients and physicians to prescribe and access Livmarli;

•our ability to effectively manage our third-party supply and manufacturing operations while increasing production capabilities for Livmarli to commercial levels; and

•our ability to manage our growth and operations to effectively support our commercialization activities.

If Livmarli fails to achieve the broad degree of physician, patient and payor adoption 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.

Livmarli may cause undesirable side effects or have other unexpected properties that could limit its commercial profile, result in post-approval regulatory action or expose us to product liability claims, any of which may adversely impact our business, financial condition, operating results and prospects.

If we or others identify undesirable side effects or other previously unknown problems caused by Livmarli or other products with the same or related active ingredients, a number of potentially negative consequences could result, including, among others:

•the FDA may withdraw its approval of Livmarli;

•we could be sued and held liable for harm caused to patients;

•regulatory authorities may require a recall of Livmarli or we or our potential partners may voluntarily recall the product;

•regulatory authorities may require the addition of warnings or contraindications in the product labeling, narrowing of the indication in the Livmarli label or field alerts to physicians;

•we may be required to create a medication guide outlining the risks of such side effects for distribution to patients or institute a REMS;

•we may have limitations on how we promote Livmarli;

•we may be required to change the way Livmarli is administered or modify the product in some other way;

•regulatory authorities may require additional clinical trials or costly post-marketing testing and surveillance to monitor the safety or efficacy of Livmarli;

•undesirable side effects may limit physicians' or patients' willingness to initiate or continue therapy with Livmarli

•sales of Livmarli may decrease significantly; and

•our brand and reputation may suffer.

Any of the above events resulting from undesirable side effects or other previously unknown problems could prevent us or our potential partners from achieving or maintaining market acceptance of Livmarli and could substantially increase our costs, which may adversely affect our business, financial condition, operating results 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 United States, 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 the Medicaid Drug Rebate Program, as administered by CMS, and other federal and state government pricing programs in the United States, and we may participate in additional government pricing programs 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, President Biden signed the American Rescue Plan Act of 2021 into law, which eliminates the statutory Medicaid drug rebate cap, currently set at 100% of a drug's AMP, for single source and innovator multiple source drugs, beginning 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. 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 successful 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 for Livmarli and our product candidates. Livmarli 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 Livmarli or our product candidates could result in injury to a patient or even death. We may face product liability suits in the future, and our insurance coverage may not be sufficient to cover our liability under any such cases.

In addition, a liability claim may be brought against us even if Livmarli or our product candidates merely appear to have caused an injury. Product liability claims may be brought against us by, among others, consumers, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our product or product candidates. If we cannot successfully defend ourselves against product liability claims, we will incur substantial liabilities and reputational harm. In addition, regardless of merit or eventual outcome, product liability claims may result in, among other things:

•the inability to commercialize Livmarli or our product candidates, if approved;

•decreased demand for Livmarli or our product candidates;

•product recall or withdrawal from the market or labeling, marketing or promotional restrictions;

•impairment of our business reputation;

•substantial costs of any related litigation or similar disputes;

•distraction of management's attention and other resources from our primary business;

•substantial monetary awards to patients or other claimants against us that may not be covered by insurance; or

•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 Livmarli, or unapproved uses of our other product candidates, if and when approved, or if physicians misuse or use off-label Livmarli or our other product candidates, if and when 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 and other regulatory agencies 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 or such other regulatory agencies 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 suffer from other forms of cholestatic pruritus, we are only able to promote Livmarli in the United States for cholestatic pruritus associated with ALGS in patients one year of age and older. If we are found to have promoted off-label uses of Livmarli or our other product candidates, if and when approved, we may receive warning or untitled letters and become subject to significant criminal and civil liability, which would materially harm our business. 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.

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. The federal government has levied significant civil and criminal penalties against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion and to undertake corrective remedies. 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.

We cannot, however, prevent a physician from prescribing Livmarli or our other product candidates, if and when approved, outside of their approved indication when, in the physician's independent professional medical judgment, he or she deems appropriate. Physicians or patients may also misuse Livmarli or our other product candidates, if and when approved, potentially leading to adverse results, side effects or injury, which may lead to product liability claims. If misused, we may become subject to costly litigation by physicians or their patients. Furthermore, the use of Livmarli or our other product candidates, if and when approved, for indications other than those approved by the FDA may not effectively treat such conditions, which could harm our reputation among physicians and patients.

We rely completely on third parties to supply, manufacture and distribute drug supplies for Livmarli, including certain sole-source suppliers and manufacturers.

We do not currently have, nor do we plan to acquire, the infrastructure or capability to supply, manufacture or distribute Livmarli, without third parties. Our ability to commercially supply Livmarli depends, in part, on our ability to contract with 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 manufacture of packaging, labeling and oral dosing dispensers. If we fail to develop and maintain supply relationships with these third parties, we may be unable to successfully commercialize Livmarli.

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

•fail to supply us with product on a timely basis or in the requested amount due to unexpected damage to or destruction of facilities, equipment, deliveries 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 commercial needs;

•be unable to meet our production demands, including due to issues related to their reliance on sole-source suppliers and manufacturers;

•supply us with product that fails to meet regulatory requirements or our requirements;

•become unavailable through business interruption or financial insolvency;

•lose regulatory status as an approved source;

•be unable or unwilling to renew current supply agreements when such agreements expire on a timely basis, on acceptable terms or at all; or

•discontinue production or manufacturing of necessary drug substances or products.

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 and foreign regulatory authorities. Any need to find and qualify new suppliers or manufacturers could delay production of Livmarli indefinitely, adversely impact our ability to market Livmarli and adversely affect our business. Replacements may not be available to us on a timely basis, on acceptable terms or at all. Additionally, we and our manufacturers do not currently maintain significant inventory of drug substances and other materials. In addition, quarantines, shelter-in-place and similar government orders, or the perception that such orders, shutdowns or other restrictions on the conduct of business operations could occur, related to COVID-19 or other infectious diseases could impact personnel at our third-party manufacturing facilities upon which we rely, or the availability or cost of materials, which could disrupt the supply chain for Livmarli. Further, our manufacturers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes or unstable political or social environments. If our manufacturers were to encounter any of these difficulties, or otherwise fail to comply with their contractual obligations, our ability to commercialize Livmarli would be jeopardized. Any interruption in the supply of a drug substance or other material or in the manufacture of Livmarli could have a material adverse effect on our business, financial condition, operating results and prospects. The FDA has granted Livmarli concurrent release of process performance qualification batches, which is often granted for orphan drugs.

Additionally, although we are ultimately responsible for ensuring compliance with regulatory requirements such as current good manufacturing practices (“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. A number of our contract suppliers and manufacturers must comply with cGMP requirements enforced by the FDA through its facilities inspection program and review of submitted technical information. If the safety of Livmarli is compromised due to a failure to adhere to applicable laws or for other reasons, we may not be able to successfully commercialize our product and we may be held liable for injuries sustained as a result. In addition, the manufacturing facilities of certain of our suppliers are located outside of the United States. This may give rise to difficulties in importing our product into the United States 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 or defective packaging. Any of these factors could adversely impact our ability to effectively commercialize Livmarli.

Livmarli is subject to ongoing and continued regulatory oversight. Failure to comply with applicable regulatory requirements could have a material adverse impact on our business.

We are subject to ongoing FDA obligations and continued regulatory review with respect to, among other things, the manufacturing, processing, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion and recordkeeping for Livmarli. These requirements include submissions of safety and other post-marketing information and reports and registration, as well as continued compliance with cGMP requirements and with the FDA's GCP.

We are also subject to FDA post-marketing requirements including the conduct and submission of non-clinical, clinical studies and registry studies.

In addition, manufacturers of drug and biologic products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP regulations. If we or a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where, or processes by which, the product is manufactured, a regulatory agency may impose restrictions on that product or us, including requesting that we initiate a product recall, or requiring notice to physicians, withdrawal of the product from the market or suspension of manufacturing.

We or our current and prospective partners may be subject to product recalls in the future that could harm our brand and reputation and could negatively affect our business.

We or our current and prospective partners may be subject to product recalls, withdrawals or seizures if Livmarli fails to meet specifications or is believed to cause injury or illness or if we are alleged to have violated governmental regulations including those related to manufacturing, labeling, promotion, sale or distribution. Any recall, withdrawal or seizure in the future could materially and adversely affect consumer confidence in our brand and lead to decreased demand for our product. In addition, a recall, withdrawal or seizure of Livmarli would require significant management attention, would likely result in substantial and unexpected expenditures and would harm our business, financial condition, operating results and prospects.

Our customers are concentrated and therefore the loss of a significant customer may harm our business. In addition, the actions of our customers could affect our ability to sell or market Livmarli profitably. Fluctuations in buying or distribution patterns by such customers could adversely affect the collectability of our accounts receivable, our revenues, financial condition, or results of operations.

We rely on a specialty pharmacy and a single distributor for all of our sales of Livmarli. Our revenues, financial condition or results of operations may be affected by fluctuations in buying or distribution patterns of these customers. These fluctuations may result from seasonality, pricing, wholesaler inventory objectives, or other factors, including the effects of the COVID-19 pandemic. In addition, the COVID-19 pandemic may increase the proportion of uninsured patients in the United States, which may increase utilization of our patient assistance or free drug program.

Further, if any of these customers becomes subject to bankruptcy, is unable to pay us for our product or is acquired by a company that wants to terminate the relationship with us, or if we otherwise lose any of these significant customers, our revenue, results of operations and cash flows would be adversely affected. Even if we replace the loss of a significant customer, such transition may result in a decline in our revenue, results of operations and cash flows. The failure of any of these customers could adversely affect the collectability of our accounts receivable, our revenues, financial condition or results of operations.

Risks Related to Our Business and Industry

We have a very limited operating history, and we have incurred significant losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future.

We were incorporated in May 2018 and commenced operations in November 2018, and we have a very limited operating history upon which you can evaluate our business and prospects. Our operations to date have been primarily focused on acquiring and in-licensing Livmarli and volixibat , organizing and staffing our company, business planning, raising capital, advancing Livmarli and volixibat through clinical development, preparing for commercialization of Livmarli and volixibat and subsequently, commercializing Livmarli. We have not yet demonstrated an ability to overcome many of the risks and uncertainties frequently encountered by companies in new and rapidly evolving fields, particularly in the biopharmaceutical area. Consequently, any predictions about our future performance may not be as accurate as they would be if we had a history of successfully developing and commercializing biopharmaceutical products.

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. We have only one product approved for commercial sale, and 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 significant losses since our inception in May 2018. For the years ended December 31, 2021 and 2020, we reported a net loss of $84.0 million and $103.3 million, respectively. As of December 31, 2021, we had an accumulated deficit of $257.2 million.

We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our clinical development of, and seek regulatory approvals for, our product candidates and as we continue commercializing Livmarli in the United States and prepare for commercialization in Western Europe, if approved. 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 revenues. Our prior losses and expected future 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.

Our business has been and could continue to be adversely affected by the continued COVID-19 pandemic in regions where we or third parties on which we rely have significant manufacturing facilities, concentrations of clinical trial sites or other business operations. The continuing COVID-19 pandemic could adversely affect our operations, including our workforce, which is currently primarily working remotely and at our clinical trial sites, as well as the business or operations of our manufacturers, clinical research organizations ("***CROs***") ***or other third parties with whom we conduct business.***

Our business has been and could continue to be adversely affected by the global COVID-19 pandemic. In response to COVID-19, we have implemented and continue to enhance safety measures in all our facilities, including establishing clear and regular COVID-19 policies, safety protocols and updates to all employees. The effects of the COVID-19 pandemic and our safety policy may negatively impact productivity, disrupt our business, delay our clinical programs and timelines and hinder our commercialization efforts, the magnitude of which will depend, in part, on the length and severity of any restrictions and limitations on our ability to conduct our business in the ordinary course. These and similar, and perhaps more severe, disruptions in our operations could negatively impact our business, operating results and financial condition.

As a result of COVID-19, we may experience, or continue to experience, ongoing disruptions that could severely impact our business, clinical trials and our commercialization efforts, including:

•the inability or difficulty of our sales force to interact in person with potential prescribers of Livmarli;

•disruption of our commercial supply chain which may lead to delay or loss of revenue and increased cost;

•disruption or delay of physicians seeing patients in person which may prevent such physicians from prescribing Livmarli;

•delays or difficulties in enrolling and retaining patients in our ongoing and planned clinical trials;

•delays in receiving approval from local regulatory authorities to initiate our planned clinical trials;

•delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;

•delays in clinical sites receiving the supplies and materials needed to conduct our clinical trials, including interruption in global shipping that may affect the transport of clinical trial materials;

•changes in local regulations as part of a response to the COVID-19 outbreak which may require us to change the ways in which our clinical trials are conducted, which may result in unexpected costs, or to discontinue the clinical trials altogether;

•diversion of healthcare resources away from the conduct of clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;

•interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel imposed or recommended by federal or state governments, employers and others, or interruption of clinical trial subject visits and study procedures, the occurrence of which could affect the integrity of clinical trial data;

•interruption or delays in the operations of the FDA or other regulatory authorities, which may impact review and approval timelines;

•risk that participants enrolled in our clinical trials will acquire COVID-19 while the clinical trial is ongoing, which could impact the results of the clinical trial, including by increasing the number of observed adverse events; and

•refusal of the FDA or other regulatory authorities to accept data from clinical trials in affected geographies.

These and other disruptions in our operations and the global economy could negatively impact our business, operating results and financial condition.

Our clinical trials have been, and may in the future be, affected by the COVID-19 pandemic. Similarly, our ability to recruit and retain principal investigators and site staff who, as healthcare providers, may have heightened exposure to COVID-19, may be adversely impacted.

Our ongoing or planned clinical trials may also be impacted by interruptions or delays in the operations of the FDA and comparable foreign regulatory agencies. For example, the inability of the FDA to inspect clinical and manufacturing sites due to COVID-19-related travel restrictions may interrupt or delay our NDA and MAA reviews. Any potential interruptions or delays could adversely affect the anticipated timelines of our NDA and MAA reviews.

We and our CROs have also made certain adjustments to the operation of our trials in an effort to ensure the monitoring and safety of patients and minimize risks to trial integrity during the pandemic in accordance with the guidance issued by the FDA, and may need to make further adjustments in the future. For example, in our ongoing clinical trials, we have adopted new protocols to allow for flexibility surrounding patient visits, including to have drug shipped to them and for patients to virtually check-in as opposed to attending standard hospital visits. Many of these adjustments are new and untested, may not be effective, and may have unforeseen effects on the progress and

completion of this clinical trial and the findings from this clinical trial. In addition, we may encounter delays in shipping of our study drug and other clinical trial materials. These events could delay our clinical trials, increase the cost of completing our clinical trials and negatively impact the integrity, reliability or robustness of the data from our clinical trials.

In addition, quarantines, shelter-in-place and similar government orders, or the perception that such orders, shutdowns or other restrictions on the conduct of business operations could occur, related to COVID-19 or other infectious diseases could impact personnel at our CROs or third-party manufacturing facilities upon which we rely, or the availability or cost of materials, which could disrupt the supply chain for our product candidates. To the extent our suppliers and service providers are unable to comply with their obligations under our agreements with them or they are otherwise unable to deliver or are delayed in delivering goods and services to us due to the COVID-19 pandemic, our ability to continue meeting clinical supply demand for our product candidates or otherwise advancing development of our product candidates may become impaired.

The spread of COVID-19 and actions taken to reduce its spread may also materially affect us economically. While the potential economic impact brought by, and the duration of, the COVID-19 pandemic may be difficult to assess or predict, there could be a significant disruption of global financial markets, reducing our ability to access capital, which could in the future negatively affect our liquidity and financial position. In addition, the trading prices for other biopharmaceutical companies have been highly volatile as a result of the COVID-19 pandemic. As a result, we may face difficulties raising capital through sales of our common stock or such sales may be on unfavorable terms.

COVID-19 and actions taken to reduce its spread continue to evolve. The extent to which COVID-19 may impede the development and commercialization of our product candidates, reduce the productivity of our employees, disrupt our supply chains, delay our clinical trials, reduce our access to capital or limit our business development activities, will depend on future developments, which are highly uncertain and cannot be predicted with confidence.

In addition, to the extent the ongoing COVID-19 pandemic adversely affects our business and results of operations, it may also have the effect of heightening many of the other risks and uncertainties described in this “Risk Factors” section.

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 revenues 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 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 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 or comparable foreign regulatory authority may not accept such patient-reported outcomes or caregiver-reported outcomes as validated. 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 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 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 or 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. For example, the FDA typically requires results from two well controlled Phase 3 clinical trials to support an NDA submission seeking approval to market a new drug. We believe that the results from a single Phase 3 clinical trial, if successful, would be sufficient to support an NDA submission seeking approval for Livmarli for the treatment of pruritus associated with PFIC; however, the FDA may not agree to this approach. Even if we believe the results from our Phase 3 clinical trials are positive, the FDA may require us to conduct additional Phase 3 trials before we are able to submit an application for approval.

To the extent that the results of our clinical trials are not satisfactory to the FDA or 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.

Further, each indication for which we are evaluating Livmarli and volixibat is a rare cholestatic 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 and volixibat. 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 studies. In addition, we are conducting clinical trials in countries that have not yet had Livmarli or volixibat trials conducted and we have not yet worked with such foreign regulatory authorities. As a result, we could face patient recruitment issues in certain countries where such foreign regulatory authorities are not familiar with Livmarli or volixibat. Additionally, other pharmaceutical companies targeting these same cholestatic liver diseases are recruiting clinical trial patients from these patient populations, and have expanded access programs available, which may delay or make it more difficult to fully enroll 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 or may require us to abandon one or more clinical trials altogether. As a result, we may experience new or additional delays and difficulties in enrollment, which would result in the delay of completion of such trials beyond our expected timelines.

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. Clinical trials often fail to demonstrate safety and efficacy of the product candidate studied for the target indication. Most product candidates that commence clinical trials are never approved by regulatory authorities for commercialization. In the case of Livmarli and volixibat, we are seeking to develop treatments for rare cholestatic liver 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. Importantly, because the measure of pruritus relies on subjective patient feedback, it is inherently difficult to evaluate, and is subject to placebo effect. It can be influenced by factors outside of our control and can vary widely from day to day for a particular patient, and from patient to patient and site to site within a clinical trial. The placebo effect may also have a significant impact on pruritus trials.

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. For example, volixibat has been evaluated primarily for the treatment of non-alcoholic steatohepatitis and has not been evaluated in ICP, PSC or PBC, and our clinical development strategy is predicated on observations of IBAT inhibition in cholestatic settings. Similarly, Livmarli has not yet been evaluated in BA or in subjects under 12 months of age. As such, our hypothesis of efficacy in these diseases will be evaluated in these target patient populations and may not show the desired clinical results. 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. For example, in the Phase 2 INDIGO clinical trial evaluating Livmarli in PFIC1 and PFIC2 patients, the primary efficacy analysis of sBA change from baseline to week 13 did not reach statistical significance for the overall group; however, a 48-week analysis of the clinical trial demonstrated a profound treatment response in a subset of patients with nt-PFIC2. In addition, we do not have experience in conducting placebo-controlled studies for PFIC, and we are studying higher doses of Livmarli than we previously have administered in this setting, in our Phase 3 MARCH clinical trial in PFIC. Furthermore, the Phase 3 MARCH clinical trial in PFIC has enrolled additional genetic sub-types of PFIC patients that may experience different results that those seen in Phase 2 trials. As a result, we may face significant setbacks as we conduct our placebo-controlled Phase 3 clinical trial in PFIC, which may delay or prevent regulatory approval of Livmarli. Further, as a result of the COVID-19 pandemic, 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 due to COVID-19 or actions taken to slow its spread, the integrity of data from our clinical trials may be compromised or not accepted by the FDA or other regulatory authorities, which would represent a significant setback for the applicable program.

Additional safety data generated from our expanded access program 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 or comparable foreign regulatory authorities along with other information, including information about product candidate CMC 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 studies to demonstrate the safety and efficacy of the product candidates in humans. Clinical testing is expensive, time consuming and uncertain as to outcome. In addition, we may rely in part on preclinical, clinical and quality data generated by CROs, and other third parties for regulatory submissions for our product candidates. While we have or will have agreements governing these third parties' services, we have limited influence over their actual performance. If these third parties do not make data available to us, or, if applicable, do not make regulatory submissions in a timely manner, in each case pursuant to our agreements with them, our development programs may be significantly delayed, and we may need to conduct additional studies or collect additional data independently. In either case, our development costs would increase.

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 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 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 IRBs;

•IRBs refusing to approve, 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 adverse effects;

•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 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;

•third-party vendors not performing manufacturing and distribution services in a timely manner or to sufficient quality standards;

•third-party clinical investigators losing the licenses or permits necessary to perform our clinical trials, not performing our clinical trials on our anticipated schedule or consistent with the clinical trial protocol, GCP, or other regulatory requirements;

•third-party contractors not performing data collection or analysis in a timely or accurate manner;

•third-party contractors becoming debarred or suspended or otherwise penalized by the FDA or other government or regulatory authorities for violations of regulatory requirements, in which case we may need to find a substitute contractor, and we may not be able to use some or all of the data produced by such contractors in support of our marketing applications; or

•the impact of COVID-19 on our ongoing and planned clinical trials.

We could also encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by a Data Safety Monitoring Board for such trial or by the FDA or comparable foreign regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including 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 or comparable foreign regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. In addition, changes in regulatory requirements and policies may occur, and we may need to amend clinical trial protocols to comply with these changes. Amendments may require us to resubmit our clinical trial protocols to IRBs for reexamination, which may impact the costs, timing or successful completion of a clinical trial.

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 authority 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 authority 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 or comparable foreign regulatory authority, 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 revenues 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 revenues. 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 United States and by comparable foreign regulatory authorities in foreign markets. In the United States, we are not permitted to market our product candidates until we receive regulatory approval from the FDA. 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 United States or internationally, we must demonstrate with substantial evidence from adequate and well-controlled clinical trials, and to the satisfaction of the FDA 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 and comparable foreign regulatory authorities. The FDA 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. For example, we have also submitted an MAA for the treatment of cholestatic liver disease in patients with ALGS to the EMA and expect potential approval in the fourth quarter of 2022. The MAA is comprised of the long-term ICONIC clinical trial in patients with ALGS, which showed a significant improvement on pruritus ($p<0.0001$) and improvement on other markers of cholestatic liver disease. The ICONIC data is supported by a new analysis, which includes an aggregated cohort of Livmarli-treated patients with ALGS ($n=84$) compared to a natural history control cohort, demonstrating a statistically significant improvement in six-year event-free survival ($p<0.0001$), with events defined as biliary diversion surgery, liver transplant, hepatic decompensation (ascites requiring therapy or variceal bleeding) or death. The EMA may not agree with our interpretation of this data and may ultimately not approve Livmarli based on this submission.

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

•the FDA 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 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 United States;

•we may be unable to demonstrate to the satisfaction of the FDA 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 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 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 or comparable foreign regulatory authorities to support the submission of an NDA or other comparable submissions in foreign jurisdictions or to obtain regulatory approval in the United States or elsewhere;

•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 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 or 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 foreign marketing application for our product candidates, the FDA or comparable foreign regulatory authority may grant approval contingent on the performance of costly additional clinical trials, including Phase 4 clinical trials, and/or the implementation of a REMS, which may be required to ensure safe use of the drug after approval. The FDA or the comparable foreign regulatory authority also may approve a product candidate for a more limited indication or patient population than we originally requested, and the FDA or comparable foreign regulatory authority 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 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 is smaller than we anticipate, we may not be able to achieve profitability and growth. We focus our clinical development of Livmarli on treatments for rare pediatric cholestatic liver diseases with relatively small patient populations. Given the small number of patients who have the diseases that we are targeting with Livmarli, it is critical to our ability to grow and become profitable that we continue to successfully identify patients with these rare pediatric cholestatic liver diseases. We are focusing our clinical development of volixibat as a treatment for ICP, PSC and PBC, diseases with relatively small patient populations. In addition, our estimates of the patient populations for our target indications, including ALGS, have been derived from a variety of sources, including the 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. For example, while we are evaluating Livmarli in patients with different types of PFIC in our Phase 3 MARCH clinical trial in PFIC, in prior studies of Livmarli, the Phase 2 INDIGO clinical trial in particular, all of the multi-parameter responders were in the nt-PFIC2 subpopulation. Further, the primary endpoint in our Phase 3 MARCH clinical trial in PFIC is designed to evaluate Livmarli's effect on pruritus associated with nt-PFIC2. As such, even if our Phase 3 MARCH clinical trial in PFIC shows positive results in other PFIC subgroups, the design of our clinical trial may limit the ability of our NDA to be approved beyond the nt-PFIC2 population, if at all. 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. Further, even if we obtain significant market share for our product candidates, because the potential target populations are very small, we may never achieve profitability despite obtaining such significant market share. Lastly, the potentially addressable patient population for PFIC and ALGS, or any of our potential indications, may even be further reduced as 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 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 United States, 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 United States, 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.

We have submitted an MAA to the EMA for approval of Livmarli for the treatment of cholestatic liver disease in patients with ALGS in the European Union ("EU"). As with the FDA, obtaining approval of an MAA from the European Commission, on the basis of a recommendation from the Committee for Medicinal Products for Human Use of the EMA, is a similarly lengthy and expensive process and the EMA has its own procedures for recommending such approval for product candidates. Regulatory authorities in jurisdictions outside of the United States and the EU also have requirements for approval for product candidates with which we must comply prior to marketing in those jurisdictions. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our product candidates in certain countries. 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 product candidates will be harmed, which would adversely affect our business, prospects, financial condition and results of operations.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval or result in significant negative consequences following marketing approval, if any.

As is the case with biopharmaceuticals generally, it is likely that there may be side effects and adverse events associated with our product candidates' use. Results of our clinical trials 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 by the FDA or other comparable foreign authorities. For example, we have observed increases in alanine aminotransferase ("ALT"), levels in certain patients being treated with Livmarli with ALGS. Furthermore, the safety profile in patients under 12 months of age is unknown and may be different than that observed in previous clinical trials. Results of our trials could reveal a high and unacceptable severity and frequency of these or other side effects.

In clinical trials of Livmarli, the most commonly reported adverse events ("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, gastrointestinal bleeding and bone fracture. The frequency of observed AEs has not increased over time. In Phase 1 clinical trials of volixibat, the most common AEs reported were mild to moderate GI events observed in the volixibat groups.

In such an event, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval for our product candidates for any or all targeted indications. 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 addition, we maintain an expanded access program for Livmarli in ALGS and PFIC, Patients who receive access to unapproved drugs through compassionate use or expanded access programs have life-threatening illnesses and generally have exhausted all other available therapies. The risk for SAEs, including those which may be unrelated to Livmarli, in this patient population is high and could have a negative impact on the safety profile of Livmarli, which could cause significant delays or impair our ability to obtain regulatory approval for Livmarli outside of the United States.

Additionally, if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such product candidates, a number of potentially significant negative consequences could result, including, among other things:

- regulatory authorities may withdraw approvals of such product;
- regulatory authorities may require additional warnings on the label;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients at significant cost;
- we could be sued and held liable for harm caused to patients; and
- our reputation or the reputation of our products may suffer.

Such events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, results of operations and prospects.

If we receive regulatory approval for a product candidate, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense and 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. In addition, if the FDA or a comparable foreign regulatory authority approves a product candidate, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for the approved product will be subject to extensive and ongoing regulatory requirements. For example, the FDA strictly regulates the promotional claims that may be made about drug products. In particular, a product may not be promoted for uses that are not approved by the FDA as reflected in the product's approved labeling. The FDA also requires submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with cGMP requirements and GCP for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with a product candidate, including AEs of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, 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, warning letters or holds on clinical trials;

•refusal by the FDA 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 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 and other regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval for our product candidates. 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 United States or Europe using accelerated approval 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 or conditional approval.

Disruptions at the FDA, EMA and other government agencies caused by funding shortages or global health concerns could negatively impact our business.

The ability of the FDA, EMA and other government agencies 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, which is inherently fluid and unpredictable.

Disruptions at the FDA, EMA and other government agencies may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the United States 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.

Separately, in response to the global COVID-19 pandemic, in March 2020, the FDA announced its intention to postpone most foreign and domestic inspections of manufacturing facilities and products. In July 2020, the FDA restarted routine pre-announced surveillance inspections and remote interactive evaluations of domestic manufacturing facilities on a risk-based basis. Regulatory authorities outside the United States have adopted similar restrictions or other policy measures in response to the COVID-19 pandemic. If a prolonged government shutdown occurs, or if global health concerns continue to prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

Even if we obtain regulatory approval for our product candidates, our product candidates may not gain market acceptance among physicians, patients, tertiary care centers, transplant centers and others in the medical community.

If any one of our product candidates is approved for commercialization, its acceptance will depend on a number of factors, including, among other things:

•the clinical indications for which the product candidate is approved;

•physicians, major operators of tertiary care centers and transplant centers and patients considering the product as a safe and effective treatment;

•the potential and perceived advantages of the product over alternative treatments;

•the prevalence and severity of any side effects;

•product labeling or product insert requirements of the FDA or other regulatory authorities, including, in particular, whether the approved label is limited to the treatment of symptoms, such as pruritus, as compared to the treatment of the underlying disease;

•limitations or warnings contained in the labeling approved by the FDA or other regulatory authorities;

•the timing of market introduction of the product as well as competitive products;

•the cost of treatment in relation to alternative treatments;

•the availability of coverage and adequate reimbursement by 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;

•relative convenience and ease of administration, including as compared to alternative treatments and competitive therapies; and

•the effectiveness of our sales and marketing efforts.

If any of our product candidates are approved but fail to achieve market acceptance among physicians, patients or others in the medical community, we will not be able to generate significant revenues, which would have a material adverse effect on our business, prospects, financial condition and results of operations. In addition, even if any of our product candidates gain acceptance, the markets for the treatment of patients with our target indications for Livmarli may not be as significant as we estimate.

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 United States and some 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. In particular, there have 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 Affordable Care Act was enacted in the United States. Among the provisions of the Affordable Care Act of importance to our potential product candidates, the Affordable Care Act: established an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents; expanded eligibility criteria for Medicaid programs; increased the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program; creates a new Medicare Part D coverage gap

discount program; established a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in and conduct comparative clinical effectiveness research, along with funding for such research; and established a Center for Medicare & Medicaid Innovation at the Centers for Medicare & Medicaid Services ("CMS") to test innovative payment and service delivery models to lower Medicare and Medicaid spending. At this time, we are unsure of the full impact that Affordable Care Act will have on our business. There have been executive, judicial and Congressional challenges to certain aspects of the Affordable Care Act. For example, U.S. federal tax legislation enacted in 2017, informally titled The Tax Cuts and Jobs Act ("Tax Act"), included a provision which repealed, effective January 1, 2019, the tax-based shared responsibility payment imposed by the Affordable Care Act on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." On June 17, 2021 the U.S. Supreme Court dismissed a challenge on procedural grounds that argued the Affordable Care Act is unconstitutional in its entirety because the "individual mandate" was repealed by Congress. Thus, the Affordable Care Act will remain in effect in its current form. Further, prior to the U.S. Supreme Court ruling, on January 28, 2021, the current administration issued an executive order that initiated a special enrollment period for purposes of obtaining health insurance coverage through the Affordable Care Act marketplace, which began on February 15, 2021 and remained open through August 15, 2021. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the Affordable Care Act. It is possible that the Affordable Care Act will be subject to judicial or Congressional challenges in the future. It is unclear how any such challenges and the health care reform measures of the current administration will impact the Affordable Care Act and our business.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. On August 2, 2011, the Budget Control Act of 2011 was signed into law, which, among other things, included reductions to Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, including the Bipartisan Budget Act of 2018, will remain in effect through 2031 unless additional Congressional action is taken. However, COVID-19 relief legislation, suspended the 2% Medicare sequester from May 1, 2020 through March 31, 2022. Under current legislation, the actual reduction in Medicare payments will vary from 1% in 2022 to up to 3% in the final fiscal year of this sequester. On January 2, 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers, including hospitals, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Additionally, on March 11, 2021, President Biden signed the American Rescue Plan Act of 2021 into law, which eliminates the statutory Medicaid drug rebate cap, currently set at 100% of a drug's average manufacturer price, for single source and innovator multiple source drugs, beginning January 1, 2024. In addition, Congress is considering additional health reform measures.

Further, there has been heightened governmental scrutiny in the United States 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.

At the federal level, the previous administration used several means to propose or implement drug pricing reform, including through federal budget proposals, executive orders and policy initiatives. For example, on July 24, 2020 and September 13, 2020, the previous administration announced several executive orders related to prescription drug pricing that attempt to implement several of the Administration's proposals. The FDA concurrently released a final rule and guidance in September 2020 implementing a portion of the importation executive order providing pathways for states to build and submit importation plans for drugs from Canada. Further, on November 20, 2020, the United States Department of Health and Human Services ("HHS") finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The implementation of the rule has been delayed by the current administration from January 1, 2022 to January 1, 2023 in response to ongoing litigation. The rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a new safe harbor for certain fixed fee arrangements between pharmacy benefit managers and

manufacturers, the implementation of which have also been delayed until January 1, 2023. On November 20, 2020, CMS issued an interim final rule implementing the previous administration's Most Favored Nation ("MFN") executive order, which would tie Medicare Part B payments for certain physician-administered drugs to the lowest price paid in other economically advanced countries, effective January 1, 2021. As a result of litigation challenging the MFN model, on December 27, 2021, CMS published a final rule that rescinded the MFN model interim final rule. In July 2021, the Biden administration released an executive order, "Promoting Competition in the American Economy," with multiple provisions aimed at prescription drugs. In response to Biden's executive order, on September 9, 2021, HHS released a Comprehensive Plan for Addressing High Drug Prices that outlines principles for drug pricing reform. The plan sets out a variety of potential legislative policies that Congress could pursue as well as potential administrative actions HHS can take to advance these principles. No legislation or administrative actions have been finalized to implement these principles.

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. 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 Livmarli 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.

We expect that the Affordable Care Act, these new laws and other healthcare reform measures that may be adopted in the future may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from third-party payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize Livmarli and our other product candidates, if approved. In addition, it is possible that additional governmental action will be taken in response to the COVID-19 pandemic.

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

We plan to seek regulatory approval for our product candidates internationally and, accordingly, we expect that we will be subject to additional risks related to operating in foreign countries if we obtain the necessary approvals, including:

- differing regulatory requirements in foreign countries, including differing reimbursement, pricing and insurance regimes;
- the potential for so-called parallel importing, which is what happens when a local seller, 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;
- unexpected changes in tariffs, trade barriers, price and exchange controls and other regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- 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 revenues, 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 United States;

•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 United States;

•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, 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.

If we fail to develop and commercialize additional product candidates, we may be unable to grow our business.

We plan to acquire rights to develop and commercialize product candidates in addition to Livmarli and volixibat. 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 do not currently have the necessary drug discovery personnel or expertise adequate 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 and applicable 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. 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 new product candidates require substantial technical, financial and human resources whether or not we ultimately identify any candidates. If we are unable to develop or commercialize any other product candidates, our business and prospects will suffer.

If we fail to develop our current and any future product candidates for additional indications, our commercial opportunity will be limited.

One of our strategies is to pursue clinical development of Livmarli and volixibat in additional cholestatic disease conditions such as BA, ICP, PSC and PBC.

Developing, obtaining regulatory approval and commercializing Livmarli and volixibat for additional indications will require substantial additional funding and is prone to the risks of failure inherent in drug development. We may not be able to successfully advance any of these indications through the development process. Even if we receive regulatory approval to market Livmarli and volixibat for the treatment of any of these additional indications, any such additional indications may not be successfully commercialized, widely accepted in the marketplace or more effective than other commercially available alternatives. If we are unable to successfully develop and commercialize Livmarli and volixibat for these additional indications, our commercial opportunity will be limited.

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 biopharmaceutical industry is characterized by intense competition and rapid innovation. Our potential competitors include major multinational pharmaceutical companies, established biotechnology companies, specialty 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. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. 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. 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.

Outside of surgery and Livmarli, there are no other approved therapies for use in patients with ALGS in the United States. UDCA, which is approved for the treatment of PBC, is sometimes used to treat patients with other cholestatic liver diseases. Cholestyramine and other bile salt resins, rifampin, and naltrexone are sometimes used to treat patients suffering from pruritus, and a number of drugs, including UDCA, rifampin and naltrexone are used off-label to treat patients suffering from cholestatic liver disease. In addition, there are product candidates in development for some of these indications.

We are aware of two other companies pursuing clinical development of therapies that reduce sBA levels via the IBAT pathway. GlaxoSmithKline plc and Albireo have IBATis in clinical development for cholestatic liver diseases. We are aware that Albireo has received approval for odevixibat for the treatment of pruritus in patients with PFIC in the United States and for the treatment of PFIC in Europe. Albireo has been granted orphan designation for PFIC in Europe and if Livmarli is deemed similar, it could prevent the approval of Livmarli in PFIC in Europe. Albireo has also announced the initiation of studies of odevixibat in BA and ALGS and plans to pursue other cholestatic liver diseases. We are aware that GlaxoSmithKline plc has completed a Phase 2 trial of its IBAT in PBC patients and has initiated a Phase 3 trial in PBC. We are also aware that Intercept Pharmaceuticals, Inc. is exploring BA as an indication for obeticholic acid. Further, we may compete with companies that are developing gene therapy for the treatment of PFIC. In adult settings of cholestasis, similar to pediatric settings, cholestyramine, UDCA, rifampin and naltrexone are commonly used agents. We are not aware of FDA approved therapeutics for the treatment of ICP or PSC. We are aware of several agents in clinical development for the treatment of PSC, including Cymabay's seladelpar, DURECT Corporation's DUR928, Gilead Sciences Inc.'s GS-9674, HighTide Biopharmaceutical Inc.'s HTD1801, Immunic Therapeutics' IMU-838, Intercept's Ocaliva, or obeticholic acid, NGM Biopharmaceuticals Inc.'s NGM282 and Pliant Therapeutics' PLN-74809. Intercept Pharmaceuticals, Inc.'s Ocaliva is approved as a second-line treatment for PBC. We are aware of several agents in clinical development for the treatment of PBC including Cymabay's seladelpar, Genfit's elafibranor and NGM Biopharmaceuticals, Inc.'s NGM282 and COUR Pharmaceuticals' CNP-104. We are also aware that Cara Therapeutics' Korsuva is in Phase 2 development for the treatment of moderate-to-severe pruritus associated with chronic liver disease.

Even though we have obtained orphan drug designation for Livmarli in PFIC, ALGS and BA, 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 United States 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 United States, or a patient population of greater than 200,000 individuals in the United States, but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the EU, the European Commission, on the basis of a positive opinion from the EMA Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the EU. In September 2013, the FDA granted orphan drug status to Livmarli for the treatment of patients with PFIC and ALGS in the United States. In October 2020, the FDA granted orphan drug status to Livmarli for the treatment of BA. We also received orphan

drug status for Livmarli for PFIC, ALGS and BA in the EU. 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 EMA from approving another marketing application for the same drug (or, in the case of the EMA, a similar drug) for the same indication for that time period. The applicable period is seven years in the United States and ten years in the EU, which may be extended by six months and two years, respectively, in the case of product candidates that have complied with the respective regulatory agency's agreed upon pediatric investigation plan. 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 EMA 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 EMA 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. Specifically, Albireo has been granted orphan designation for PFIC in the EU and if Livmarli is deemed similar it could prevent the approval of Livmarli for PFIC in the EU. 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, ALGS and BA, if we receive approval for Livmarli for a modified or different indication, our current orphan designations may not provide us with exclusivity.

Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process in the United States. 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.

Although we have received a breakthrough therapy designation for Livmarli, this may not lead to a faster development, regulatory review or approval process, and it does not increase the likelihood that Livmarli will receive marketing approval in the United States.

We have received a breakthrough therapy designation for Livmarli for the treatment of PFIC2. A breakthrough therapy is defined as a therapy that is intended, alone or in combination with one or more other therapies, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the therapy may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For therapies that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Therapies designated as breakthrough therapies by the FDA may also be eligible for priority review and accelerated approval. The breakthrough therapy designation for Livmarli may not result in a faster development process, review or approval compared to therapies considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, the FDA may later decide that Livmarli no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

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 Livmarli, our product candidates and any future product candidates that we may develop. We also intend to establish commercial partnerships outside of North America and in major European markets.

For example, we entered into a licensing agreement with CANbridge Pharmaceuticals, Inc. ("CANbridge"), pursuant to which CANbridge has agreed to develop and commercialize Livmarli in Greater China (China, Hong Kong, Macau and Taiwan). Under the terms of the licensing agreement, CANbridge has obtained the exclusive right to develop and commercialize Livmarli within the Greater China regions for ALGS, PFIC, and BA. Further, we entered into a licensing agreement, with GC Pharma, pursuant to which GC Pharma has agreed to develop and commercialize Livmarli in South Korea. Under the terms of the licensing agreement, GC Pharma has obtained the exclusive right to develop and commercialize Livmarli within South Korea for ALGS, PFIC, and BA. Lastly, we entered into an exclusive licensing agreement for the development and commercialization of Livmarli in Japan for ALGS, PFIC, and BA with Takeda. Under the terms of the agreement, Takeda will be responsible for regulatory approval and commercialization of Livmarli in Japan. Takeda will also be responsible for development, including conducting clinical trials in cholestatic indications.

Any of these 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. Moreover, we may not be successful in our efforts to establish a strategic partnership or other alternative arrangements for volixibat because it may be deemed to be at too early of a stage of development for collaborative effort, and third parties may not view volixibat as having the requisite potential to demonstrate safety and efficacy. 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 products 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 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 liver disease. 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 products. 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 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 EMA and other similar 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 products that we acquire may not be manufactured or sold profitably or achieve market acceptance.

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. We are highly dependent on our management, 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 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. 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, 2021, we had 137 full and part-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 Livmarli and volixibat, 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 products 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 also 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. However, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for our product candidates or otherwise advance our business. 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. 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 products and product candidates and, accordingly, may not achieve our research, development and commercialization goals.

Business disruptions could seriously harm our future revenues 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 manmade 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 Livmarli and volixibat. Our ability to obtain clinical supplies of Livmarli and volixibat 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 and other similar foreign regulatory bodies, including those laws that require the reporting of true,

complete and accurate information to the FDA and other similar foreign regulatory bodies; (2) manufacturing standards; (3) healthcare fraud and abuse laws in the United States 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 United States and in the EU, 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 Medicare, Medicaid and other federal healthcare 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 and state healthcare fraud and abuse laws, false claims laws, transparency laws, health information privacy and security 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 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;

•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 HITECH Act, 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; 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 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 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 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 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 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, 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 United States will also likely subject us to foreign equivalents of the healthcare laws mentioned above, among other foreign laws.

Our business is subject to complex and evolving 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, 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, processing) personal data and other sensitive information, including proprietary and confidential business data, trade secrets, intellectual property, data we collect about trial participants in connection with clinical trials, and sensitive third-party data.

Our data processing activities subject us to numerous data privacy and security obligations, such as various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contracts, and other obligations that govern the processing of personal data by us and on our behalf. In the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal data privacy laws, and consumer protection laws. For example, the federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), imposes specific requirements relating to the privacy, security, and transmission of individually identifiable health information. Additionally, the Telephone Consumer Protection Act ("TCPA") imposes specific requirements 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. Class action suits are the most common method for private enforcement.

At the state level, the California Consumer Privacy Act ("CCPA"), which took effect on January 1, 2020, imposes obligations on covered businesses. These obligations include, but are not limited to, providing specific disclosures in privacy notices and affording California residents certain rights related to their personal data. The CCPA provides for civil penalties for violations (up to $7,500 per violation) and a private right of action for certain data breaches. Although the CCPA exempts some data processed in the context of clinical trials, the CCPA may increase compliance costs and potential liability with respect to other personal data we maintain about California residents. The CCPA is expected to expand substantially as a result of the California Privacy Rights Act of 2020 ("CPRA"), effective January 1, 2023. The CPRA establishes a new California Privacy Protection Agency to implement and enforce the CPRA, which could increase the risk of enforcement. Other states have enacted data privacy laws. For example, Virginia passed the Consumer Data Protection Act, and Colorado passed the Colorado Privacy Act, both of which become effective in 2023. In addition, data privacy and security laws have been proposed at the federal, state, and local levels in recent years, which could further complicate compliance efforts. Privacy advocates and industry groups have also proposed, and may propose, standards with which we are legally or contractually bound to comply.

Outside the United States, an increasing number of laws, regulations, and industry standards apply to data privacy and security. For example, the European Union's General Data Protection Regulation ("EU GDPR"), the United Kingdom's GDPR ("UK GDPR"), Brazil's General Data Protection Law (Lei Geral de Proteção de Dados Pessoais, or "LGPD") (Law No. 13,709/2018), and China's Personal Information Protection Law ("PIPL") impose strict requirements for processing personal data.

Among the most stringent of these laws is the EU GDPR. The EU 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 the personal data. In addition, the EU GDPR expanded the definition of what is expressly noted to constitute personal data (including by broadening the relevant definition to capture expressly the "pseudonymized" or key-coded data that is commonly processed in a clinical trial-related context).

We may be subject to the EU GDPR because of our data processing activities that involve the personal data of individuals residing in the European Economic Area ("EEA") and/or UK, such as in connection with our clinical trials in Europe, and early access program in multiple EU countries, as well as in connection with any processing of personal data carried out in the context of the activities of our Dutch subsidiary. In addition, we maintain an office in Switzerland, which has privacy and data protection laws and regulations similar to the EU GDPR. 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 offences or convictions. In the UK, the UK Data Protection Act 2018 complements the UK GDPR in this regard. This fact may lead to greater divergence on the law that applies to the processing of such personal data across the EEA and/or UK, which may increase our costs and overall compliance risk.

Certain jurisdictions have enacted data localization laws and cross-border personal data transfer laws, which could make it more difficult to transfer information across jurisdictions (such as transferring or receiving personal data that originates in the EU, the UK or in other foreign jurisdictions). Existing mechanisms that facilitate cross-border personal data transfers may change or be invalidated. For example, absent appropriate safeguards or other circumstances, the EU GDPR generally restricts the transfer of personal data to countries outside of the European Economic Area ("EEA") that the European Commission does not consider to provide an adequate level of data privacy and security, such as the United States. To comply with the EU GDPR's restrictions on transfer of personal data out of Europe, we have relied on the Standard Contractual Clauses ("SCCs") for personal data transfers approved by the European Commission that are designed to be a mechanism to facilitate personal data transfers out of the EEA to these jurisdictions. Currently, these SCCs are a valid mechanism to transfer personal data outside of the EEA, but there exists some uncertainty regarding whether the SCCs will remain a valid mechanism. Additionally, the SCCs impose additional compliance burdens, such as conducting transfer impact assessments to determine whether additional security measures are necessary to protect the at-issue personal data. If we are unable to implement a valid solution for personal data transfers from Europe, including, for example, obtaining individuals' explicit consent to the transfer of their personal data to the United States or other countries, we will face increased exposure to regulatory actions, substantial fines and injunctions against transferring personal data from Europe. Inability to export personal data from Europe may also: restrict our activities in Europe; limit our ability to collaborate with partners as well as other service providers, contractors and other companies in Europe; limit our ability to receive personal data from our Dutch subsidiary, limit our Dutch subsidiary's ability to transfer personal data outside of Europe, and generally limit our ability to operate the business of our Dutch subsidiary as a seamlessly integrated part of our wider operations; and/or require us to increase our processing capabilities within Europe at significant expense or otherwise cause us to change the geographical location or segregation of our relevant systems and operations – any or all of which could adversely affect our operations or financial results. Additionally, Switzerland and the UK similarly restrict personal data transfers outside of those jurisdictions to countries such as the United States that do not provide an adequate level of personal data protection. Other countries outside of Europe (e.g. Russia, China, Brazil) have enacted or are considering enacting similar cross-border data transfer restrictions and laws requiring local data residency, which could increase the cost and complexity of delivering our services and operating our business.

The relationship between the UK and the EEA in relation to certain aspects of data protection law remains somewhat uncertain. On June 28, 2021, the European Commission issued an adequacy decision under the GDPR which allows transfers (other than those carried out for the purposes of UK immigration control) of personal data from the EEA to the UK to continue without restriction for a period of four years ending June 27, 2025. After that period, the adequacy decision may be renewed only if the UK continues to ensure an adequate level of data

protection. During these four years, the European Commission will continue to monitor the legal situation in the UK and could intervene at any point if the UK deviates from the level of data protection in place at the time of issuance of the adequacy decision. If the adequacy decision is withdrawn or not renewed, transfers of personal data from the EEA to the UK will require a valid 'transfer mechanism' and we may be required to implement new processes and put new agreements in place, such as SCCs, to enable transfers of personal data from the EEA to the UK to continue, which could disrupt our operations.

In addition, while the UK data protection regime currently permits data transfers from the UK to the EEA and other third countries covered by a European Commission adequacy decision, and currently includes a framework to permit the continued use of the existing version of the SCCs for personal data transfers from the UK to third countries, this is subject to change in the future, and any such changes could have implications for our transfers of personal data from the UK to the EEA and other third countries. In particular, the UK Information Commissioner's Office has stated that it is working on its own bespoke version of the Standard Contractual Clauses and it is not clear whether the new SCCs published by the European Commission will be accepted as a valid mechanism to permit the transfer of personal data from the UK to third countries and/or whether any UK version of the SCCs will supersede the existing and/or new EU version of the SCCs. This could necessitate the implementation of both UK and EU versions of SCCs, which would require significant resources and result in significant cost to implement and manage.

Failure to comply with the requirements of the EU GDPR and UK GDPR and the applicable national data protection laws of the EEA Member States/the UK may result in fines of up to €20,000,000 / £17,500,000 or up to 4% of the total worldwide annual turnover of the preceding financial year, whichever is higher. In addition to administrative fines, a wide variety of other potential enforcement powers are available to competent authorities in respect of potential and suspected violations of the EU GDPR and UK GDPR, including extensive audit and inspection rights, and powers to order temporary or permanent bans on all or some processing of personal data carried out by non-compliant actors. Implementing mechanisms to endeavor to ensure compliance with the EU GDPR and UK GDPR and relevant local legislation in EEA Member States and the UK may be onerous and may interrupt or delay our development activities, and adversely affect our business, financial condition, results of operations, and prospects. In addition to the foregoing, an actual or perceived breach of the EU GDPR, UK 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.

Additionally, these obligations may be subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions. The EU GDPR, the UK GDPR, the CCPA and many other laws and regulations relating to privacy and data protection are still being tested in courts, and they are subject to new and differing interpretations by courts and regulatory officials. 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. It is possible that the EU GDPR, the UK GDPR, the CCPA or other laws and regulations relating to privacy and data protection may be interpreted and applied in a manner that is inconsistent from jurisdiction to jurisdiction or inconsistent with our current policies and practices.

Although we endeavor to comply with all applicable data privacy and security obligations, we may at times fail (or be perceived to have failed) to do so. Moreover, despite our efforts, our personnel or third parties upon whom we rely may fail to comply with such obligations, which could negatively impact our business operations and compliance posture. For example, any failure by a third-party processor to comply with applicable law, regulations, or contractual obligations could result in adverse effects, including inability to or interruption in our ability to operate our business and proceedings against us by governmental entities or others.

Our 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 regulatory fines and bans on processing personal information, investigations and enforcement actions, penalties and other liabilities, claims for damages by affected individuals, orders to destroy or not use personal information, imprisonment of

company officials and damage to our reputation, any of which could materially affect our business, financial condition, results of operations and growth prospects.

The withdrawal of the UK from the EU, commonly referred to as "Brexit," may adversely impact our ability to obtain regulatory approvals of our product candidates in the UK, result in restrictions or imposition of taxes and duties for importing our product candidates into the EU or the UK, and may require us to incur additional expenses in order to develop, manufacture and commercialize our product candidates in the EU or the UK.

Following the result of a referendum in 2016, the UK left the EU on January 31, 2020, commonly referred to as Brexit. Pursuant to the formal withdrawal arrangements agreed between the UK and the EU, the UK was subject to a transition period until December 31, 2020 ("Transition Period"), during which EU rules continued to apply. A trade and cooperation agreement ("Trade and Cooperation Agreement") that outlines the future trading relationship between the UK and the EU applied provisionally from January 1, 2021 and formally entered into force on May 1, 2021.

Since a significant proportion of the regulatory framework in the UK applicable to our business and our product candidates is derived from EU directives and regulations, Brexit has had, and will continue to have, a material impact on the regulatory regime with respect to the development, manufacture, importation, approval and commercialization of our product candidates in the UK. For example, Great Britain (England, Scotland and Wales) is no longer covered by the centralized procedure for obtaining EU-wide marketing authorization from the EMA and a separate marketing authorization will be required to market our product candidates in the Great Britain. Any delay in obtaining, or an inability to obtain, any marketing approvals would delay or prevent us from commercializing our product candidates and restrict our ability to generate revenue and achieve and sustain profitability.

While the Trade and Cooperation Agreement provides for the tariff-free trade of medicinal products between the UK and the EU, there are additional non-tariff costs to such trade that did not exist prior to the end of the Transition Period. Further, should the UK diverge from the EU from a regulatory perspective in relation to medicinal products, tariffs could be put into place in the future. We could therefore, both now and in the future, face significant additional expenses to operate our business, which could significantly and materially harm or delay our ability to generate revenues or achieve profitability of our business. Any further changes in international trade, tariff and import/export regulations as a result of Brexit or otherwise may impose unexpected duty costs or other non-tariff barriers on us. These developments, or the perception that any of them could occur, may significantly reduce global trade and, in particular, trade between the EU and the UK It is also possible that Brexit may negatively affect our ability to attract and retain employees, particularly those from the EU.

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. 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 and certain patents and know-how related to Livmarli and volixibat from Satiogen. We have in-licensed certain patents and know-how related to volixibat from Shire and Sanofi. 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 and, in the case of Satiogen, our sublicensing revenues. 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 for Livmarli and volixibat. 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 and 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 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 or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

Our CROs are not our employees and, except for remedies available to us under our agreements with such CROs, we cannot 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 revenues 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, 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.

In addition, quarantines, shelter-in-place and similar government orders, or the perception that such orders, shutdowns or other restrictions on the conduct of business operations could occur, related to COVID-19 or other infectious diseases could impact personnel at our CROs, which could disrupt our clinical timelines, which could have a material adverse impact on our business, prospects, financial condition and results of operations.

We rely completely on third parties to manufacture our preclinical. clinical and commercial drug supplies, and these third parties may fail to obtain and maintain regulatory approval for their facilities, fail to provide us with sufficient quantities of drug product 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 our clinical and commercial drug supplies. Instead, we rely on contract manufacturers for such production.

We do not currently have any long-term agreements or commitment with a manufacturer to produce raw materials, active pharmaceutical ingredients (“APIs”) and the finished products of our product candidates or the associated packaging used in our current product formats. 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. As our product candidates are intended to treat rare liver diseases,

we will only require a low-volume of raw materials and APIs, and in the case of Livmarli and volixibat, 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 any product candidates that are approved. Further, any delay in identifying and qualifying a manufacturer for commercial production could delay the potential commercialization of our product candidates, and, in the event that we do not have sufficient product to complete our planned clinical trials, it could delay such trials. The facilities used by our contract manufacturers to manufacture our product candidates must be approved by the applicable regulatory authorities, including the FDA, pursuant to inspections that will be conducted after an NDA or comparable foreign regulatory marketing application is submitted. We do not control the manufacturing process of our product candidates and are completely dependent on our contract manufacturing partners for compliance with the FDA's cGMP requirements for manufacture of both the active drug substances and finished drug product. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the FDA's strict regulatory requirements, they will not be able to secure or maintain FDA approval for the manufacturing facilities. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or any other applicable regulatory authority does not approve these facilities for the manufacture of our product candidates or if it withdraws any such approval in the future, or if our suppliers or contract manufacturers decide they no longer want to supply or manufacture for us, 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 Livmarli and volixibat.

In addition, the manufacture of pharmaceutical products is complex and requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. In addition, manufacturers and their facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to current good manufacturing practices, or cGMPs. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up and validating initial production and absence of contamination. These problems include difficulties with production costs and yields, quality control, including stability of the product, quality assurance testing, operator error, shortages of qualified personnel, as well as compliance with strictly enforced federal, state and foreign regulations. Furthermore, if contaminants are discovered in our supply of Livmarli or volixibat or in the manufacturing facilities, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. Any stability or other issues relating to the manufacture of our product candidates may occur in the future. In addition, quarantines, shelter-in-place and similar government orders, or the perception that such orders, shutdowns or other restrictions on the conduct of business operations could occur, related to COVID-19 or other infectious diseases could impact personnel at our third-party manufacturing facilities upon which we rely, or the availability or cost of materials, which could disrupt the supply chain for our product candidates. Further, our manufacturers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes or unstable political environments. If our manufacturers were to encounter any of these difficulties, or otherwise fail to comply with their contractual obligations, our ability to provide our product candidate to patients in clinical trials would be jeopardized. Any delay or interruption in the supply of clinical trial supplies could delay the completion of 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.

Further, we are in the process of switching or adding contract manufacturing organizations for both Livmarli and volixibat, which is cost-intensive and time-consuming. The success of these transfers is necessary for continuous supply to clinical trials and potential future commercial demand.

Risks Related to Our Financial Position and Capital Requirements

We will need substantial additional financing to continue our commercialization efforts for Livmarli, develop our product candidates and implement our operating plans. If we fail to obtain additional financing, 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 commercialization efforts for Livmarli, 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 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 will 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 the COVID-19 pandemic and actions taken to slow its spread, 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 Livmarli or volixibat 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. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a stockholder. 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, such as the RIPA with the Purchasers, we may have to relinquish valuable rights to Livmarli, our intellectual property, future revenue streams or grant licenses on terms that are not favorable to us. For instance, as part of the RIPA, the Purchasers have the right to receive certain revenue interests from us based on the net sales of Livmarli, and we have granted the Purchasers a senior security interest in certain of our assets. 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.

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, 2021, we had federal and state net operating loss ("NOL") carryforwards of approximately $150.0 million and $6.7 million, respectively. The federal NOL carryforwards do not expire, and the state NOL carryforwards will begin to expire in 2039, unless previously utilized. Our ability to utilize our NOL carryforwards and certain other tax attributes may be limited. We also have federal and state research and development credit carryforwards totaling $23.1 million and $2.1 million,

respectively. The federal research and development credit carryforwards will begin to expire in 2032, unless previously utilized. The state research and development credits will not expire.

Under the Tax Act, as modified by the Coronavirus Aid, Relief, and Economic Security Act ("CARES Act"), federal NOLs generated in taxable years beginning after December 31, 2017 may be carried forward indefinitely, but the deductibility of such federal NOLs in taxable years beginning after December 31, 2020 is limited to 80% of taxable income. It is uncertain if and to what extent various states will conform to the Tax Act or the CARES Act. 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. In addition, at the state level, there may be periods during which the use of net operating losses is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. For example, on June 29, 2020, California enacted AB 85, which imposed limits on the usability of California state net operating losses and certain tax credits in tax years beginning after 2019 and before 2023. 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.

New or future changes to tax laws could materially adversely affect our company.

New income, sales, use or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could adversely affect our business operations and financial performance. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us. For example, the Tax Act enacted many significant changes to the U.S. tax laws. Future guidance from the Internal Revenue Service and other tax authorities with respect to the Tax Act may affect us, and certain aspects of the Tax Act could be repealed or modified in future legislation. For example, the CARES Act modified certain provisions of the Tax Act and proposals have recently been made in Congress (which have not yet been enacted) to increase the federal income tax rate applicable to corporate income and make other tax law changes that could have a material adverse impact on us. In addition, it is uncertain if and to what extent various states will conform to the Tax Act, the CARES Act or any newly enacted federal tax legislation. The impact of the Tax Act and CARES Act and any future changes in tax laws on holders of our common stock is also uncertain and could be adverse.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain sufficient intellectual property protection for Livmarli 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 Livmarli and our 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. 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 United States or in many jurisdictions outside of the United States. Changes in either the patent laws or interpretations of patent laws in the United States and other countries 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 Livmarli or our product candidates, proprietary technologies and their uses by obtaining and defending patents. These risks and uncertainties include the following:

- the United States 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 may 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 Livmarli or our potential 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 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 United States and most other countries 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 Livmarli 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 United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns; and
- countries other than the United States 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 United States 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 Livmarli, 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 Livmarli 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 countries 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 products;
- we cannot ensure that we will be able to successfully commercialize our products 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 interference, opposition or invalidity proceedings before U.S. or non-U.S. patent offices.

We cannot be certain that the claims in our issued patents and pending patent applications covering Livmarli or volixibat will be considered patentable by the USPTO, courts in the United States, or by patent offices and courts

in foreign countries. Furthermore, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. 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 Livmarli or volixibat in the United States or in foreign countries. Even if such patents do successfully issue, third parties may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. Any successful opposition to our patents could deprive us of exclusive rights necessary for the successful commercialization of Livmarli or volixibat. Furthermore, even if they are unchallenged, our patents may not adequately protect our intellectual property, provide exclusivity for Livmarli or volixibat or prevent others from designing around our claims. If the breadth or strength of protection provided by the patents we hold with respect to Livmarli or volixibat is threatened, it could dissuade companies from collaborating with us to develop, or threaten our ability to commercialize, Livmarli or volixibat.

Further, if we encounter delays in our development efforts, including our clinical trials, the period of time during which we could market Livmarli or volixibat under patent protection would be reduced. In addition, patents have a limited lifespan. In the United States, 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 United States 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, a 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 is developing 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. It remains unclear what impact the America Invents Act will have on the operation of our business. Moreover, 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 Livmarli 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 Livmarli and our product candidates, such as volixibat, 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 United States, Europe and other countries covering the methods of treating cholestatic liver diseases using IBATis, including Livmarli and volixibat. A patent based on any of these patent applications may never be issued. 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 formulation patent applications.

Composition-of-matter patents on active pharmaceutical ingredients 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 United States 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 countries 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 United States and other governments may, at any time, enact changes to law and regulation 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 countries throughout the world would be prohibitively expensive. In addition, the laws of some foreign countries do not protect intellectual property rights in the same manner and to the same extent as laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States. 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 United States. These products may compete with our products 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 countries. For example, unlike other countries, China has a heightened requirement for patentability, and specifically requires a detailed description of medical uses of a claimed drug. In India, unlike the United States, there is no link between regulatory approval for a drug and its patent status. In addition to India, certain countries in Europe and developing countries, including China, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In those countries, 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.

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 Livmarli 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 Livmarli and volixibat 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 Livmarli or volixibat, 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 Livmarli 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 including Shire, Pfizer, Satiogen and Sanofi, related to Livmarli and our product candidates. For example, we have our license agreements with Shire and Satiogen for both Livmarli and volixibat. We have our license agreement with Shire, Satiogen and Pfizer for our intellectual property rights covering Livmarli. 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 Livmarli 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 may 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 products, 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 United States, 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 Livmarli 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 Livmarli 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 Livmarli 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 Livmarli or one of our current or future product candidates. Nevertheless, we are not aware of any issued patents that will prevent us from marketing Livmarli 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 Livmarli 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 Livmarli, 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 Livmarli 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 Livmarli 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 products 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 United States, 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 Livmarli 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 United States may be maintained in secrecy until the patents are issued;

•patent applications in the United States 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, Livmarli, our product candidates or the use of 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 products. Further, we may incorrectly determine that our technologies, products, 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 United States or internationally that we consider relevant may be incorrect, which may negatively impact our ability to develop and market Livmarli 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 Livmarli, our product candidates and future approved products 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 Livmarli 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 Livmarli 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 United States. 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 countries 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 Livmarli 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 Livmarli 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 countries where the laws may not protect those rights as fully as in the United States.

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 United States, 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 relevant 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.

Because of the expense and uncertainty of litigation, we may not be in a position to enforce our intellectual property rights against third parties.

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 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 products 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 United States 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 will over time be disseminated within the industry through independent development, the publication of journal articles and the movement of personnel skilled in the art from 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 products 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 occurs 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 United States 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, it may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA. Furthermore, in many countries, 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 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 to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks. Similar requirements exist in Europe.

Any collaboration arrangements that we have or may enter into in the future may not be successful, which could adversely affect our ability to develop and commercialize our products.

Any existing or future collaborations that we enter into may not be successful. The success of our collaboration arrangements depend 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 products 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;
- 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 products 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 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 its trading began on July 18, 2019 and to March 8, 2022 has

ranged from a low of $6.84 to a high of $26.59. In addition to the factors discussed in this "Risk Factors" section, these factors include, among others:

- the degree of physician and patient adoption of Livmarli and use of Livmarli necessary for commercial success;
- our failure to grow and maintain our own sales force to market Livmarli;
- our ability to commercialize Livmarli in Western Europe, if approved, and our ability to grow and maintain an international sales force;
- any delay in our regulatory filings for Livmarli or volixibat 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 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;
- adverse regulatory decisions, including failure to receive regulatory approval for our product candidates;
- changes in laws or regulations applicable to Livmarli 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 Livmarli 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;
- management transitions and additions or departures of key scientific or management personnel;
- unanticipated serious safety concerns related to the use of Livmarli 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 ALGS, PFIC and other cholestatic liver diseases that we may target;

•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;

•general political, health and economic conditions, including the COVID-19 pandemic; and

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

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. Further, the RIPA limits our ability to declare dividends, including to whom they can be paid and the aggregate amount payable.

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.

Sales of a substantial number of shares of our common stock in the public market 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 reduce the market price of our common stock. As of December 31, 2021, there were 30,582,596 shares of our common stock outstanding, excluding 122,464 shares subject to repurchase, as described in the notes to our consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

In addition, 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 ("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 payment obligations under the RIPA may adversely affect our financial position or results of operations and our ability to raise additional capital which in turn may increase our vulnerability to adverse regulatory developments or economic or business downturns.

As described in "Management's Discussion and Analysis of Financial Condition and Results of Operations—Liquidity and Capital Resources," on December 8, 2020, we entered into the RIPA. Pursuant to the RIPA, the Purchasers paid us $50.0 million on closing and $65.0 million in April 2021, less certain transaction expenses. We may also be entitled to receive up to approximately $50.0 million at the option of the Purchasers to finance in-licenses or other acquisitions on or prior to December 31, 2022. As consideration for such payments, the Purchasers will have a right to receive certain Revenue Interests from us based on annual net sales of Livmarli, which will be tiered payments (the "Revenue Interest Payments") based on whether such annual net sales are (i) less than or equal to $350.0 million ("Tier 1"), (ii) exceeding $350.0 million and less than or equal to $1.1 billion ("Tier 2"), or (iii) exceeding $1.1 billion ("Tier 3"). The Revenue Interest Payments will initially be 9.75% (at Tier 1) and 2.00% (at Tier 2 and Tier 3) of such annual net sales in the Covered Territory; provided that (i) if the Purchasers have received Revenue Interest Payments in an amount equal to or greater than 110.0% of the total payments actually made by the Purchasers to us, exclusive of transaction expenses (the "Cumulative Purchaser Payments"), on or prior to December 31, 2026, the Revenue Interests shall be reduced to 2.00% at Tier 1 and 0.00% at Tier 3 for all subsequent calendar years beginning on January 1, 2027 and (ii) if the Purchasers have not received Revenue Interest Payments in an amount equal to or greater than 110.0% of the Cumulative Purchaser Payments on or prior to December 31, 2026, the Revenue Interests shall be increased for all subsequent calendar years beginning on January 1, 2027 to a single defined rate (with no separate tiers) that would have provided the Purchasers with an amount equal to 110.0% of the Cumulative Purchaser Payments on or prior to December 31, 2026 had such rate applied to Tier 1 of initial Revenue Interest Payments. The Purchasers' rights to receive the Revenue Interest Payments shall terminate on the date on which the Purchasers have received Revenue Interest Payments of 195.0% of the Cumulative Purchaser Payments, unless the RIPA is terminated earlier. The RIPA and the revenue interest stream payable to the Purchasers could have important negative consequences to the holders of our securities. For example, a portion of our cash flow from operations will be needed to pay certain revenue interests to the Purchasers and will not be available to fund future operations. Additionally, we may have increased vulnerability to adverse general economic and industry conditions. Payment requirements under the RIPA will increase our cash outflows. Our future operating performance is subject to market conditions and business factors that are beyond our control. If our cash inflows 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. If we raise funds by selling additional equity, such sale would result in dilution to our stockholders. If we are required to secure funding, we may not be able to do so on terms acceptable to us, or at all. Failure to pay certain amounts to the Purchasers when due would result in a default under the RIPA and result in foreclosure on certain of our assets which would have a material adverse effect on our operations and financial condition. The RIPA contains customary affirmative and negative non-financial covenants and events of default, including covenants and restrictions that, among other things, grant a senior security interest in our assets and restrict our ability to incur liens, incur additional indebtedness, make loans and investments, engage in mergers and acquisitions, and engage in asset sales. Additionally, the Purchasers under the RIPA have an option (the "Put Option") to terminate the RIPA and to require us to repurchase future Revenue Interests upon enumerated events such as a bankruptcy event, an uncured material breach, a material adverse effect (which can include adverse developments related to the regulatory approval of our product candidates) or a change of control. The triggering of the Put Option, including by our failure to comply with these covenants, could permit the Purchasers to declare certain amounts to be immediately due and payable. Further, if we are liquidated, the Purchasers' right to repayment would be senior to the rights of the holders of our common stock. Any triggering of the Put Option or other declaration by the Purchasers of an event of default under the RIPA could significantly harm our financial condition, business and prospects and could cause the price of our common stock to decline.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to the Shelf Registration, Sales Agreement and our equity incentive plans, 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. 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 the Shelf Registration. For example, in August 2020, we entered into the Sales Agreement with SVB Leerink, 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 $75.0 million under the Shelf Registration through SVB Leerink acting as the sales agent and/or principal. 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, 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, in March 2020, we adopted a 2020 Inducement Plan, pursuant to which 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.

Our business could be negatively affected as a result of actions of 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 a delisting, we can provide no assurance that any action taken by us to restore compliance with listing requirements would 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, the president 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 to amend any bylaws by stockholder action or to amend specific provisions of our 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 Delaware General Corporate Law, 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.

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 United States of America 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 Delaware General Corporation Law, 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 Delaware General Corporation Law 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 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 further provides that the federal district courts of the United States of America 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. 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

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

In the course of our business, we may process proprietary, confidential and sensitive information, including personal data (such as health-related data), intellectual property and trade secrets (collectively, sensitive information). We may rely upon third-party service providers and technologies to 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 email, and other functions. 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.

The sensitive information processed and stored in our technology systems, and those of our research collaborators, CROs, contractors, consultants and other third parties on which we depend to operate our business, may be vulnerable to cyberattacks, malicious internet-based activity and online and offline fraud. These threats come from a variety of sources, including traditional computer "hackers," threat actors, personnel (such as through theft or misuse), sophisticated nation states, and nation-state-supported actors. Such incidents may also result from errors or malfeasance by our personnel or the personnel of the third parties with which we work, malware (including as a result of advanced persistent threat intrusions), viruses, software vulnerabilities, hacking, denial of service attacks (such as credential stuffing), social engineering (including phishing), ransomware, supply-chain attacks, server malfunctions, software or hardware failure, loss of data or other information technology assets, adware, telecommunications failures and other similar threats. 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. Similarly, supply-chain attacks have increased in frequency and severity, and we cannot guarantee that third parties and infrastructure in our supply chain or our third-party partners' supply chains have not been compromised or that they do not contain exploitable defects or bugs that could result in a breach of or disruption to our information technology systems or the third-party information technology systems that support us and our services. Threat actors, personnel (such as through theft or misuse), sophisticated nation-states, and nation-state-supported actors now engage in cyber-attacks, 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 upon which we rely may be vulnerable to a heightened risk of these attacks, including cyber-attacks that could materially disrupt our systems and operations, supply chain, and ability to produce, sell and distribute our goods and services.

Additionally, as a result of the ongoing COVID-19 pandemic, certain functional areas of our workforce remain in a remote work environment and outside of our corporate network security protection boundaries, which imposes additional risks to our business, including increased risk of industrial espionage, phishing and other cybersecurity attacks and unauthorized dissemination of sensitive information, any of which could have a material adverse effect on our business.

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.

Any of the previously identified or similar threats could cause a security incident or other interruption. A security incident or other interruption could result in unauthorized, unlawful, or accidental acquisition, modification, destruction, loss, alteration, encryption, disclosure of, or access to our sensitive information.

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 may require us to implement and maintain specific security measures, industry-standard or reasonable security measures to protect our information technology systems and sensitive information. While we have developed systems and processes designed to protect the integrity, confidentiality and security of the sensitive information under our control, we cannot assure you that our security measures or those of the third parties we depend on will be effective in preventing security incidents. There are many different and rapidly evolving cybercrime and hacking techniques, and we may be unable to anticipate attempted security breaches, identify them before our sensitive information is exploited, or react in a timely manner.

Although, to our knowledge, we have not experienced a material system failure or security incident to date, if such an event were to occur, it could result in a material disruption of our development programs and our business operations, whether due to a loss of sensitive information or other similar disruptions. 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. Likewise, we rely on third-party research institution collaborators, CROs, other contractors and consultants for many aspects of our business, including research and development activities and manufacturing of Livmarli and our product candidates, and similar events relating to their computer systems could also have a material adverse effect on our business. 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. Such security incidents could also subject us to significant liability, harm our competitive position and delay the further commercialization and development of Livmarli and our product candidates.

If we (or a third party upon whom we rely) experience a security incident or are perceived to have experienced a security incident, we may experience adverse consequences. These consequences may include: government enforcement actions (for example, investigations, fines, penalties, audits, and inspections), additional reporting requirements and/or oversight, restrictions on processing sensitive information (including personal data), litigation (including class claims), indemnification obligations, negative publicity, reputational harm, monetary fund diversions, 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. Additionally, applicable data privacy and security obligations may require us to notify relevant stakeholders of security incidents. Such disclosures are costly, and the disclosure or the failure to comply with such requirements could lead to adverse consequences.

Our insurance coverage may not be adequate for cybersecurity liabilities, may not continue to be available to us on economically reasonable terms, or at all, and any insurer may deny coverage as to any future claim. 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.

Threat actors, personnel (such as through theft or misuse), sophisticated nation-states, and nation-state-supported actors now engage and are expected to continue to engage in cyber-attacks, 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 upon which we rely may be vulnerable to a heightened risk of these attacks, including cyber-attacks that could materially disrupt our systems and operations,

supply chain, and ability to produce, sell and distribute our goods and services. For example, we have operations and third parties upon which we rely to support our business located in unstable regions and regions experiencing (or expected to experience) geopolitical or other conflicts, including through the use of cyber-attacks.

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 United States, to sell our products 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 United States 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 are an emerging growth company and a smaller reporting company, and the reduced reporting requirements applicable to emerging growth companies and smaller reporting companies may make our common stock less attractive to investors.

We are an emerging growth company, as defined in the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and exemptions from the requirements of holding nonbinding advisory votes on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company until December 31, 2024, although circumstances could cause us to lose that status earlier, including if we become a "large accelerated filer" as defined in Rule 12b-2 under the Exchange Act or if we have total annual gross revenue of $1.07 billion or

more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31 or, if we issue more than $1.0 billion in non-convertible debt during any three year period before that time, we would cease to be an emerging growth company immediately. Even after we no longer qualify as an emerging growth company, we may still qualify as a "smaller reporting company" which would allow us to take advantage of many of the same exemptions from disclosure requirements including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. Investors may find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies. As a result, changes in rules of accounting principles generally accepted in the United States of America or their interpretation, the adoption of new guidance or the application of existing guidance to changes in our business could significantly affect our financial position and results of operations.

We are also a "smaller reporting company" as defined in the Exchange Act. We may continue to be a smaller reporting company even after we are no longer an emerging growth company. We may take advantage of certain of the scaled disclosures available to smaller reporting companies and will be able to take advantage of these scaled disclosures for so long as our voting and non-voting common stock held by non-affiliates is less than $250.0 million measured on the last business day of our second fiscal quarter, or our annual revenue is less than $100.0 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is less than $700.0 million measured on the last business day of our second fiscal quarter.

If we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired.

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 will require that we incur substantial additional professional fees and internal costs to expand our accounting and finance functions and that we expend significant management efforts. Prior to our initial public offering, we have never been required to test our internal controls within a specified period, and, as a result, we may experience difficulty in meeting these reporting requirements in a timely manner.

We may discover weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our consolidated financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. 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 are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. 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 other regulatory authorities.

We have incurred and will continue to incur significant increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company, we have incurred and will continue to incur significant legal, accounting and other expenses that we did not incur as a private company. 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, or 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. Recent legislation permits emerging growth companies to implement many of these requirements over a longer period. We intend to take advantage of this new legislation, but we may be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

We expect the rules and regulations applicable to 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 products or services. For example, we expect these rules and regulations 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 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 2. Properties.

We lease 11,200 square feet of space for our headquarters in Foster City, California under an agreement that expires in March 2025. We also lease approximately 1,400 square feet of space for an office in Basel, Switzerland under an agreement that expires in May 2024. We believe that our existing facilities are adequate to meet our current needs, and that suitable additional alternative spaces will be available in the future on commercially reasonable terms.

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 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.

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 March 4, 2022 there were 31,730,420 shares of our common stock outstanding held by approximately 10 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. Further, the RIPA limits our ability to declare dividends, including to whom they can be paid and the aggregate amount payable. 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 (this "Annual Report") for information about our equity compensation plans which is incorporated by reference herein.

Stock Performance Graph

Not applicable.

Use of Proceeds

Not applicable.

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 focused on the identification, acquisition, development and commercialization of novel therapies for debilitating rare and orphan diseases. We focus on diseases for which the unmet medical need is high and the biology for treatment is clear.

Our lead product LIVMARLI (maralixibat) oral solution ("Livmarli"), a novel, oral, minimally-absorbed agent designed to selectively inhibit ASBT, also known as the IBAT, is approved for the treatment of cholestatic pruritus in patients with ALGS 1 year of age and older in the United States. We market and commercialize Livmarli in the United States through our specialized and focused commercial team. We have also filed for approval in Europe of Livmarli for the treatment of cholestatic liver disease in patients with ALGS and plan to commercialize Livmarli in Western Europe with our international team based in Switzerland. We have entered license and distribution agreements with several rare disease companies for the commercialization of Livmarli in additional countries. We are also developing Livmarli for PFIC and BA. Livmarli has been granted breakthrough designation for ALGS and PFIC type 2.

We are advancing our second 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 ICP, 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 ASBT and bile acid markers such as 7αC4, fecal bile acids and cholesterol, demonstrating potent biological activity.

We were incorporated in May 2018 and commenced operations in November 2018. 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 Livmarli, and conducting business development activities relating to, among other things, portfolio expansion through collaborations.

We have a limited operating history and incurred significant operating losses since our inception and expect to continue to incur significant operating losses for the foreseeable future. We have only one product approved for commercial sale and have not generated significant revenues from product sales as of December 31, 2021. Since inception, we have funded our operations to date primarily through debt, equity and revenue interest financings.

Financing Transactions

In January 2020, we completed a follow-on public offering of our common stock pursuant to which we sold an aggregate of 2,400,000 shares of common stock at a public offering price of $20.00 per share, resulting in net proceeds of $44.7 million after deducting underwriting discounts, commissions and offering expenses payable by us.

In August 2020, the Securities and Exchange Commission ("SEC") declared effective a registration statement on Form S-3 ("Shelf Registration") covering the sale of up to $300.0 million of our securities. Also, in August 2020, we entered into a sales agreement ("Sales Agreement") with SVB Leerink LLC ("SVB Leerink") 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 $75.0 million under the Shelf Registration through SVB Leerink acting as the sales agent and/or principal. During the year ended December 31, 2020, we sold 305,969 shares of common stock in at-the-market offerings pursuant to the Sales Agreement at a weighted-average price of $21.55 per share, resulting in gross proceeds of $6.6 million. The net proceeds after deducting sales commissions to SVB Leerink and other issuance expenses were

approximately $6.3 million. The remaining capacity under the Sales Agreement is approximately $68.4 million as of December 31, 2021. In January and February 2022, we sold 992,389 shares of common stock in at-the-market offerings pursuant to the Sales Agreement at a weighted-average price of $18.04 per share, resulting in net proceeds of $17.4 million.

In December 2020, we completed an underwritten public offering of our common stock pursuant to the Shelf Registration. We sold 3,750,000 shares of common stock at a price of $20.00 per share, resulting in net proceeds of $70.0 million after deducting underwriting discounts, commissions and offering expenses. In addition, we granted the underwriters an option, exercisable for 30 days, to purchase up to 562,500 additional shares of our common stock at the public offering price, less the underwriting discounts and commissions. In January 2021, the underwriters exercised their option for 375,654 shares of our common stock, resulting in net proceeds of $7.1 million after deducting underwriting discounts.

In December 2020, we entered into a Revenue Interest Purchase Agreement, as amended in September 2021 ("RIPA"), with Mulholland SA LLC, an affiliate of Oberland Capital LLC ("Oberland"), as agent for purchasers party thereto (the "Purchasers"), and the Purchasers named therein, pursuant to which the Purchasers paid us $50.0 million on closing, less certain issuance costs. In April 2021, upon acceptance for filing by the FDA of our NDA for the treatment of cholestatic pruritus in patients with ALGS, we received an additional $65.0 million, less certain transaction costs, from the Purchasers. We may also be entitled to up to an additional $50.0 million at the option of the Purchasers to finance in-license or other acquisitions ("Purchaser Payments"). We were entitled to receive an additional $35.0 million upon FDA approval of Livmarli, which we elected to forgo. As consideration for the Purchaser Payments, the Purchasers have the right to receive the Revenue Interests from us based on the net sales of Livmarli, which will be tiered payments ranging from 2.00% to 9.75% of our net sales in the covered territory. The initial Revenue Interest rate of 9.75% will decrease upon certain revenue achievements. The Purchasers' rights to receive such payments shall terminate on the date on which the Purchasers have received payments totaling 195.0% of the total payments made by the Purchasers to us, exclusive of transaction expenses, unless the RIPA is terminated earlier.

Concurrently with our entry into the RIPA, we entered into a Common Stock Purchase Agreement (the "Stock Purchase Agreement") with TPC Investments II LP, TPC Investments Solutions LP and TPC Investments Solutions Co-Invest LP, each of which is an affiliate of Oberland. Pursuant to the Stock Purchase Agreement, we issued an aggregate of 509,164 shares of our common stock at a price per share of $19.64, resulting in net proceeds to us of $10.0 million.

In November 2021, we entered into a definitive agreement to sell the PRV that we received from the FDA in connection with the approval of Livmarli for the treatment of cholestatic pruritus in patients with ALGS one year of age and older, for cash proceeds of $110.0 million. In December 2021, we completed our sale of the PRV and received net proceeds of $108.0 million, after deducting commission costs.

Financial Overview

Our net loss was $84.0 million and $103.3 million for the years ended December 31, 2021 and 2020, respectively. As of December 31, 2021, we had an accumulated deficit of $257.2 million and cash, cash equivalents, restricted cash equivalents and investments of $261.5 million, of which $100.0 million is restricted from use under terms of the RIPA.

We expect our expenses and operating losses will increase substantially as we conduct our ongoing and planned clinical trials, continue our research and development activities, continue commercial activities for Livmarli, and seek regulatory approvals for our product candidates, as well as hire additional personnel and protect our intellectual property. In addition, as our product candidates progress through development and toward commercialization, we will need to make milestone payments to the licensors and other third parties from whom we have in-licensed or acquired our product candidates. We have also entered into collaboration arrangements with other companies whereby we are entitled to receive upfront and license fees, research and development funding, development and sales-based milestones, and tiered royalties based on sales of commercialized products. Arrangements that include upfront payments may require deferral of revenue recognition to a future period until we perform obligations under these arrangements. The event-based milestone and other contingent payments represent

variable consideration, and we use the most likely amount method or expected value method to estimate this variable consideration, depending on the nature of the contingency and the variable payments. Given the high degree of uncertainty around the occurrence of these events, we generally determine the milestone and other contingent amounts to be fully constrained until the uncertainty associated with these payments is resolved. We will recognize revenue from sales-based royalty payments when or as the sales occur. We will re-evaluate the transaction price in each reporting period as uncertain events are resolved and other changes in circumstances occur. The timing of these activities may fluctuate significantly. As a result, our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending in particular on the timing of our clinical trials and non-clinical studies and our expenditures on other research and development activities.

We expect our revenue from product sales, net will increase in the future from the launch of Livmarli. However, the timing and amount of product sales, net are unknown. Accordingly, until such time as we can generate substantial product revenues to support our cost structure and sustain operating activities, if ever, we expect to finance our cash needs through 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.

Prior to the regulatory approval of Livmarli, the manufacturing and related costs were expensed as research and development as any future economic benefit was not considered probable; accordingly, these costs were not capitalized and as a result gross margins resulting from product sales, net will initially be higher until we deplete inventories that we had expensed prior to receiving approval.

COVID-19

The coronavirus (including its variants, "COVID-19") pandemic has had a significant economic impact across the global marketplace presenting challenges to maintaining business continuity and dramatically changing the ways in which we live and interact with others. Although the initial impact of the pandemic has subsided, we are uncertain as to how more transmissible variants may impact our business. We are working diligently to ensure the advancement of all of our clinical development programs in the safest manner possible. While we are unable to reliably estimate the duration or extent of any potential business disruption or financial impact during this time, we remain committed to (i) prioritizing the safety, health and well-being of patients, their caregivers, healthcare providers and our employees; (ii) ensuring patients are well supported and have continued uninterrupted access to our product candidates, for which we currently do not expect any supply disruption; and (iii) advancing our clinical trials. Examples include a "Work from Home Policy" for our employees and access to home health care to assist families with safer participation in our trials.

Although we did not see a significant financial impact to our business operations as a result of COVID-19 for the year ended December 31, 2021, there may be potential impacts to our business in the future that are highly uncertain and difficult to predict such as temporary closures of our offices or those of our third-party manufacturers or suppliers, disruptions or restrictions on our employees' ability to travel, disruptions to or delays in ongoing non-clinical trials, clinical trials, third-party manufacturing supply and other operations, inability for patients to see their healthcare providers and access our products, the potential diversion of healthcare resources away from the conduct of clinical trials to focus on pandemic concerns, interruptions or delays in the operations of the FDA or other regulatory authorities, and our ability to raise capital and conduct business development activities. The ultimate impact of the COVID-19 pandemic, including any lasting effects on our revenue and the way we conduct our business, is highly uncertain and subject to continued change. We recognize that this pandemic may continue to present unique challenges for us throughout 2022.

We continue to believe that existing cash equivalents and investments, excluding restricted cash equivalents, and existing sources of and access to financing are adequate to satisfy our needs for working capital, capital expenditures, debt service requirements and other business development initiatives that we plan to strategically

pursue in the 12 months from the issuance of the consolidated financial statements included in this Annual Report. However, should the COVID-19 pandemic and any associated recession or depression continue for a prolonged period, our results of operations, financial condition, liquidity and cash flows could be materially impacted as a result of a lower likelihood of effectively and efficiently developing new medicines and successfully commercializing our products.

License Agreements

Assignment and License Agreement with Shire (Takeda)

In November 2018, we entered into the Shire License Agreement with Shire, which was subsequently acquired by Takeda Pharmaceutical Company Limited ("Takeda"), in which we were granted an exclusive, royalty bearing worldwide license to develop and commercialize our two product candidates, Livmarli and volixibat. As part of the Shire License Agreement, we were assigned license agreements held by Shire with Satiogen Pharmaceuticals, Inc. ("Satiogen" and altogether, the "Satiogen License"), Pfizer Inc., and Sanofi, collectively the Assigned License Agreements ("Assigned License Agreements"). In partial consideration for the rights granted to us under the Shire License Agreement, we made an upfront payment to Shire of $7.5 million and issued Shire 1,859,151 shares of our common stock with an estimated fair value of $7.0 million.

Under the Shire License Agreement and Assigned License Agreements, to date, we have paid aggregate development and regulatory milestones of $51.0 million related to our Livmarli and volixibat programs.

Licensing Agreement with Takeda

In September 2021, we entered into an exclusive licensing agreement with Takeda for the development and commercialization of Livmarli in Japan for ALGS, PFIC and BA. Under the terms of the agreement, Takeda will be responsible for regulatory approval and commercialization of Livmarli in Japan. Takeda will also be responsible for development, including conducting clinical studies in cholestatic indications. We are responsible for commercial supply to Takeda. In exchange, we are eligible to receive a percentage of Takeda's annualized 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.

Components of Results of Operations

Revenue

Product Sales, Net

To date, we have not generated significant revenue from product sales. Our approved product, Livmarli, was approved by the FDA in September 2021 for the treatment of cholestatic pruritus in patients with ALGS one year of age and older. We recorded our first product sales of Livmarli in October 2021. We expect our product sales of Livmarli will increase as a result of our continued commercial activities. However, as it is early in our product launch, we do not yet have a trend and while we believe revenue will increase as we further engage with health care providers in the United States and globally, we cannot predict revenues with any certainty.

Our revenue from product sales, net further depends on our prescription mix of U.S. commercial payors, Medicaid and free drugs under our patient assistance program. Our experience to date in our recent product launch is not sufficient to allow us to reliably estimate the payor mix and resulting gross to net adjustments.

License Revenue

Under the exclusive licensing agreements with CANbridge and GC Pharma, we have recognized as revenue the upfront nonrefundable payments related to the licenses granted upon satisfaction of certain performance obligations. Pursuant to the agreements, we are eligible to receive future milestone payments. These milestone payments were fully constrained and not recognized currently in revenue due to the degree of uncertainty in achieving the milestones associated with these payments. We are also eligible to receive royalty payments related to the agreements, which will be recognized as the underlying product sales occur.

Cost of Product Sales

Cost of product sales consist of third party manufacturing costs, personnel, facility and other costs of manufacturing commercial products, transportation and freight, amortization of capitalized intangibles associated

with contractual milestone payments paid to licensors upon certain regulatory approval and sales-based events and royalty payments payable on net sales of Livmarli under licensing agreements. Cost of product sales may also include period costs related to certain manufacturing services and inventory adjustment charges. Prior to receiving approval from the FDA in September 2021 to market and sell Livmarli in the United States, we expensed all costs incurred related to the manufacture of Livmarli 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 as a company of regulatory approval of drug candidates. Our inventory of Livmarli produced prior to FDA approval is available for commercial or clinical use.

Operating Expenses

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 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;
- research and development funding for collaboration arrangements;
- 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 anticipate that our research and development expenses will increase in the future as we continue the clinical and development activity for further indications of Livmarli, as well as expand the clinical and development activity associated with our volixibat pipeline. These increases will likely include increased costs related to hiring of additional development personnel and fees to outside clinical development organizations that support clinical trial activity.

Selling, General and Administrative Expense

Selling expenses consist of professional fees related to preparation and commercialization of Livmarli, finished goods warehousing costs, as well as salaries and related employee benefits for commercial employees, including stock-based compensation.

General and administrative expenses consist primarily of salaries and employee-related costs, including stock-based compensation, for personnel in executive, finance and other administrative functions. Other significant costs include facility-related costs, legal fees relating to intellectual property and corporate matters, professional fees for accounting and consulting services and insurance costs.

We anticipate that our selling, general and administrative expenses will increase in the future to support our continued commercialization efforts of Livmarli in the United States and internationally as well as increased costs of operating as a global commercial stage biopharmaceutical public company. These increases will likely include

increased costs related to hiring of additional personnel and fees to outside consultants to support further marketing, legal and accounting activities.

Interest Income

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

Interest Expense

Interest expense for the year ended December 31, 2021 was related to the RIPA. Costs during the year consist primarily of costs associated with our liability and non-cash interest costs associated with the amortization of the related debt discount and deferred issuance costs. We impute interest expense associated with this liability using the effective interest rate method which is calculated based on the rate that would enable the debt to be repaid in full over the anticipated life of the arrangement.

Change in Fair Value of Derivative Liability

Change in fair value of derivative liability consists of the gain or loss from remeasurement of the compound derivative liability related to the revenue interest liability during the period.

Gain from Sale of Priority Review Voucher, Net

Gain from the sale of priority review voucher represents the sale of the PRV that was granted by the FDA in September 2021 with the approval of Livmarli for the treatment of cholestatic pruritus in patients with ALGS one year of age and older.

Other Income (Expense), Net

Other income (expense), net consists of transactional currency exchange gain or loss.

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.

Product Sales, Net

Revenues from 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. The transaction price, which may include fixed or variable consideration reflecting the impact of discounts and allowances, 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. Estimates are reviewed and updated quarterly as additional information 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.

Government Rebates. We are obligated to pay rebates for mandated discounts under the Medicaid Drug Rebate Program. Our 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. 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.

Revenue Interest Liability, Net

We impute interest expense associated with this liability using the effective interest rate method. The effective interest rate is calculated based on the rate that would enable the debt to be repaid in full over the anticipated life of the arrangement. The effective interest rate on the liability may vary during the term of the agreement depending on a number of factors, including the level of actual and forecasted product sales, net. We evaluate the interest rate quarterly based on actual product sales, net and on our current product sales, net forecasts utilizing the prospective method. A significant increase or decrease in product sales, net will materially impact the revenue interest liability, interest expense and the time period for repayment.

At December 31, 2021, the revenue interest liability is calculated using our current estimate of global forecasted net sales of Livmarli and impacted by a debt discount comprising the estimated value of a bifurcated derivative liability and issuance costs incurred.

The fair value of the derivative liability is valued using a "with-and-without" method. The "with-and-without" methodology involves valuing the whole instrument on an as-is basis and then valuing the instrument without the individual embedded derivative. The difference between the entire instrument with the embedded derivative compared to the instrument without the embedded derivative was the fair value of the derivative liability. The estimated probability and timing of underlying events triggering the exercisability of the derivative liability bifurcated from within the RIPA, forecasted cash flows and the discount rate are significant unobservable inputs used to determine the estimated fair value of the entire instrument with the embedded derivative.

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.

Uncertain Tax Positions

We are subject to income taxes in the United States and certain foreign jurisdictions. The evaluation of uncertain tax positions involves significant judgment in the interpretation and application of GAAP and complex tax laws. Although management believes our reserves are reasonable, no assurance can be given that the final tax outcome of these matters will not be different from that which is reflected in our reserves. Reserves are adjusted considering changing facts and circumstances, such as the closing of a tax examination or the refinement of an estimate. Resolution of these uncertainties in a manner inconsistent with management's expectations could have a material impact on our financial condition and operating results.

Legal and Other Contingencies

We are subject to various legal proceedings and claims that arise in the ordinary course of business, the outcomes of which are inherently uncertain. We record a liability when it is probable that a loss has been incurred and the amount can be reasonably estimated, the determination of which requires significant judgment. Resolution of legal matters in a manner inconsistent with management's expectations could have a material impact on our financial condition and operating results.

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, 2021 and 2020

The following table summarizes our results of operations for the years ended December 31, 2021 and 2020 (in thousands):

	Year Ended December 31,		
	2021	2020	Change
Revenue:			
Product sales, net	$ 3,138	$ —	$ 3,138
License revenue	16,000	—	$ 16,000
Total revenue	19,138	—	19,138
Operating expenses:			
Cost of sales	$ 1,903	$ —	$ 1,903
Research and development	131,428	81,605	49,823
General and administrative	59,220	22,691	36,529
Total operating expenses	192,551	104,296	88,255
Loss from operations	(173,413)	(104,296)	(69,117)
Other income (expense):			
Interest income	366	1,559	(1,193)
Interest expense	(17,590)	(335)	(17,255)
Change in fair value of derivative liability	(732)	—	(732)
Other expense, net	(582)	(192)	(390)
Gain from sale of priority review voucher, net	108,000	—	108,000
Net loss before provision for income taxes	(83,951)	(103,264)	(88,687)
Provision for income taxes	37	6	31
Net Loss	$ (83,988)	$ (103,270)	$ (88,718)

Product Sales, Net

Product sales, net was $3.1 million for the year ended December 31, 2021, compared to zero for the year ended December 31, 2020 as a result of our commercial launch of Livmarli in the United States in September 2021.

License Revenue

License revenue was $16.0 million for the year ended December 31, 2021, compared to zero for the year ended December 31, 2020. The increase in license revenue was due to the satisfaction of certain performance obligations associated with the licenses granted under the license agreements with CANbridge and GC Pharma.

Cost of Sales

For the year ended December 31, 2021, cost of sales was $1.9 million, compared to zero for the year ended December 31, 2020, and primarily consisted of amortization of capitalized intangible assets, royalties payable on net sales of Livmarli under licensing agreements and indirect overhead costs associated with the manufacturing and distribution of Livmarli. There were minimal inventory costs reflected in cost of sales as inventory related to current product sold were expensed as research and development costs prior to Livmarli approval in September 2021.

Research and Development Expenses

The following tables summarize 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,		
	2021	2020	Change
Product-specific costs:			
Livmarli	$ 35,525	$ 42,413	$ (6,888)
Volixibat	15,691	7,531	8,160
Non product-specific costs:			
Collaboration funding	18,855	—	18,855
Stock-based compensation	9,888	5,129	4,759
Personnel	21,999	13,272	8,727
License fees (milestone payments)	23,161	10,000	13,161
Other	6,309	3,260	3,049
Total research and development expenses	$ 131,428	$ 81,605	$ 49,823

Research and development expenses were $131.4 million for the year ended December 31, 2021, an increase of $49.8 million compared to the year ended December 31, 2020. The increase was primarily due to:

•for Livmarli programs, a decrease of $6.9 million, primarily due to a decrease of $8.2 million manufacturing activities as a result of prior year completion of NDA registrational production offset by a net increase of $1.1 million clinical trial expenses due to increases for the Phase 2b EMBARK clinical trial in BA, the Phase 3 MARCH clinical trial in PFIC, an infant safety study and an expanded access program, which were partially offset by $2.2 million in research and development funding pursuant to our license agreements with CANbridge and GC Pharma which was recorded as a reduction of clinical trial expenses, and $0.1 million of regulatory fees and other general development expenses;

•for volixibat programs, an increase of $8.2 million, primarily due to an increase of $3.2 million in clinical trial expenses for PSC, PBC and ICP, $4.4 million related manufacturing activities supporting clinical supply, and $0.6 million in other general development expenses;

•for collaboration funding, an increase of $18.9 million, due to Vivet Collaboration Agreement program development funding. We terminated this agreement in December 2021 and we do not expect to incur additional funding;

•for stock-based compensation, an increase of $4.8 million, related to an increase in employee headcount as well as expense associated with performance-based restricted stock units that vested upon achievement of FDA approval of Livmarli;

•for personnel related expenses, an increase of $8.7 million, related to an increase in employee headcount to support our development pipeline;

•for license fees, an increase of $13.2 million, related to calendar year 2021 developmental milestone payments of $19.0 million consisting of $15.0 million associated with the acceptance of our NDA for

filing for Livmarli for the treatment of cholestatic pruritus in patients with ALGS one year of age and older, $4.0 million associated with the initiation of the Phase 2b VISTAS clinical trial in PSC and the Phase 2b EMBARK clinical trial in BA and a $4.2 million upfront fee in connection with our Vivet Collaboration Agreement compared to $10.0 million in calendar year 2020 upon acceptance of an MAA filing to the EMA for Livmarli for the treatment of PFIC2; and

•for other general expense, an increase of $3.0 million, primarily due to outside consulting expenses.

Selling, General and Administrative Expenses

Selling, general and administrative expenses were $59.2 million for the year ended December 31, 2021, an increase of $36.5 million compared to the year ended December 31, 2020. The increase was primarily due to an increase of $14.1 million in professional and consulting service expenses associated with commercial launch activities for Livmarli, $17.1 million of personnel and other compensation related expenses, including an increase of $5.7 million in stock-based compensation, reflecting an increase in the number of our administrative employees to support commercial launch activities for Livmarli and increased requirements of operating as a public company, such as regulatory compliance, an increase of $2.5 million in expenses primarily related to general legal and public relations activities, an increase of $1.5 million in consulting and professional services general corporate compliance and support, and an increase of $1.4 million in other general administrative expenses.

Interest Income

Interest income was $0.4 million for the year ended December 31, 2021, a decrease of $1.2 million compared to the year ended December 31, 2020. The decrease was primarily due to lower interest earned on our cash equivalents and investment balances compared to the prior year largely a result of current economic conditions.

Interest Expense

Interest expense was $17.6 million for the year ended December 31, 2021, an increase of $17.3 million compared to the year ended December 31, 2020. The increase reflected a full year of accreted interest recognized on the revenue interest liability in connection with the RIPA.

Change in Fair Value of Derivative Liability

Change in fair value of derivative liability was $0.7 million for the year ended December 31, 2021 compared to zero for the year ended December 31, 2020. The change was related to the remeasurement of the derivative liability at December 31, 2021 and reflects an increase in value of the put option associated with the RIPA primarily resulting from changes in forecast product sales, net.

Gain from Sale of Priority Review Voucher, Net

Gain from sale of the PRV, net of transaction costs, totaled $108.0 million for the year ended December 31, 2021. We did not sell a PRV in the year ended December 31, 2020.

Liquidity and Capital Resources

Overview

To date, we have funded our operations primarily from the sale of our equity securities, revenue interest financings and collaboration arrangements and to a lesser extent from product revenue from the sales of Livmarli.

We had $261.5 million of cash, cash equivalents, restricted cash equivalents and investments as of December 31, 2021, inclusive of $100.0 million restricted cash equivalents, compared to $231.8 million as of December 31, 2020. Since inception, we have incurred operating losses and negative cash flows from operations. As of December 31, 2021, we had an accumulated deficit of $257.2 million.

In January 2020, we completed a follow-on public offering of our common stock pursuant to which we sold an aggregate of 2,400,000 shares of common stock at a price of $20.00 per share, resulting in net proceeds of $44.7 million after deducting underwriting discounts, commissions and offering expenses payable by us.

In August 2020, the SEC declared effective the Shelf Registration covering the sale of up to $300.0 million of our securities. Also, in August 2020, we entered into the Sales Agreement with SVB Leerink, 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 $75.0 million under the Shelf Registration through SVB Leerink acting as the sales agent and/or principal. During the year ended December 31, 2021, we did not sell any common stock pursuant to the Sales Agreement. As of December 31, 2020, we had sold 305,969 shares of common stock in an at-the-market offering pursuant to the Sales Agreement at a weighted-average price of $21.55 per share, resulting in gross proceeds of $6.6 million. The net proceeds after deducting sales commissions to SVB Leerink and other issuance expenses were approximately $6.3 million. The remaining capacity under the Sales Agreement is approximately $68.4 million as of December 31, 2021. In January and February 2022, we sold 992,389 shares of common stock in at-the-market offerings pursuant to the Sales Agreement at a weighted-average price of $18.04 per share, resulting in net proceeds of $17.4 million.

In December 2020, we completed an underwritten public offering of our common stock pursuant to the Shelf Registration. We sold 3,750,000 shares of common stock at a public offering price of $20.00 per share, resulting in net proceeds of $70.0 million after deducting underwriting discounts, commissions and offering expenses. In addition, we granted the underwriters an option, exercisable for 30 days, to purchase up to 562,500 additional shares of our common stock at the public offering price, less the underwriting discounts and commissions. In January 2021, the underwriters exercised their option for 375,654 shares of our common stock resulting in net proceeds of $7.1 million after deducting underwriting discounts.

Pursuant to the RIPA, we received $115.0 million from the Purchasers consisting of an upfront cash payment of $50.0 million and an additional $65.0 million as a result of acceptance for filing of our NDA for Livmarli. We may also be entitled to receive up to an additional $50.0 million at the option of the Purchasers to finance in-license or other acquisitions. In return, the Purchasers have a right to receive Revenue Interests based on net product sales of Livmarli. We were entitled to receive an additional $35.0 million upon FDA approval of Livmarli, which we elected to forgo.

Pursuant to the Stock Purchase Agreement, entered into concurrently with our entry into the RIPA, we issued an aggregate of 509,164 shares of our common stock at a price per share of $19.64, resulting in net proceeds to us of $10.0 million.

In December 2021, we received net cash proceeds of $108.0 million, after deducting commission costs, related to the sale of the PRV which we received from the FDA in connection with the approval of Livmarli for the treatment of cholestatic pruritus in patients with ALGS one year of age and older. Of these proceeds, $100.0 million was placed in a segregated bank account as restricted cash, per the terms of the RIPA.

Based on our current and anticipated level of operations, we believe our 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. Our unrestricted cash, cash equivalents and investments include money market funds, government agency securities, corporate debt and commercial paper. We maintain established guidelines relating to diversification and maturities of our investments to preserve principal and maintain liquidity.

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, continue commercial activities for Livmarli and potentially expand into additional markets, hire additional staff, including clinical, scientific, operational, financial and management personnel and pay potential development and commercial milestones in connection with our license agreements.

Our primary use of cash is to fund operating expenses, which consist primarily of research and development expenditures, and to a lesser extent, selling, general and administrative expenditures, including commercial expenses. Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses.

Although Livmarli has been approved by the FDA for the treatment of cholestatic pruritus in patients with ALGS one year of age and older, and we expect product revenues to increase as we continue commercial activities, Livmarli may not achieve commercial success. Our principal sources of liquidity are cash from the sale of our equity securities, revenue interest financings and collaboration arrangements and, to a lesser extent, from product revenue from the sale of Livmarli. Until such time, if ever, as we can generate substantial product revenue from sales of Livmarli, our current product candidates or any future product candidates, if approved, we expect to finance our cash needs through a combination of equity offerings, debt financings, revenue interest purchase agreements and potential collaboration, license or development agreements. Our primary cash needs are for day-to-day operations, to pay our debt obligations under the RIPA 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 the RIPA, 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. As a result of the COVID-19 pandemic and actions taken to slow its spread, the global credit and financial markets have experienced 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 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.

Contractual Obligations

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

Pursuant to the RIPA, the Purchasers have a right to receive Revenue Interests based on net product sales of Livmarli. The amounts of quarterly Revenue Interest Payments will change each reporting period based upon the underlying net product sales of Livmarli and will initially be 9.75% of net product sales. Per the terms of the agreement, every $100.0 million of net sales generated, less than or equal to $350 million in an annual aggregate, would result in a repayment obligation of approximately $9.8 million. Additionally, every $100.0 million of net sales generated in excess of $350.0 million in an annual aggregate would result in a repayment obligation of approximately $2.0 million. In the future, as net sales thresholds set forth in the agreement are met and the repayment percentage rate changes, the amount of the obligation and timing of payment is likely to change. A significant increase or decrease in actual and forecast net sales will materially impact the revenue interest liability, interest expense and the time period for repayment.

Under the Shire License Agreement, 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 the consolidated financial statements.

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 are party to certain license and collaboration agreements, which contain a number of contractual obligations. Those contractual obligations may entitle us to receive, or may obligate us to make, certain payments. The amount and timing of those payments are unknown or uncertain as we are unable to estimate the timing or likelihood of the events that will obligate those payments.

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 cancelable contracts.

Cash Flows

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

	Year Ended December 31,	
	2021	2020
Net cash used in operating activities	$ (132,758)	$ (89,075)
Net cash provided by investing activities	48,547	37,874
Net cash provided by financing activities	73,466	181,288
Effect of exchange rate on cash and cash equivalents	(1)	29
Net increase (decrease) in cash and cash equivalents	$ (10,746)	$ 130,116

Net Cash Used in Operating Activities

Net cash used in operating activities was $132.8 million for the year ended December 31, 2021, reflecting our net loss of $84.0 million partially offset by non-cash items of $42.2 million and a non-cash gain of $108.0 million from the sale of the PRV. Non-cash items consisted primarily of $23.1 million of stock-based compensation, $17.6 million of effective interest expense in connection with the RIPA, $0.7 million related to the change in fair value of the derivative liability, and $1.0 million of depreciation and amortization of our fixed assets and operating lease right-of use assets. Additionally, cash used in operating activities reflected changes in net operating assets of $17.0 million, consisting primarily of a $22.7 million increase in accounts payable, accrued expenses and other liabilities primarily associated with clinical, manufacturing and commercial planning activities and collaboration funding pursuant to the Vivet Collaboration Agreement and $5.0 million increase in prepaid expenses, inventory and other assets, partially offset by a $0.6 million decrease in our operating lease liability.

Net cash used in operating activities was $89.1 million for the year ended December 31, 2020, reflecting our net loss of $103.3 million partially offset by non-cash items of $13.6 million. Non-cash items consisted primarily of $12.6 million of stock-based compensation, $0.6 million of depreciation and amortization of our fixed assets and our operating lease right-of use assets and $0.3 million of interest expense in connection with the RIPA. Additionally, cash used in operating activities reflected changes in net operating assets of $0.6 million, consisting primarily of a $3.7 million increase in accounts payable, accrued expenses and other liabilities due to clinical and manufacturing activities, offset by a $1.8 million increase to prepaid expenses primarily associated with clinical and manufacturing activities, $1.0 million increase in long term assets primarily associated with advances to vendors associated with our clinical trials, and a $0.4 million decrease in our operating lease liability.

Net Cash Provided by Investing Activities

Net cash provided by investing activities was $48.5 million for the year ended December 31, 2021, primarily due to $108.0 million of net proceeds received from the sale of the PRV, proceeds of $155.6 million from maturities of investments, and proceeds of $2.0 million from paydowns of investments partially offset by $198.0 million used in purchases of investments and $19.0 million in milestone payments triggered under license agreements.

Net cash provided by investing activities was $37.9 million for the year ended December 31, 2020 primarily due to proceeds of $85.9 million from maturities of investments and proceeds of $26.8 million from paydowns of investments, partially offset by $74.6 million used in purchases of investments and $0.2 million used for purchases of property and equipment.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $73.5 million for the year ended December 31, 2021, due to net proceeds of $64.6 million pursuant to the RIPA, net proceeds of $6.9 million received from the underwriters when they exercised their option to purchase 375,654 shares of our common stock in January 2021 following the follow-on underwritten public offering of our common stock in December 2020, and proceeds of $2.1 million from

employee equity award exercises, partially offset by $0.1 million of revenue interest payments made under the RIPA.

Net cash provided by financing activities was $181.3 million for the year ended December 31, 2020, due to net proceeds of $44.7 million received from the completion of a follow-on public offering of our common stock pursuant to which we sold an aggregate of 2,400,000 shares of common stock at a price of $20.00 per share, net proceeds of $70.0 million received from completion of a public offering of our common stock pursuant to which we sold an aggregate of 3,750,000 shares of common stock at $20.00 per share, net proceeds of $49.6 million pursuant to the RIPA, net proceeds of $10.0 million pursuant to the Stock Purchase Agreement entered into in connection with the RIPA for the sale of an aggregate of 509,164 shares of our common stock at a price per share of $19.64, net proceeds of $6.3 million from the sale of common stock under the Sales Agreement with SVB Leerink, pursuant to which we sold an aggregate of 305,969 shares of common stock at a weighted-average price of $21.55 per share, and proceeds of $0.8 million from the issuance of common stock pursuant to the exercise of outstanding options pursuant to our equity incentive plans. These proceeds were offset by the payment of $0.2 million for deferred offering costs associated with the Shelf Registration. The deferred offering costs are reflected as other assets on our consolidated balance sheet until such time as we complete sales of shares under the Shelf Registration.

JOBS Act

As an emerging growth company under the Jumpstart Our Business Startups Act of 2012 ("JOBS Act"), we can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies. We intend to rely on other exemptions provided by the JOBS Act, including without limitation, not being required to comply with the auditor attestation requirements of Section 404(b) of the Sarbanes-Oxley Act of 2002, as amended ("Sarbanes-Oxley Act").

We will remain an emerging growth company until the earliest of (i) December 31, 2024, (ii) the last day of the fiscal year in which we have total annual gross revenue of at least $1.07 billion, (iii) the last day of the fiscal year in which we are deemed to be a "large accelerated filer" as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended ("Exchange Act"), or (iv) the date on which we have issued more than $1.0 billion in non-convertible debt securities during the prior three-year period.

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

Interest Rate Risk

Our cash, cash equivalents, restricted cash equivalents and investments as of December 31, 2021 consist of readily available checking, money market funds, and investments. The primary objective of our investment activities is to preserve our capital to fund operations. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. However, due to the short-term nature of the instruments in our portfolio and the low risk profile of our investments, a hypothetical change in interest rates of 100 basis points would not have a material impact on the fair market value of our cash equivalents, restricted cash equivalents and investments as of December 31, 2021. 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 entered into the RIPA. Our primary exposure to interest rate risk is that the effective interest rate on the liability may vary during the term of the RIPA primarily due to the level of actual forecast net product sales. Due to the nature of the RIPA instrument, the total interest due under this facility is fixed. However, a significant increase or decrease in net product sales may materially impact the interest expense recognized each reporting period.

Foreign Currency Rate Risk

Our operations include activities in the United States and Switzerland. In addition, we contract with vendors that are located outside of the United States and certain invoices are denominated in foreign currencies. While our operating results are exposed to changes in foreign currency exchange rates between the U.S. dollar and various

foreign currencies, the most significant of which are the Swiss Franc and the Euro, we do not currently believe that foreign currency could have a significant impact.

Effects of Inflation

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation and changing prices had a significant impact on our results of operations for any periods presented herein.

Item 8. Financial Statements and Supplementary Data.

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

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, 2021 and 2020, the related consolidated statements of operations, comprehensive loss, stockholders' equity and cash flows for each of the two years in the period ended December 31, 2021, 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, 2021 and 2020, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2021, in conformity with U.S. generally accepted accounting principles.

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 Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our 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. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

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.

/s/ Ernst & Young LLP

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

Irvine, California
March 9, 2022

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

	December 31, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 31,340	$ 142,086
Short-term investments	125,201	89,734
Accounts receivable	3,267	—
Inventory	1,513	—
Prepaid expenses and other current assets	5,271	4,530
Total current assets	166,592	236,350
Restricted cash equivalents	100,000	—
Long-term investments	4,983	—
Property and equipment, net	981	1,293
Operating lease right-of-use assets	1,569	1,949
Intangible assets, net	18,740	—
Other assets	1,786	1,272
Total assets	$ 294,651	$ 240,864
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 9,166	$ 3,151
Accrued expenses	30,723	13,411
Operating lease liabilities	711	636
Derivative liability	1,996	1,264
Total current liabilities	42,596	18,462
Revenue interest liability, net	129,923	47,651
Operating lease liabilities, noncurrent	1,903	2,627
Other liabilities	17	29
Total liabilities	174,439	68,769
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, $0.0001 par value; 10,000,000 shares authorized as of December 31, 2021 and 2020, respectively; no shares issued and outstanding as of December 31, 2021 and 2020, respectively; and liquidation value of zero as of December 31, 2021 and 2020, respectively	—	—
Common stock, $0.0001 par value; 200,000,000 shares authorized; 30,705,060 shares issued and 30,582,596 shares outstanding, excluding 122,464 shares subject to repurchase as of December 31, 2021; and 30,032,600 shares issued and 29,776,544 shares outstanding, excluding 256,056 shares subject to repurchase as of December 31, 2020	3	3
Additional paid-in capital	377,403	345,180
Accumulated deficit	(257,159)	(173,171)
Accumulated other comprehensive (loss) income	(35)	83
Total stockholders' equity	120,212	172,095
Total liabilities and stockholders' equity	$ 294,651	$ 240,864

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, 2021	2020
Revenue:		
Product sales, net	$ 3,138	$ —
License revenue	16,000	—
Total revenue	19,138	—
Operating expenses:		
Cost of sales	1,903	—
Research and development	131,428	81,605
Selling, general and administrative	59,220	22,691
Total operating expenses	192,551	104,296
Loss from operations	(173,413)	(104,296)
Other income (expense):		
Interest income	366	1,559
Interest expense	(17,590)	(335)
Change in fair value of derivative liability	(732)	—
Other expense, net	(582)	(192)
Gain from sale of priority review voucher, net	108,000	—
Net loss before provision for income taxes	(83,951)	(103,264)
Provision for income taxes	37	6
Net loss	$ (83,988)	$ (103,270)
Net loss per share, basic and diluted	$ (2.77)	$ (4.09)
Weighted-average shares of common stock outstanding, basic and diluted	30,321,722	25,251,968

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,	
	2021	2020
Net loss	$ (83,988)	$ (103,270)
Other comprehensive gain (loss):		
Unrealized loss on available-for-sale investments	(117)	(75)
Cumulative translation adjustments	(1)	29
Comprehensive loss	$ (84,106)	$ (103,316)

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)

	Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Deficit	Accumulated Other Comprehensive Income (Loss)	Total Stockholders' Equity
	Shares	Amount	Shares	Amount				
Balance as of December 31, 2019	—	$ —	22,600,338	$ 2	$ 200,119	$ (69,901)	$ 129	$ 130,349
Issuance of common stock in underwritten public offerings, net of issuance costs of $8,322	—	—	6,150,000	1	114,678	—	—	114,679
Issuance of common stock in connection with Revenue Interest Purchase Agreement	—	—	509,164	—	10,766	—	—	10,766
Issuance of common stock in at-the-market offerings, net of issuance costs of $314	—	—	305,969	—	6,279	—	—	6,279
Issuance of common stock in connection with common stock option exercises			47,604	—	328	—	—	328
Issuance of common stock in connection with Employee Stock Purchase Plan	—	—	29,876	—	456	—	—	456
Restricted common stock vested in the period	—	—	133,593	—	—	—	—	—
Stock-based compensation	—	—	—	—	12,554	—	—	12,554
Net loss	—	—	—	—	—	(103,270)	—	(103,270)
Other comprehensive loss	—	—	—	—	—	—	(46)	(46)
Balance as of December 31, 2020	—	$ —	29,776,544	$ 3	$ 345,180	$ (173,171)	$ 83	$ 172,095
Issuance of common stock in connection with equity award plans	—	—	207,595	—	844	—	—	844
Issuance of common stock in connection with public offering, net of issuance costs $476	—	—	375,654	—	7,038	—	—	7,038
Restricted common stock vested in the period	—	—	133,592	—	—	—	—	—
Issuance of common stock in connection with Employee Stock Purchase Plan	—	—	89,211	—	1,254	—	—	1,254
Stock-based compensation	—	—	—	—	23,087	—	—	23,087
Net loss	—	—	—	—	—	(83,988)	—	(83,988)
Other comprehensive loss	—	—	—	—	—	—	(118)	(118)
Balance as of December 31, 2021	—	$ —	30,582,596	$ 3	$ 377,403	$ (257,159)	$ (35)	$ 120,212

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,	
	2021	**2020**
Operating activities		
Net loss	$ (83,988)	$ (103,270)
Reconciliation of net loss to net cash used in operating activities:		
Stock-based compensation	23,087	12,554
Depreciation and amortization	595	305
Amortization of operating lease right-of-use assets	380	318
Net (accretion) amortization of discounts on investments	(137)	72
Non-cash interest expense related to the revenue interest liability	17,590	335
Change in fair value of derivative liability	732	—
Gain on sale of priority review voucher, net	(108,000)	—
Change in operating assets and liabilities:		
Prepaid and other current assets	(4,009)	(1,827)
Operating lease right-of-use assets	—	94
Inventory	(495)	—
Other assets	(519)	(954)
Accounts payable, accrued expenses and other liabilities	22,655	3,683
Operating lease liabilities	(649)	(385)
Net cash used in operating activities	(132,758)	(89,075)
Investing activities		
Proceeds from maturities of investments	155,600	85,900
Proceeds from paydown of investments	2,000	26,761
Purchase of investments	(198,029)	(74,562)
Purchase of property and equipment	(24)	(225)
Proceeds from sale of priority review voucher, net	108,000	—
Additions to intangible assets	(19,000)	—
Net cash provided by investing activities	48,547	37,874
Financing activities		
Proceeds from issuance of common stock in public offerings, net of issuance costs	6,914	121,087
Proceeds from issuance of common stock in private placement, net of issuance costs	—	10,000
Proceeds from issuance of common stock pursuant to equity award plans	2,098	784
Proceeds from revenue interest liability, net of issuance costs	64,575	49,575
Payments on revenue interest liability	(121)	—
Payment of deferred offering costs in connection with the shelf registration and sales agreement	—	(158)
Net cash provided by financing activities	73,466	181,288
Effect of exchange rate on cash, cash equivalents and restricted cash equivalents	(1)	29
Net (decrease) increase in cash, cash equivalents and restricted cash equivalents	(10,746)	130,116
Cash, cash equivalents and restricted cash equivalents at beginning of period	142,086	11,970
Cash, cash equivalents and restricted cash equivalents at end of period	$ 131,340	$ 142,086
Supplemental disclosure of cash flow information:		
Inventory purchases in accrued liabilities	$ 1,018	$ —
Cash paid for income taxes	$ —	$ 11
Operating cash flows paid for operating lease	$ 864	$ 576
Noncash investing and financing activities:		
Revenue interest liability issuance costs included in accrued liabilities	$ —	$ 229
Deferred offering costs included in accrued liabilities	$ —	$ 129
Compound derivative liability related to revenue interest liability	$ —	$ 1,264

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 focused on the identification, acquisition, development and commercialization of novel therapies for debilitating rare diseases.

The Company received U.S. Food and Drug Administration ("FDA") approval for LIVMARLI® (maralixibat) oral solution ("Livmarli"), the first and only FDA-approved medication for the treatment of cholestatic pruritus in patients with Alagille syndrome ("ALGS") one year of age and older, on September 29, 2021.

The Company's development pipeline consists of two clinical-stage product candidates, Livmarli and volixibat. The Company commenced significant operations in November 2018.

The Company views its operations and manages its business as one operating segment.

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, 2021, the Company had an accumulated deficit of $257.2 million and cash, cash equivalents, restricted cash equivalents and investments of $261.5 million. The Company believes that its unrestricted cash, cash equivalents and investments of $161.5 million as of December 31, 2021, provide sufficient capital resources to continue its operations for at least twelve months from the issuance date of the accompanying consolidated financial statements. The consolidated financial statements have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business. The consolidated financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of this uncertainty. Management expects to continue to incur additional substantial losses in the foreseeable future as a result of the Company's research and development activities.

In December 2021, the Company completed the sale of the Rare Pediatric Disease Priority Review Voucher ("PRV"), for net proceeds of $108.0 million, after deducting commission costs. In addition, in January and February 2022, the Company sold 992,389 shares of common stock in an at-the-market offering pursuant to a Sales Agreement entered into with SVB Leerink LLC in August 2020, at a weighted-average price of $18.04 per share, resulting in net proceeds of $17.4 million.

2. Summary of Significant Accounting Policies

Basis of Presentation

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 and expenses and the disclosure of contingent assets and liabilities in the financial statements and accompanying notes. The most significant estimates in the Company's consolidated financial statements relate to the calculation of estimated rebates for applicable discounts and allowances that are offered within contracts to customers, payors and other third parties, the calculation of the revenue interest liability, effective interest rate and corresponding expense, accrued research and development expenses, the valuation of derivative liabilities and the valuation allowance of deferred tax assets resulting from net operating losses. 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.

In December 2019, a novel strain of coronavirus, which causes COVID-19, was identified. Due to the rapid and global spread of the virus, on March 11, 2020, the World Health Organization declared the COVID-19 outbreak a pandemic. To slow the proliferation of COVID-19, governments have implemented extraordinary measures, which include the mandatory closure of businesses, restrictions on travel and gatherings, and quarantine and physical distancing requirements.

There were no significant estimates contained in the preparation of the Company's consolidated financial statements or impacts to the Company's consolidated financial statements for the year ended December 31, 2021 that were directly a result of the COVID-19 pandemic. 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 Equivalents

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.

Restricted cash equivalents consists of deposits placed in a segregated bank account as required under the terms of the Company's RIPA in connection with the sale of the PRV in December 2021.

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

	December 31,	
	2021	2020
Cash and cash equivalents	$ 31,340	$ 142,086
Restricted cash equivalents	100,000	—
Total cash, cash equivalents, and restricted cash equivalents	$ 131,340	$ 142,086

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 short and long-term 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 and/or commercial paper with short maturities. 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 specialty pharmacy and a single distributor for all of the Company's sales of Livmarli 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.

Investments

The Company classifies all investments as available-for-sale, as the sale of such securities may be required prior to maturity. Management determines the appropriate classification of its investments in debt 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. The Company periodically evaluates whether declines in fair values of its available-for-sale securities below their book value are other-than-temporary. This evaluation consists of several qualitative and quantitative factors regarding the severity and duration of the unrealized loss 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 interest income (loss). To date, the Company has not identified any other than temporary declines in fair value of its investments.

Fair Value of Financial Instruments

The accounting guidance defines fair value, establishes a consistent framework for measuring fair value and expands disclosure for each major asset and liability category measured at fair value on either a recurring or nonrecurring basis. 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. As a basis for considering such assumptions, the accounting guidance establishes a three-tier fair value hierarchy, which prioritizes the inputs used in measuring fair value as follows:

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.

The carrying amounts of all cash equivalents, available-for sale investments, accounts payable and accrued liabilities are reasonable estimates of their fair value.

Accounts Receivable

Accounts receivable are recorded net of allowances for sales discounts and any allowance for doubtful accounts. Management estimates the allowance for doubtful accounts based on existing contractual payment terms, actual payment patterns of customers and individual customer circumstances. To date, an allowance for doubtful accounts has not been material.

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. 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.

Prior to the initial regulatory approval for Livmarli, the Company expensed costs relating to raw materials and production of inventory as research and development expense in the accompanying consolidated statements of operations, in the period incurred.

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, 2021, property and equipment consisted primarily of leasehold improvements of $1.3 million and furniture and equipment of $0.2 million. As of December 31, 2020, property and equipment consisted primarily of leasehold improvements of $1.3 million and furniture and equipment of $0.3 million. Accumulated depreciation as of December 31, 2021 and 2020 was $0.7 million and $0.3 million, 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

Upon FDA approval of Livmarli in September 2021, contractual milestone payments the Company was then obligated to pay to licensors were evaluated as intangible assets for the completed regulatory approval and right to commercialize the product. The evaluation of intangible assets includes assessing the amortization period for which the asset is expected to contribute to the expected future cash flows of the Company. The Company determined the pattern of intangible asset expected future cash flows could not be readily determined with a high level of precision. As a result, the intangible asset is being amortized on a straight-line basis over the estimated useful life of 18 years. The Company tests its definite lived intangible assets for impairment annually 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 becomes impaired, the carrying value is written down to its 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.

The following table provides detail of the carrying amount of the Company's intangible assets:

		December 31, 2021
Gross carrying value	$	19,000
Less accumulated amortization		(260)
Net carrying value	$	18,740

The following table summarizes the estimated future amortization expense associated with our intangible assets (in thousands):

Years Ended December 31,		Amount
2022	$	1,038
2023		1,038
2024		1,038
2025		1,038
2026		1,038
Thereafter		13,550
	$	18,740

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 lease 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 sheet. 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.

Revenue Interest Liability, Net

The revenue interest liability, net, associated with the RIPA that the Company entered into in December 2020 with Mulholland SA LLC, an affiliate of Oberland Capital LLC ("Oberland"), as agent for purchasers party thereto (the "Purchasers"), and the Purchasers, is presented net of issuance costs and a debt discount on the consolidated balance sheets. The Company imputes interest expense associated with this liability using the effective interest rate method. The effective interest rate is 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 may vary during the term of the agreement depending on a number of factors, including the level of actual and forecasted product sales, net. The Company evaluates the interest rate quarterly based on actual product sales, net and forecast product sales, net, utilizing the prospective method. A significant increase or decrease in product sales, net will materially impact the revenue interest liability, interest expense and the time period for repayment.

Derivative Liability

The RIPA contains certain features that meet the definition of being an embedded derivatives requiring bifurcation as a separate compound financial instrument apart from the RIPA. The derivative liability is initially measured at fair value on issuance and is subject to remeasurement at each reporting period with changes in fair value recognized as other income (expense) in the accompanying consolidated statements of operations as the change in fair value of derivative liability.

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 our 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 Livmarli. Estimates of variable consideration are calculated based on the actual product sales each reporting period. 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 quarterly 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 in 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 Livmarli 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.

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)* ("ASC 808") and determine 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)* ("ASC 606") for distinct units of account that are reflective of a vendor-customer relationship. For other elements of collaboration arrangements, such as assistance with development of the drug products, the Company applies the illustrative examples in ASC 808 and generally records reimbursements received as a reduction of research and development expenses.

In determining the appropriate amount of revenue to be recognized 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 we satisfy 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 Product Sales

Prior to receiving approval from the FDA in September 2021 to sell Livmarli in the United States, the Company expensed all costs incurred related to the manufacture of Livmarli 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 approval when commercialization was considered probable and the future economic benefit is expected to be realized, the Company began capitalizing inventory costs incurred. At December 31, 2021, the Company capitalized $1.5 million of inventory recorded as a component of other current assets on the consolidated balance sheet.

Cost of product sales consist of manufacturing costs, transportation and freight, amortization of capitalized intangibles, royalties and indirect overhead costs associated with the manufacturing and distribution of Livmarli. Cost of product 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.

We expense the costs of advertising, including promotional expenses, as incurred. Advertising expenses were $9.2 million and $0.6 million for the years ended December 31, 2021 and 2020, 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. For stock-based awards with service conditions, the fair value of the awards is amortized 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.

Income Taxes

Income taxes are recorded using the liability method, under which deferred tax assets and liabilities are determined based on differences between 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 attributable to common stockholders 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 attributable to common stockholders 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, contingently issuable employee stock purchase plan shares and contingently issuable overallotment shares because their effect would be anti-dilutive due to the Company's net loss. Since the Company incurred a net loss in each of the periods presented, basic and diluted net loss per share were the same.

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

	December 31,	
	2021	2020
Options to purchase common stock and restricted stock units	6,940,566	5,071,740
Common stock subject to repurchase	122,464	256,056
Employee stock purchase plan contingently issuable	23,116	12,931
Underwriter option shares contingently issuable	—	562,500
Total	7,086,146	5,903,227

Recently Adopted Accounting Pronouncements

In November 2018, the FASB issued Accounting Standards Update ("ASU") No.2018-18, Collaborative Arrangements (Topic 818): Clarifying the Interaction Between Topic 808 and Topic 606, which clarifies when transactions between participants in a collaborative arrangement are within the scope of the FASB's revenue standard, Topic 606. The standard is effective for fiscal years beginning after December 15, 2019 and interim periods within those fiscal years, with early adoption permitted. The Company adopted this standard on January 1, 2021, as it had no collaboration arrangements prior to that date. There was no impact on the accompanying consolidated financial statements as of the adoption date.

Recent Accounting Pronouncements Not Yet Adopted

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments-Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments* ("ASU 2016-13"). ASU 2016-13 requires an entity to utilize a new impairment model that requires measurement and recognition of expected credit losses for most financial assets and certain other instruments, including but not limited to available-for-sale debt securities. Credit losses relating to available-for-sale debt securities will be recorded through an allowance for credit losses rather than as a direct write-down to the security. The new guidance requires the use of forward-looking expected credit loss models based on historical experience, current conditions, and reasonable and supportable forecasts that affect the collectability of the reported amount, which may result in earlier recognition of credit losses under the new guidance. The new guidance also modifies the impairment models for available-for-sale debt securities and for purchased financial assets with credit deterioration since their origination. Subsequent to the issuance of ASU 2016-13, the FASB issued ASU 2018-19, *Codification Improvements to Topic 326, Financial Instruments - Credit Losses*. This ASU does not change the core principle of the guidance in ASU 2016-13, instead these amendments are intended to clarify and improve operability of certain topics included within the credit losses guidance. The FASB also subsequently issued ASU No. 2019-04, *Codification Improvements to Topic 326, Financial Instruments—Credit Losses, Derivatives and Hedging (Topic 815), and Financial Instruments (Topic 842),* which did not change the core principle of the guidance in ASU 2016-13 but clarified that expected recoveries of amounts previously written off and expected to be written off should be included in the valuation account and should not exceed amounts previously written off and expected to be written off. In March 2020, the FASB issued ASU No. 2020-3, *Codification Improvements to Financial Instruments* which makes narrow-scope improvements to various financial instruments topics, including the new credit losses standard and clarifies the following areas (i) the contractual term of a net investment in a lease should be the contractual term used to measure expected credit losses; (ii) when an entity regains control of financial assets sold, an allowance for credit losses should be recorded. The guidance is effective for fiscal years, and interim periods within those years, beginning after December 15, 2019 for public business entities, excluding smaller reporting companies. For smaller reporting companies, the guidance will be effective during the first quarter of 2023. The Company is in the process of assessing the impact adoption will have on its consolidated financial statements.

In October 2021, the FASB, issued Accounting Standards Update No. 2021-08, *Business Combinations (Topic 805), Accounting for Contract Assets and Contract Liabilities from Contracts with Customers*, which requires an entity (acquirer) to recognize and measure contract assets and liabilities acquired in a business combination in accordance with Topic 606, Revenue from Contracts with Customers. This update is effective for fiscal years beginning after December 15, 2022, and interim periods within those fiscal years, with early adoption permitted. The amendments should be applied prospectively to business combinations occurring on or after the effective date of the amendments. The Company is currently evaluating the impact the 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, 2021			
	Level 1	Level 2	Level 3	Total
Financial assets:				
Money market fund	$ 128,420	$ —	$ —	$ 128,420
Commercial paper	—	$ 115,221	—	115,221
U.S. government bonds	—	$ 14,963	—	14,963
Total financial assets	$ 128,420	$ 130,184	$ —	$ 258,604
Financial liabilities:				
Derivative liability	—	—	1,996	1,996
Total financial liabilities	$ —	$ —	$ 1,996	$ 1,996

	December 31, 2020			
	Level 1	Level 2	Level 3	Total
Financial assets:				
Money market fund	$ 127,783	$ —	$ —	$ 127,783
U.S. treasury bills	29,997	—	—	29,997
Corporate debt securities	—	23,201	—	23,201
Commercial paper	—	41,460	—	41,460
U.S. government bonds	—	5,066	—	5,066
Asset-backed securities	—	2,006	—	2,006
Total financial assets	$ 157,780	$ 71,733	$ —	$ 229,513
Financial liabilities:				
Derivative liability	—	—	1,264	1,264
Total financial liabilities	$ —	$ —	$ 1,264	$ 1,264

The carrying amounts of certain financial instruments such as cash and cash equivalents, accounts receivable, prepaid expenses, other current assets, accounts payable, accrued expenses, and other current liabilities as of December 31, 2021 and 2020 approximate their related fair values due to the short-term maturities of these instruments. 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 carrying amount of the revenue interest liability as of December 31, 2021 and 2020 approximates its fair value and is based on the Company's contractual repayment obligation to the Purchasers, based on current estimates of future revenues, over the life of the RIPA. The derivative liability is considered a Level 3 input based on the three-level hierarchy. Refer to Note 6 "Revenue Interest Purchase Agreement" for further information.

Derivative Liability

The debt pursuant to the RIPA contains an embedded derivatives requiring bifurcation as a single compound derivative instrument. The Company estimated the fair value of the derivative liability using a "with-and-without" method. The "with-and-without" methodology involves valuing the whole instrument on an as-is basis and then valuing the instrument without the individual embedded derivative. The difference between the entire instrument with the embedded derivative compared to the instrument without the embedded derivative was the fair value of the derivative liability at December 31, 2021 and 2020. The estimated probability and timing of underlying events triggering the exercisability of the put options contained within the RIPA, forecasted cash flows and the discount rate are significant unobservable inputs used to determine the estimated fair value of the entire instrument with the embedded derivative. As of December 31, 2021 and 2020, the discount rate used for valuation of the derivative liability is 15.7% and 15.9%, respectively.

The following table provides a summary of the change in the estimated fair value of the Company's derivative liability, classified as Level 3 in the fair value hierarchy:

Balance at January 1, 2020	$ —
Initial fair value of derivative liability	1,264
Balance at December 31, 2020	1,264
Change in fair value of derivative liability	732
Balance at December 31, 2021	$ 1,996

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, 2021			
	Amortized Cost	Unrealized Gain	Unrealized Loss	Estimated Fair Value
Cash equivalents and investments:				
Money market fund	$ 128,420	$ —	$ —	$ 128,420
Commercial paper	115,221	—	—	115,221
U.S. government bonds	14,999	—	(36)	14,963
Total cash equivalents and investments	$ 258,640	$ —	$ (36)	$ 258,604
Classified as:				
Cash equivalents				$ 28,420
Cash equivalents - Restricted				100,000
Short-term investments				125,201
Long-term investments				4,983
Total cash equivalents and investments				$ 258,604

	December 31, 2020			
	Amortized Cost	Unrealized Gain	Unrealized Loss	Estimated Fair Value
Cash equivalents and investments:				
Money market fund	$ 127,783	$ —	$ —	$ 127,783
U.S. treasury bills	29,995	2	—	29,997
Corporate debt securities	23,126	75	—	23,201
Commercial paper	41,460	—	—	41,460
U.S. government bonds	5,067	—	(1)	5,066
Asset-backed securities	2,001	5	—	2,006
Total cash equivalents and investments	$ 229,432	$ 82	$ (1)	$ 229,513
Classified as:				
Cash equivalents				$ 139,779
Short-term investments				89,734
Total cash equivalents and investments				$ 229,513

As of December 31, 2021, the remaining contractual maturities of available-for-sale debt securities were as follows: (in thousands):

	Estimated Fair Value
Due within one year	$ 125,201
One to two years	4,983
Total	$ 130,184

During the year ended December 31, 2021 and 2020, there have been no significant realized gains or losses on available-for-sale investments, no investments had been in a continuous unrealized loss position for more than 12 months, and the Company did not recognize any other-than-temporary impairment losses on these securities.

5. Balance Sheet Components

Inventory

The following is a summary of the Company's inventory by category:

	December 31, 2021
Raw material	$ 856
Work in progress	570
Finished goods	87
Total inventory	$ 1,513

The Company did not have any inventory as of December 31, 2020.

Accrued Expenses

Accrued expenses consist of the following (in thousands):

	December 31,	
	2021	2020
Accrued clinical trials	$ 6,732	$ 3,673
Accrued professional service fees	3,068	2,157
Accrued contract manufacturing and non-clinical costs	4,635	2,780
Accrued compensation and related benefits	9,988	4,801
Accrued collaboration funding	6,300	—
Total accrued expenses	$ 30,723	$ 13,411

6. Revenue Interest Purchase Agreement

In December 2020, the Company entered into the RIPA, as amended in September 2021 with Oberland 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 has received $115.0 million consisting of an upfront payment of $50.0 million in December 2020 and $65.0 million in April 2021 associated with the acceptance for filing by the FDA of an NDA for Livmarli for the treatment of cholestatic pruritus in patients with ALGS, less certain transaction expenses.

The Company may also be entitled to receive up to approximately $50.0 million at the option of the Purchasers to finance in-licenses or other acquisitions on or prior to December 31, 2022. The Company was entitled to receive an additional $35.0 million upon FDA approval of Livmarli, which it elected to forgo.

As consideration for such payments, the Purchasers have the right to receive the Revenue Interests from the Company based on annual product sales, net of Livmarli, if approved, which will be tiered payments (the "Revenue Interest Payments") based on whether such annual product sales, net are (i) less than or equal to $350.0 million

("Tier 1"), (ii) exceeding $350.0 million and less than or equal to $1.1 billion ("Tier 2"), or (iii) exceeding $1.1 billion ("Tier 3").

The Revenue Interest Payments will initially be 9.75% (at Tier 1) and 2.00% (at Tier 2 and Tier 3) of such annual net sales. If the Purchasers have received Revenue Interest Payments in an amount equal to or greater than 110.0% of the total payments actually made by the Purchasers to the Company, exclusive of transaction expenses (the "Cumulative Purchaser Payments"), on or prior to December 31, 2026, the Revenue Interests shall be reduced to 2.00% at Tier 1 and 0.00% at Tier 3 for all subsequent calendar years beginning on January 1, 2027. If the Purchasers have not received Revenue Interest Payments in an amount equal to or greater than 110.0% of the Cumulative Purchaser Payments on or prior to December 31, 2026, the Revenue Interests shall be increased for all subsequent calendar years beginning on January 1, 2027 to a single defined rate (with no separate tiers) that would have provided the Purchasers with an amount equal to 110.0% of the Cumulative Purchaser Payments on or prior to December 31, 2026 had such rate applied to Tier 1 of initial Revenue Interest Payments. The Purchasers' rights to receive the Revenue Interest Payments shall terminate on the date on which the Purchasers have received Revenue Interest Payments of 195.0% of the Cumulative Purchaser Payments, unless the RIPA is terminated earlier.

Under the RIPA, the Company has an option (the "Call Option") to terminate the RIPA and repurchase future Revenue Interests at any time upon advance written notice. Additionally, the Purchasers have an option (the "Put Option") to terminate the RIPA and to require the Company to repurchase future Revenue Interests upon enumerated events such as a bankruptcy event, an uncured material breach, a material adverse effect or a change of control, or upon the 12th anniversary of the first payment made by Purchasers. If the Put Option is exercised prior to the first anniversary of the closing date by the Purchasers (except pursuant to a change of control), the required repurchase price will be 120.0% of the Cumulative Purchaser Payments (minus all payments Company has made to the Purchasers in connection with the Revenue Interests). In all other cases, if the Put Option or the Call Option are exercised, the required repurchase price will be 175.0% of the Cumulative Purchaser Payments (minus all payments Company has made to the Purchasers in connection with the Revenue Interests), if such option is 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 is exercised thereafter.

In addition, the RIPA contains various representations and warranties, information rights, non-financial covenants, indemnification obligations and other provisions that are customary for a transaction of this nature. The Purchaser's obligations to fund the scheduled installments are subject to certain customary conditions as set forth in the RIPA.

Concurrently with the RIPA, the Company entered into a Common Stock Purchase Agreement ("CSPA") with certain affiliates of Oberland, pursuant to which the Company sold an aggregate of 509,164 shares of its common stock for an aggregate purchase price of $10.0 million. The $50.0 million received pursuant to the RIPA and $10.0 million received pursuant to the CSPA was allocated between the resulting financial instruments on a relative fair value basis, with $49.2 million allocated to the debt under the RIPA and $10.8 million allocated to the common stock issued under the CSPA.

The Put Options under the RIPA that are exercisable by Purchasers upon certain contingent events were determined to be embedded derivatives requiring bifurcation and separately accounted for as a single compound derivative instrument. The Company recorded the initial fair value of the derivative liability of $1.3 million as a debt discount, which is amortized to interest expense over the expected term of the debt using the effective interest method.

In connection with the RIPA, as of December 31, 2021 and 2020, $129.9 and $47.7 million, respectively, was recorded as a revenue interest liability on the accompanying consolidated balance sheet. The Company imputes interest expense associated with this liability using the effective interest rate method. The effective interest rate is 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 this liability may vary during the term of the agreement depending on a number of factors, including the level of forecasted product sales, net. The Company evaluates the interest rate quarterly based on its actual product sales, net and forecast product sales, net utilizing the prospective method. A significant increase or decrease in product sales, net will materially impact the revenue interest liability, interest expense and the time

period for repayment. The Company recorded $17.6 million and $3.8 million in interest expense related to this arrangement for the year ended December 31, 2021 and 2020, respectively.

As of December 31, 2021, the Company had incurred $0.9 million of issuance costs in connection with the RIPA, which are amortized to interest expense over the estimated term of the debt.

Revenue Interest Payments made as a result of the Company's product sales, net reduce the revenue interest liability. During the year ended December 31, 2021, the Company made a payment of $0.1 million in connection with the RIPA.

The following table summarizes the revenue interest liability activity during the years ended December 31, 2021 and 2020 (in thousands):

Revenue interest liability at inception	$	49,234
Interest expense recognized		335
Capitalized issuance costs		(654)
Debt discount from embedded derivatives		(1,264)
Revenue interest liability at December 31, 2020	$	47,651
Proceeds from purchaser payments		65,000
Interest expense recognized		17,590
Capitalized issuance costs		(197)
Revenue interest payments		(121)
Revenue interest liability at December 31, 2021	$	129,923

7. Asset Acquisitions

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, and made an upfront payment to Shire of $7.5 million and issued Shire 1,859,151 shares of redeemable common stock with an estimated fair value of $7.0 million, or $3.76 per share. 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 Pharmaceuticals, Inc. ("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 product sales milestones on total licensed products up to an aggregate of $30.0 million. 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. The Company paid $32.0 million and $10.0 million Livmarli development and regulatory milestones for the year ended December 31, 2021 and 2020, respectively and $2.0 million and none volixibat development and regulatory milestones for the year ended December 31, 2021 and

2020, respectively. As of December 31, 2021, no milestones had been accrued as there were no potential milestones yet considered probable.

Satiogen License

Through the Shire Agreement, the Company was assigned a license agreement with Satiogen 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 to Satiogen up to an aggregate of $10.5 million upon the achievement of certain milestones, of which $0.5 million was for initiation of certain development activities, $5.0 million for the completion of regulatory approvals and $5.0 million for commercialization activities. Additionally, the Company will be required to pay a low single-digit royalty on net sales. The Company's royalty obligations continue on a licensed product-by-licensed product and country-by-country basis until the expiration of the last valid claim in a licensed patent covering the applicable licensed product in such country. Royalty obligations under the Satiogen license are creditable against the royalty obligations to Shire under the Shire Agreement. In October 2021, the Company paid $4.0 million associated with milestones for the FDA approval and for the first commercial sale of Livmarli. As of December 31, 2021, no milestones had been accrued as there were no potential milestones yet considered probable.

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 will be 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. As of December 31, 2021, no milestones had been accrued as there were no potential milestones yet considered probable.

8. Collaboration and License Agreement

License and Collaboration Agreement with CANbridge

In April 2021, the Company entered into an exclusive license and collaboration agreement with CANbridge Pharmaceuticals, Inc. ("CANbridge"). Under the terms of the agreement, CANbridge has obtained the exclusive right to develop and commercialize Livmarli within the Greater China regions (China, Hong Kong, Macau and Taiwan). In connection with the agreement, the Company received an upfront payment of $11.0 million, which, upon satisfaction of the performance obligation and receipt by CANbridge of the right to use and benefit from the license, was recorded as license revenue in the accompanying consolidated statements of operations. Additionally, the Company is eligible to receive up to $5.0 million in research and development funding, and up to $109.0 million for the achievement of future regulatory and commercial milestones, with double-digit tiered royalties based on product net sales. The Company concluded at inception of the agreement that the transaction price should not include the variable consideration related to unachieved developmental and regulatory milestones as this consideration was considered to be constrained as it is probable that the inclusion of such variable consideration could result in a significant reversal in cumulative revenue. The Company will recognize any consideration related to sales-based payments (including milestones and royalties) when the related sales occur, as the Company has determined that these amounts relate predominantly to the license granted and therefore will be recognized 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). The Company re-evaluates the transaction price at each reporting period as uncertain events are resolved and other changes in circumstances occur. For the year ended December 31, 2021, no adjustments were made to the transaction price. For the year ended December 31, 2021, the Company recorded research and development funding of $1.9 million, payable by CANbridge to the Company which is reflected as a reduction of research and development expense in the accompanying consolidated statements of operations. As of December 31, 2021, such research and development funding of $0.8 million was recorded as a receivable which was included in prepaids and other current assets on the accompanying consolidated balance sheets. In January 2022, CANbridge achieved a regulatory milestone, triggering a milestone payment to the Company of $2.0 million.

License and Collaboration Agreement with GC Pharma

In July 2021, the Company entered into an exclusive license and collaboration agreement with GC Pharma. Under the terms of the agreement, GC Pharma has obtained the exclusive right to develop and commercialize Livmarli within South Korea for ALGS, PFIC, and BA. In connection with the agreement, the Company received a $5.0 million upfront payment, which, upon satisfaction of the performance obligation and receipt by GC Pharma of the right to use and benefit from the license, was recorded as license revenue in the accompanying consolidated financial statements of operations. Additionally, the Company is entitled to certain research and development funding and up to $23.0 million for the achievement of future regulatory and commercial milestones, with double-digit tiered royalties based on product net sales. At inception of the agreement, the Company concluded that the transaction price should not include the variable consideration related to unachieved developmental and regulatory milestones as this consideration was considered to be constrained as it is probable that the inclusion of such variable consideration could result in a significant reversal in cumulative revenue for this contract when the uncertainty is resolved in the future. The Company will recognize any consideration related to sales-based payments (including milestones and royalties) when the related sales occur, as the Company has determined that these amounts relate predominantly to the license granted and therefore will be recognized on the later to occur of satisfaction of the performance obligation or the occurrence of the related sales. The Company will re-evaluate the transaction price at each reporting period as uncertain events are resolved and other changes in circumstances occur. For the year ended December 31, 2021, no adjustments were made to the transaction price. For the year ended December 31, 2021, the Company recorded research and development funding of $0.3 million payable by GC Pharma to the Company which is reflected as a reduction of research and development expense in the accompanying consolidated statements of operations. As of December 31, 2021, such research and development funding of $0.3 million was recorded as a receivable which was included in prepaids and other current assets on the accompanying consolidated balance sheets.

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. Under the terms of the agreement, Takeda will be responsible for regulatory approval and commercialization of Livmarli in Japan. Takeda will also be responsible for development, including conducting clinical studies in cholestatic indications. The Company is responsible for commercial supply to Takeda. In exchange, the Company is eligible to receive a percentage of Takeda's annualized 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.

Option, License and Collaboration Agreement with Vivet

In April 2021, the Company entered into an Option, License and Collaboration Agreement ("Vivet Collaboration Agreement") with Vivet Therapeutics SAS ("Vivet"). Pursuant to the Vivet Collaboration Agreement, Vivet granted the Company the exclusive option, at the Company's discretion, to develop and subsequently commercialize Vivet's two proprietary AAV gene therapy programs for PFIC, subtypes 3 and 2. Under the terms of the Vivet Collaboration Agreement, the Company paid an upfront fee of $4.2 million and agreed to provide funding to support certain research and development costs associated with the two gene therapy programs through July 2023. In December 2021, the Company elected not to exercise its option, terminating the agreement. The Company made a final payment of $6.3 million in January 2022 under terms of the agreement, which was reflected in accrued expenses at December 31, 2021 on the accompanying consolidated balance sheets.

For the year ended December 31, 2021, pursuant to the terms of the Vivet Collaboration Agreement, the Company recorded research and development expense of $18.9 million in the accompanying consolidated statements of operations.

9. Leases

In January 2019, the Company entered into an operating lease agreement for office space which consisted of approximately 5,600 square feet (the "initial lease"). The lease term is approximately four years with an option to extend the term for one five-year term, which at the time was not reasonably assured of exercise and therefore, not included in the lease term. The lease contained a tenant improvement allowance of $0.4 million, which has been recorded as leasehold improvements in the accompanying consolidated balance sheets with a corresponding reduction of the ROU asset at inception of the lease. Rent payments commenced in August 2019.

In November 2019, the Company amended the operating lease agreement (the "amended agreement") to extend the term of the initial lease through March 2025. This extension was accounted for as a lease modification and the Company recorded an increase to the ROU asset and lease liability of $0.6 million at the time of the amendment.

Additionally, pursuant to the amended agreement, the Company expanded the office space by 5,555 square feet for a five-year term expiring in March 2025 (the "expanded space"). The Company accounted for the expanded space as a separate contract as there were material additional rights of use that were not included in the initial lease. The amended lease contained a tenant improvement allowance of $0.8 million in connection with the expanded space, which has been recorded as leasehold improvements on the accompanying consolidated balance sheet with a corresponding reduction of the ROU asset at inception of the lease for the expanded space.

The ROU and corresponding lease liabilities were estimated using a weighted-average incremental borrowing rate of 8.0%.

As of December 31, 2021, the Company recorded an aggregate ROU asset of $1.6 million and an aggregate lease liability of $2.6 million on the accompanying consolidated balance sheet. The weighted-average remaining lease term is 3.1 years.

As of December 31, 2021, undiscounted future minimum payments under the Company's operating leases are as follows (in thousands):

Years Ended December 31,	Undiscounted Rent Payments
2022	$ 889
2023	918
2024	926
2025	229
Total undiscounted lease payments	2,962
Less: imputed interest	(348)
Total lease liability	$ 2,614

Rent expense was $0.7 million and $0.6 million for the years ended December 31, 2021 and 2020, respectively. Variable lease payments for lease operating expenses were immaterial for the years ended December 31, 2021 and 2020.

10. Stockholders' Equity (Deficit)

Common Stock

In January 2020, the Company completed a follow-on public offering of its common stock, pursuant to which the Company sold 2,400,000 shares of common stock at a price of $20.00 per share, resulting in net proceeds of $44.7 million after deducting underwriting discounts, commissions and offering expenses.

In August 2020, the SEC declared effective a registration statement on Form S-3 ("Shelf Registration") covering the sale of up to $300.0 million of the Company's securities. Also, in August 2020, the Company entered into a sales agreement ("Sales Agreement") with SVB Leerink LLC ("SVB Leerink") pursuant to which the Company may elect to issue and sell, from time to time, shares of common stock having an aggregate offering price

of up to $75.0 million under the Shelf Registration through SVB Leerink acting as the sales agent and/or principal. During the year ended December 31, 2020, the Company sold 305,969 shares of common stock in an at-the-market offering pursuant to the Sales Agreement at a weighted-average price of $21.55 per share, resulting in gross proceeds of $6.6 million. The net proceeds after deducting sales commissions to SVB Leerink and other issuance expenses were approximately $6.3 million. The remaining capacity under the Sales Agreement is approximately $68.4 million as of December 31, 2021. In January and February 2022, the Company sold 992,389 shares of common stock in at-the-market offerings pursuant to the Sales Agreement at a weighted-average price of $18.04 per share, resulting in net proceeds of $17.4 million.

In December 2020, the Company completed an underwritten public offering of its common stock pursuant to the Shelf Registration. The Company sold 3,750,000 shares of common stock at a price of $20.00 per share, resulting in net proceeds of $70.0 million after deducting underwriting discounts, commissions and offering expenses. In addition, the Company granted the underwriters an option, exercisable for 30 days, to purchase up to 562,500 additional shares of its common stock at the public offering price, less the underwriting discounts and commissions. In January 2021, the underwriters partially exercised their option and purchased 375,654 shares of the Company's common stock at a price of $20.00 per share, resulting in net proceeds of $7.1 million after deducting underwriting discounts.

As of December 31, 2021 and 2020, 122,464 and 256,056 shares of common stock, respectively, were subject to repurchase by the Company. The unvested stock liability related to these shares is immaterial to all periods presented.

Common Stock Reserved for Issuance

Common stock reserved for issuance is as follows:

	December 31,	
	2021	2020
Stock options and RSUs issued and outstanding	6,940,566	5,071,740
Reserved for future stock awards or option grants	2,434,619	2,009,410
Reserved for employee stock purchase plan	911,138	700,023
Reserved for underwriter option shares	—	562,500
	10,286,323	8,343,673

11. 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. A total of 1,401,443 shares of common stock were approved to be initially reserved for issuance under the 2019 Plan, including 101,443 shares that remained available for issuance under the 2018 Plan as of July 17, 2019. 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, 2021, 1,362,583 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). At adoption, the 2020 Inducement Plan authorized 750,000 shares of the Company's common stock for future issuance. In 2021 and 2020, the Company's board of directors authorized an additional 1,000,000 and 750,000 shares of the Company's common stock for future issuance, respectively. As of December 31, 2021, 1,072,036 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, 2021 (in thousands, except share and per share data):

	Number of Shares	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life (in Years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2020	5,071,740	$ 9.99	8.6	$ 40,631
Granted	2,359,200	$ 18.10		
Exercised	(127,505)	$ 6.62		
Canceled and forfeited	(432,818)	$ 14.37		
Outstanding as of December 31, 2021	6,870,617	$ 12.56	8.0	$ 32,637
Vested and exercisable as of December 31, 2021	2,999,810	$ 8.73	7.2	$ 23,812

Intrinsic value is calculated as the difference between the exercise price of the underlying options and the fair value of the common stock 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 year ended December 31, 2021 and 2020 was $13.39 and $12.70 per share, respectively. The total intrinsic value of options exercised during the year ended December 31, 2021 and 2020 was $1.3 million and $0.5 million, respectively. As of December 31, 2021, the total unrecognized stock-based compensation related to unvested stock option awards granted was $40.9 million, which the Company expects to recognize over a weighted-average period of approximately 2.5 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 Company estimated expected volatility based on the historical volatility of a group of similar companies that are publicly traded. The historical volatility data was computed using the daily closing prices for the selected 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 period:

	Year ended December 31,	
	2021	2020
Exercise price	$14.05-$21.22	$10.40-$26.59
Expected term (in years)	5.5-6.1	5.5-6.1
Expected volatility	82.76%-94.06%	77.07%-95.85%
Risk-free interest rate	0.62%-1.34%	0.33%-1.73%
Expected dividend yield	—	—
Grant date fair value of options granted	$9.62-$16.01	$7.38-$20.21

Performance Stock Units

In March 2021, the Company granted PSUs to employees which are subject to a performance condition of FDA marketing approval of Livmarli ("NDA Approval") by a certain date. Upon achievement, 50% vest immediately and 50% vest on June 30, 2023, subject to the employees' continuous service through each vesting date. As of September 29, 2021, the Company determined achievement of the NDA Approval performance condition was met. As a result, 76,027 of the PSUs vested on that date.

Additionally, in March 2021, the Company granted an aggregate of 75,688 PSUs to certain executive participants ("Executive PSUs"). The Executive PSUs are subject to performance and market conditions: (1) NDA Approval by a certain date and (2) achievement of a specified stock price. As of December 31, 2021, the NDA approval criteria was met; however, the Company did not achieve the specified stock price. As a result, the Executive PSU's were forfeited.

As the Executive PSU's contained a market condition, the grant date fair value was determined using a Monte Carlo simulation model and the weighted-average grant date fair value of these Executive PSU's was $7.09 per share.

As of December 31, 2021, the Company determined achievement of the NDA Approval performance condition was met as applied under the Accounting Standards Codification 718, Compensation — Stock Compensation (Topic 718). The total unrecognized stock-based compensation related to the PSUs and the Executive PSUs was $0.8 million as of December 31, 2021.

The following table summarizes the activity related to PSUs for the year ended December 31, 2021:

	Number of Shares	Weighted-Average Grant Date Fair Value
Unvested and Outstanding as of December 31, 2020	$ —	$ —
Granted	229,856	14.78
Vested	(80,090)	18.56
Cancelled and forfeited	(79,817)	7.68
Unvested and Outstanding as of December 31, 2021	$ 69,949	$ 18.57

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"). The ESPP became effective on July 17, 2019. A total of 500,000 shares of common stock were approved to be initially reserved for issuance under the ESPP. In addition, the number of shares of common stock available for issuance under the ESPP will be automatically increased on the first day of each calendar year during the first ten years of the term of the ESPP, beginning with January 1, 2020 and ending with January 1, 2029, by an amount equal to the lesser of (i) 1% of the outstanding number of shares of common stock on December 31st of the preceding calendar year, (ii) 1,500,000 shares of common stock or (iii) such lesser amount as determined by the Company's board of directors. During the year ended December 31, 2021, 89,211 shares were issued under the ESPP. As of December 31, 2021, the Company had 911,138 shares available for future issuance under the ESPP. The stock-based compensation related to the ESPP for the year ended December 31, 2021 was $0.7 million.

Restricted Stock

In November 2018, in connection with the issuance of the Series A Preferred Stock, the Company's founders agreed to modify their outstanding shares of common stock to include vesting provisions that require continued service to the Company in order to vest in those shares. As such, the 562,500 modified shares of common stock became compensatory upon such modification. The modified shares have a four-year vesting period and a measurement date fair value of $2.94 per share. For the years ended December 31, 2021 and 2020, 133,592 and 133,593 shares vested, respectively. The total fair value of shares vested was $0.4 million during the years ended December 31, 2021 and 2020, which was recorded as stock-based compensation in the consolidated statement of

operations. As of December 31, 2021, the total unrecognized compensation expense related to unvested restricted stock was $0.4 million expected to be recognized over a weighted-average period of approximately 0.9 years.

Total stock-based compensation is reflected in the consolidated statements of operations as follows (in thousands):

	Year Ended December 31,	
	2021	2020
Selling, general and administrative	$ 13,128	$ 7,425
Research and development	9,888	5,129
Total	$ 23,016	$ 12,554

12. Gain from Sale of Priority Review Voucher

In November 2021, the Company entered into a definitive agreement to sell the PRV that it received from the FDA in connection with the approval of Livmarli for the treatment of cholestatic pruritus in patients with ALGS one year of age and older, for cash proceeds of $110.0 million. In December 2021, the Company completed its sale of the PRV and received net proceeds of $108.0 million, after deducting commission costs, which was recorded as a gain within other income (loss) in the accompanying consolidated statements of operations.

13. Income Taxes

The Company's losses before provision for income taxes are as follows (in thousands):

	Year Ended December 31,	
	2021	2020
U.S. loss before taxes	$ (84,217)	$ (103,371)
Foreign income before taxes	266	107
Loss before income taxes	$ (83,951)	$ (103,264)

For the years ended December 31, 2021 and 2020, the Company had a current tax provision of $37,000 and $6,000 related to foreign taxes, respectively.

A reconciliation of the federal statutory income tax rate to the Company's effective income tax rate is as follows:

	Year Ended December 31,	
	2021	2020
Federal statutory income tax rate	21.00 %	21.00 %
State tax	1.32	0.42
Permanent differences	(1.70)	(0.89)
Other	0.02	—
Tax credits	7.72	6.42
Change in valuation allowance	(28.41)	(26.96)
Total tax benefit	(0.05) %	(0.01) %

The significant components of the Company's deferred taxes are as follows (in thousands):

	December 31,	
	2021	2020
Deferred tax assets:		
Net operating losses	$ 31,819	$ 26,122
Tax credit carryforwards	18,501	11,294
Accrued expenses	4,027	812
Intangibles	10,277	5,560
Lease liability	543	661
Stock-based compensation	5,815	2,769
Total deferred tax assets	70,982	47,218
Deferred tax liabilities:		
Operating lease right-of-use assets	(319)	(385)
Fixed assets	(138)	(184)
Total deferred tax liabilities	(457)	(569)
Valuation allowance	(70,525)	(46,649)
Net deferred tax assets	$ —	$ —

The valuation allowance increased by $23.9 million and $27.8 million for the years ended December 31, 2021 and 2020, 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, 2021 and 2020, 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.

The Company had federal and state net operating loss carryforwards of approximately $150.0 million and $6.7 million at December 31, 2021, and $123.5 million and $2.6 million at December 31, 2020, respectively. Federal losses do not expire, and California net operating and other state income net operating losses will begin to expire in 2039 and 2041, respectively. The Company also has federal general business credit and California research and development credit carryforwards totaling $23.1 million and $2.1 million at December 31, 2021, and $14.5 million and $0.9 million at December 31, 2020, respectively. The federal research and development credit carryforwards will begin to expire in 2032, unless previously utilized. The California research credits do not expire.

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, 2021, the Company has not determined to what extent a potential ownership change will impact the annual limitation that may be placed on the Company's utilization of its NOL carryovers and research and development credit carryforwards.

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,	
	2021	2020
Balance at beginning of year	$ 3,933	$ 1,490
(Decrease) related to prior year tax positions	—	(17)
Increases related to current year tax positions	2,466	2,460
Balance at end of year	$ 6,399	$ 3,933

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 rollforward above. The Company's effective income tax rate would not be impacted if the unrecognized tax benefits were recognized in 2021 and 2020, as the Company is in a full valuation allowance position.

The Company is subject to taxation in the United States federal jurisdiction, state jurisdictions and Switzerland. Due to the Company's losses incurred, the Company is subject to the income tax examination by authorities since inception on May 2, 2018. The Company's policy is to recognize interest expense and penalties related to income tax matters as tax expense. As of December 31, 2021, 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, 2021 and 2020, 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. We have elected to account for Global Intangible Low-taxed Income (GILTI) as a current period expense when incurred.

14. Contingencies

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 financial statements.

An estimated loss contingency is accrued in the Company's 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 accrual for loss contingencies associated with such government regulations, claims or legal actions, determined that an unfavorable outcome is probable or reasonably possible, or determined that the amount or range of any possible loss is reasonably estimable.

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, have evaluated 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, as of December 31, 2021, our disclosure controls and procedures were effective to provide reasonable assurance that information we are required to disclose in reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in SEC rules and forms, and that such information is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure.

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 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.

Internal control over financial reporting cannot provide absolute assurance of achieving financial reporting objectives because of its inherent limitations. Internal control over financial reporting is a process that involves human diligence and compliance and is subject to lapses in judgment and breakdowns resulting from human failures. Internal control over financial reporting also can be circumvented by collusion or improper management override. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. Further, the design of a control system must reflect the fact that there are resource constraints, and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, within the Company have been detected. Because of such limitations, there is a risk that material misstatements may not be prevented or detected on a timely basis by internal control over financial reporting. However, these inherent limitations are known features of the financial reporting process. Therefore, it is possible to design into the process safeguards to reduce, though not eliminate, this risk.

Under the supervision and with the participation of our Chief Executive Officer and our Chief Financial Officer, our management conducted an evaluation of the effectiveness of our internal control over financial reporting based on the criteria set forth in "Internal Control—Integrated Framework" issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework). 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, 2021.

Attestation Report of the Independent Registered Public Accounting Firm

This Annual Report on Form 10-K does not include an attestation report of our registered public accounting firm regarding internal control over financial reporting due to an exemption established by the JOBS Act for "emerging growth companies" and because we qualify as a "non-accelerated filer" (i.e., we do not qualify as either an "accelerated filer" or a "large accelerated filer" as defined in Rule 12b-2 under the Exchange Act).

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, 2021 that materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information

None.

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 contained in our definitive proxy statement to be filed with the Securities and Exchange Commission in connection with our 2022 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, 2021 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 substantive amendments to the Code of Conduct or grant any waiver from a provision of the Code of Conduct to any executive officer or director 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 set forth in our 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 set forth in our 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 set forth in our Definitive Proxy Statement and is incorporated herein by reference.

Item 14. Principal Accountant Fees and Services.

The information required by this item will be set forth in our Definitive Proxy Statement and is incorporated here 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 November 16, 2021, by and between the Registrant and Janssen Biotech, Inc. (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on November 19, 2021).
3.1	Amended and Restated Certificate of Incorporation, as currently in effect (filed as 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 (filed as 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 (filed as 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	Investors' Rights Agreement, dated November 5, 2018 (filed as Exhibit 4.2 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).
4.3	Description of Common Stock of the Registrant (filed as Exhibit 4.3 to the Registrant's Annual Report on Form 10-K, filed with the SEC on March 12, 2020, and incorporated by reference herein).
10.1+	Mirum Pharmaceuticals, Inc. 2018 Equity Incentive Plan (filed as 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).
10.2+	Forms of grant notice, stock option agreement and notice of exercise under the Mirum Pharmaceuticals, Inc. 2018 Equity Incentive Plan (filed as 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).
10.3+	Mirum Pharmaceuticals, Inc. 2019 Equity Incentive Plan (filed as 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).
10.4+	Forms of grant notice, stock option agreement and notice of exercise under the Mirum Pharmaceuticals, Inc. 2019 Equity Incentive Plan (filed as 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).
10.5+	Forms of restricted stock unit grant notice and award agreement under the Mirum Pharmaceuticals, Inc. 2019 Equity Incentive Plan (filed as 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).
10.6*	Forms of international grant notice, stock option agreement and notice of exercise under the Mirum Pharmaceuticals, Inc. 2019 Equity Incentive Plan
10.7*	Forms of international director grant notice, stock option agreement and notice of exercise under the Mirum Pharmaceuticals, Inc. 2019 Equity Incentive Plan
10.8*	Forms of international restricted stock unit grant notice and award agreement under the Mirum Pharmaceuticals, Inc. 2019 Equity Incentive Plan
10.9+	Mirum Pharmaceuticals, Inc. 2019 Employee Stock Purchase Plan (filed as 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).
10.10*	Mirum Pharmaceuticals, Inc. 2019 Employee Stock Purchase Plan Terms and conditions – Non-U.S. Participants
10.11+	Mirum Pharmaceuticals, Inc. 2020 Inducement Plan (filed as Exhibit 10.1 to the Registrant's From 10-Q, filed with the SEC on May 7, 2020, and incorporated by reference herein).
10.12+	Forms of grant notice, stock option agreement and notice of exercise under the Mirum Pharmaceuticals, Inc. 2020 Inducement Plan (filed as Exhibit 10.2 to the Registrant's From 10-Q, filed with the SEC on May 7, 2020, and incorporated by reference herein).

10.13+	Forms of restricted stock unit grant notice and award agreement under the Mirum Pharmaceuticals, Inc. 2020 Inducement Plan (filed as Exhibit 10.3 to the Registrant's From 10-Q, filed with the SEC on May 7, 2020, and incorporated by reference herein).
10.14*	Forms of international grant notice, stock option agreement and notice of exercise under the Mirum Pharmaceuticals, Inc. 2020 Inducement Plan
10.15*	Forms of international restricted stock unit grant notice and award agreement under the Mirum Pharmaceuticals, Inc. 2020 Inducement Plan
10.16+	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).
10.17+	Mirum Pharmaceuticals, Inc. Severance Benefit Plan and form of Participation Agreement thereunder (filed as Exhibit 10.8 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).
10.18+	Amended and Restated Offer Letter by and between the Registrant and Michael Grey, dated May 15, 2019, as amended by Letter Agreement by and between the Registrant and Michael Grey, dated December 24, 2019 (filed as Exhibit 10.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on December 27, 2019, and incorporated by reference herein).
10.19+	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).
10.20+	Offer Letter by and between the Registrant and Pamela Vig, Ph.D., dated December 1, 2018 (filed as Exhibit 10.11 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).
10.21+	Offer Letter by and between the Registrant and Lara Longpre, dated December 1, 2018 (filed as Exhibit 10.12 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).
10.22+	Offer Letter by and between the Registrant and Ian Clements, Ph.D., dated March 4, 2019 (filed as Exhibit 10.13 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).
10.23+	Offer Letter by and between the Registrant and Peter Radovich, dated April 28, 2020 (filed as 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).
10.24#	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).
10.25#	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).
10.26#	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).
10.27#	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).
10.28#	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).

10.29#	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).
10.30	Office Lease by and between the Registrant and Hudson Metro Center, LLC, dated January 22, 2019, as amended by First Amendment by and between the Registrant and Hudson Metro Center, LLC, dated June 1, 2019, and as further amended by Second Amendment by and between the Registrant and Hudson Metro Center, LLC, dated November 22, 2019 (filed as Exhibit 10.15 to the Registrant's Registration Statement on Form S-1 (File No. 333-235825) filed with the SEC on January 6, 2020, and incorporated by reference herein).
10.31	Sales Agreement by and between the Registrant and SVB Leerink LLC, dated August 3, 2020 (incorporated by reference to Exhibit 1.2 to the Registrant's Registration Statement on Form S-3, as amended (File No. 333-240290), filed with the SEC on August 3, 2020, and incorporated by reference herein).
10.32	Revenue Interest Purchase Agreement by and among the Registrant, the Purchasers from time to time party thereto and Mulholland SA LLC, dated December 8, 2020 (incorporated by reference to Exhibit 99.1 to the Registrant's Current Report on Form 8-K, as filed with the SEC on December 10, 2020, and incorporated by reference herein).
10.33	Amendment No. 1 to Revenue Interest Purchase Agreement, dated September 28, 2021, by and among the Registrant, Mulholland SA LLC and the Purchasers (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on September 29, 2021).
10.34	Common Stock Purchase Agreement by and among the Registrant, TPC Investments II LP, TPC Investments Solutions LP and TPC Investments Solutions Co-Invest LP, dated December 8, 2020 (incorporated by reference to Exhibit 99.2 to the Registrant's Current Report on Form 8-K, as filed with the SEC on December 10, 2020, and incorporated by reference herein).
10.35*	Mirum Pharmaceuticals, Inc. Non-Employee Director Compensation Policy, as amended March 18, 2020
21.1	Subsidiaries of the Registrant (filed as Exhibit 21.1 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).
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.
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.
# Certain portions have been omitted in accordance with 17 CFR § 229.601(b).

¥ 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: March 9, 2022

By: /s/ Christopher Peetz
Christopher Peetz
President, 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 and Ian Clements, Ph.D. 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	President, Chief Executive Officer and Director (Principal Executive Officer)	March 9, 2022
/s/ Ian Clements, Ph.D. Ian Clements, Ph.D.	Chief Financial Officer (Principal Financial and Accounting Officer)	March 9, 2022
/s/ Michael Grey Michael Grey	Director	March 9, 2022
/s/ Carol L. Brosgart, M.D. Carol L. Brosgart, M.D.	Director	March 9, 2022
/s/ Laura Brege Laura Brege	Director	March 9, 2022
/s/ Laurent Fischer, M.D. Laurent Fischer, M.D.	Director	March 9, 2022
/s/ Patrick Heron Patrick Heron	Director	March 9, 2022
/s/ Edward T. Mathers Edward T. Mathers	Director	March 9, 2022
/s/ Niall O'Donnell, Ph.D. Niall O'Donnell, Ph.D.	Director	March 9, 2022
/s/ William C. Fairey William C. Fairey	Director	March 9, 2022

Exhibit 10.6

MIRUM PHARMACEUTICALS, INC.

STANDARD STOCK OPTION GRANT NOTICE - INTERNATIONAL
(2019 EQUITY INCENTIVE PLAN)

Mirum Pharmaceuticals, Inc. (the "***Company***"), pursuant to its 2019 Equity Incentive Plan (the "***Plan***"), hereby grants to Optionholder an option to purchase the number of shares of the Company's Common Stock set forth below. This option is subject to all of the terms and conditions as set forth in this Stock Option Grant Notice, in the Option Agreement, the Plan and the Notice of Exercise, all of which are attached hereto and incorporated herein in their entirety, including any special terms and conditions for the Optionholder's country set forth in the attached appendix (the "***Appendix***"). Capitalized terms not explicitly defined herein but defined in the Plan or the Option Agreement will have the same definitions as in the Plan or the Option Agreement. If there is any conflict between the terms in this Stock Option Grant Notice and the Plan, the terms of the Plan will control.

Optionholder:
Date of Grant:
Vesting Commencement Date:
Number of Shares Subject to Option:
Exercise Price (Per Share):
Total Exercise Price:
Expiration Date:

Type of Grant: Nonstatutory Stock Option

Exercise Schedule: [Same as Vesting Schedule]

Vesting Schedule: **[**_____, subject to Optionholder's Continuous Service as of each such date.]

Payment: By one or a combination of the following items (described in the Option Agreement):
☐ By cash, check, bank draft or money order payable to the Company
☐ Pursuant to a Regulation T Program if the shares are publicly traded
☐ By delivery of already-owned shares if the shares are publicly traded
☐ Subject to the Company's consent at the time of exercise, by a "net exercise" arrangement

Additional Terms/Acknowledgements: Optionholder acknowledges receipt of, and understands and agrees to, this Stock Option Grant Notice, the Option Agreement (including the Appendix) and the Plan. Optionholder acknowledges and agrees that this Stock Option Grant Notice and the Option Agreement (including the Appendix) may not be modified, amended or revised except as provided in the Plan. Optionholder further acknowledges that as of the Date of Grant, this Stock Option Grant Notice, the Option Agreement (including the Appendix), and the Plan set forth the entire understanding between Optionholder and the Company regarding this option award and supersede all prior oral and written agreements, promises and/or representations on that subject with the exception of, if applicable, (i) equity awards previously granted and delivered to Optionholder, (ii) any compensation recovery policy that is adopted by the Company or is otherwise required by applicable law and (iii) any written employment agreement, severance agreement, offer letter or other written agreement entered into between the Company and Participant

specifying the terms that should govern this specific option. By accepting this option, Optionholder consents to receive such documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

MIRUM PHARMACEUTICALS, INC.	**OPTIONHOLDER:**
By:	
Signature	Signature
Title:	Date:
Date:	

ATTACHMENTS: Option Agreement (including the Appendix), 2019 Equity Incentive Plan and Notice of Exercise

ATTACHMENT I

MIRUM PHARMACEUTICALS, INC.

OPTION AGREEMENT - INTERNATIONAL
(2019 EQUITY INCENTIVE PLAN)
(NONSTATUTORY STOCK OPTION)

Pursuant to your Stock Option Grant Notice ("***Grant Notice***") and this Option Agreement, including any special terms and conditions for your country set forth in the appendix attached hereto (the "***Appendix***") Mirum Pharmaceuticals, Inc. (the "***Company***") has granted you an option under its 2019 Equity Incentive Plan (the "***Plan***") to purchase the number of shares of the Company's Common Stock indicated in your Grant Notice at the exercise price indicated in your Grant Notice. The option is granted to you effective as of the date of grant set forth in the Grant Notice (the "***Date of Grant***"). If there is any conflict between the terms in this Option Agreement and the Plan, the terms of the Plan will control. Capitalized terms not explicitly defined in this Option Agreement or in the Grant Notice but defined in the Plan will have the same definitions as in the Plan.

The details of your option, in addition to those set forth in the Grant Notice and the Plan, are as follows:

1.VESTING. Subject to the provisions contained herein, your option will vest as provided in your Grant Notice. Vesting will cease

2.NUMBER OF SHARES AND EXERCISE PRICE. The number of shares of Common Stock subject to your option and your exercise

3.METHOD OF PAYMENT. You must pay the full amount of the exercise price for the shares you wish to exercise. You may pay the exercise price in cash or by check, bank draft or money order payable to the Company or in any other manner ***permitted by your Grant Notice,*** which may include one or more of the following:

(a) Provided that at the time of exercise the Common Stock is publicly traded, pursuant to a program developed under

(b) Provided that at the time of exercise the Common Stock is publicly traded, by delivery to the Company (either by actual

Common Stock in a form approved by the Company. You may not exercise your option by delivery to the Company of Common Stock if doing so would violate the provisions of any law, regulation or agreement restricting the redemption of the Company's stock.

(c) Subject to the consent of the Company at the time of exercise, by a "net exercise" arrangement pursuant to which the

your option and will not be exercisable thereafter if those shares (i) are used to pay the exercise price pursuant to the "net exercise," (ii) are delivered to you as a result of such exercise, and (iii) are withheld to satisfy your tax and/or social security withholding obligations.

4. WHOLE SHARES. You may exercise your option only for whole shares of Common Stock.

5. SECURITIES LAW COMPLIANCE. In no event may you exercise your option unless the shares of Common Stock issuable upon

exercise would not be in material compliance with such laws and regulations (including any restrictions on exercise required for compliance with Treas. Reg. 1.401(k)-1(d)(3), if applicable).

6. TERM. You may not exercise your option before the Date of Grant or after the expiration of the option's term. The term of your

(a) immediately upon the termination of your Continuous Service for Cause;

(b) three (3) months after the termination of your Continuous Service for any reason other than Cause, your Disability or your death (except as otherwise provided in Section 6(d) below); *provided, however,* that if during any part of such three (3) month period

the termination of your Continuous Service; *provided further,* if during any part of such three (3) month period, the sale of any Common Stock received upon exercise of your option would violate the Company's insider trading policy, then your option will not expire until the earlier of the Expiration Date or until it has been exercisable for an aggregate period of three (3) months after the termination of your Continuous Service during which the sale of the Common Stock received upon exercise of your option would not be in violation of the

(c) twelve (12) months after the termination of your Continuous Service due to your Disability (except as otherwise

(d) eighteen (18) months after your death if you die either during your Continuous Service or within three (3) months after

(e) the Expiration Date indicated in your Grant Notice; or

(f) the day before the tenth (10th) anniversary of the Date of Grant.

7.EXERCISE.

(a) You may exercise the vested portion of your option (and the unvested portion of your option if your Grant Notice so

additional documents as the Company may then require.

(b) By exercising your option you agree that, as a condition to any exercise of your option, the Company may require you

8.TRANSFERABILITY. Your option is not transferable, except by will or by the laws of descent and distribution, and is exercisable

9.OPTION NOT A SERVICE CONTRACT. Your option is not an employment or service contract, and nothing in your option will be

Consultant for the Company or an Affiliate. By accepting your option you acknowledge, understand and agree that:

(a)the Plan is established voluntarily by the Company, it is discretionary in nature, and may be amended, suspended or

(b)the grant of your option is voluntary and occasional and does not create any contractual or other right to receive future

(c)your option and any shares of Common Stock acquired under the Plan on exercise of your option, and the income and

(d)the future value of the shares of Common Stock underlying the option is unknown, indeterminable, and cannot be

(e)neither the Company nor any Affiliate shall be liable for any foreign exchange rate fluctuation between your local

(f)for purposes of the option, your Continuous Service will be considered terminated as of the date you are no longer

of the reason for such termination and whether or not later found to be invalid or in breach of employment laws in the jurisdiction where you

any notice period or any period of "garden leave" or similar period mandated under employment laws in the jurisdiction where you are employed or the terms of your employment agreement, if any); and the Plan Administrator shall have the exclusive discretion to determine when you are no longer actively providing services for purposes of the option (including whether you may still be considered to be providing services while on a leave of absence);

(g) no claim or entitlement to compensation or damages shall arise from forfeiture of this option resulting from the

waive your ability, if any, to bring any such claim, and release the Company and any Affiliate from any such claim; if, notwithstanding the foregoing, any such claim is allowed by a court of competent jurisdiction, then, by participating in the Plan, you shall be deemed irrevocably to have agreed not to pursue such claim and agree to execute any and all documents necessary to request dismissal or withdrawal of such claim.

10. WITHHOLDING OBLIGATIONS.

(a) At the time you exercise your option, in whole or in part, and at any time thereafter as requested by the Company, you

withholding obligations of the Company or an Affiliate, if any, which arise in connection with the exercise of your option.

(b) If this option is a Nonstatutory Stock Option, then upon your request and subject to approval by the Company, and

withheld by law (or such lower amount as may be necessary to avoid classification of your option as a liability for financial accounting purposes). Notwithstanding the filing of such election, shares of Common Stock shall be withheld solely from fully vested shares of Common Stock determined as of the date of exercise of your option that are otherwise issuable to you upon such exercise. Any adverse consequences to you arising in connection with such share withholding procedure shall be your sole responsibility.

(c) You may not exercise your option unless the tax and/or social security withholding obligations of the Company and/or

11.TAX CONSEQUENCES. You hereby agree that the Company does not have a duty to design or administer the Plan or its other

12.NOTICES. Any notices provided for in your option or the Plan will be given in writing (including electronically) and will be

participate in the Plan by electronic means. By accepting this option, you consent to receive such documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

13.GOVERNING PLAN DOCUMENT. Your option is subject to all the provisions of the Plan, the provisions of which are hereby made

recoupment in accordance with The Dodd–Frank Wall Street Reform and Consumer Protection Act and any implementing regulations thereunder, any clawback policy adopted by the Company and any compensation recovery policy otherwise required by applicable law.

14.OTHER DOCUMENTS. You hereby acknowledge receipt of and the right to receive a document providing the information

15.EFFECT ON OTHER EMPLOYEE BENEFIT PLANS. The value of this option will not be included as compensation, earnings,

16.VOTING RIGHTS. You will not have voting or any other rights as a stockholder of the Company with respect to the shares to be

17.SEVERABILITY. If all or any part of this Option Agreement or the Plan is declared by any court or governmental authority to be

lawful and valid.

18.MISCELLANEOUS.

(a) The rights and obligations of the Company under your option will be transferable to any one or more persons or entities,

(b) You agree upon request to execute any further documents or instruments necessary or desirable in the sole

(c) You acknowledge and agree that you have reviewed your option in its entirety, have had an opportunity to obtain the

(d) This Option Agreement will be subject to all applicable laws, rules, and regulations, and to such approvals by any

(e) All obligations of the Company under the Plan and this Option Agreement will be binding on any successor to the

19.PERSONAL DATA. The Company will collect and process information relating to you in accordance with the privacy notice from

20.LANGUAGE. If you have received this Agreement, or any other document related to this Option and/or the Plan translated into a

21.INSIDER TRADING/MARKET ABUSE. You acknowledge that, depending on your country, you may be subject to insider trading

imposed under any applicable Company insider trading policy. You acknowledge that it is your responsibility to comply with any applicable restrictions, and you are advised to speak to your personal advisor on this matter.

22.FOREIGN ASSET/ACCOUNT, EXCHANGE CONTROL AND TAX REPORTING. You may be subject to foreign asset/account,

report such accounts, assets and balances therein, the value thereof and/or the transactions related thereto to the applicable authorities in such country. You may also be required to repatriate sale proceeds or other funds received as a result of your participation in the Plan to your country through a designated bank or broker within a certain time after receipt. You acknowledge that it is your responsibility to be compliant with such regulations and you are encouraged to consult with your personal legal advisor for any details.

23.IMPOSITION OF OTHER REQUIREMENTS. The Company reserves the right to impose other requirements on your participation in

24.APPENDIX. Notwithstanding any provisions in this Option Agreement, your Option shall be subject to the special terms and

* * *

This Option Agreement (including the Appendix) will be deemed to be signed by you upon the signing by you of the Stock Option Grant Notice to which it is attached.

Appendix

This Appendix includes special terms and conditions that govern the Option granted to you under the Plan if you reside and/or work in any country listed below.

The information contained herein is general in nature and may not apply to your particular situation, and you are advised to seek appropriate professional advice as to how the relevant laws in your country may apply to your situation.

SWITZERLAND

Language Acknowledgement.

You confirm having read and understood the documents relating to the Plan, including the Option Agreement, with all terms and conditions included therein, which were provided in the English language only. You confirm that you have sufficient language capabilities to understand these terms and conditions in full.

Du bestätigst, dass du den Plan sowie die dazugehörigen Dokumente, inklusive der Vereinbarung, mit all den darin enthaltenen Bedingungen und Voraussetzungen, welche in englischer Sprache verfasst sind, gelesen und verstanden hast. Du bestätigst, dass Deine Sprachkenntnisse genügend sind, um diese Bedingungen und Voraussetzungen vollumfänglich zu verstehen.

Securities Law Information.

YOU ACKNOWLEDGE THAT THE PLAN IS NOT INTENDED TO BE PUBLICLY OFFERED IN OR FROM SWITZERLAND. NEITHER THE AGREEMENT NOR ANY OTHER MATERIALS RELATING TO THE OPTION CONSTITUTES A PROSPECTUS AS SUCH TERM IS UNDERSTOOD PURSUANT TO ARTICLE 652A OF THE SWISS CODE OF OBLIGATIONS, AND NEITHER THE AGREEMENT NOR ANY OTHER MATERIALS RELATING TO THE OPTION MAY BE PUBLICLY DISTRIBUTED NOR OTHERWISE MADE PUBLICLY AVAILABLE IN SWITZERLAND.

ATTACHMENT II

2019 EQUITY INCENTIVE PLAN

ATTACHMENT III

NOTICE OF EXERCISE

MIRUM PHARMACEUTICALS, INC.

Date of Exercise: _____

This constitutes notice to Mirum Pharmaceuticals, Inc. (the "***Company***") under my stock option that I elect to purchase the below number of shares of Common Stock of the Company (the "***Shares***") for the price set forth below.

Type of option:	Nonstatutory	
Stock option dated:		
Number of Shares as to which option is exercised:		
Certificates to be issued in name of:		
Total exercise price:	US$	
Cash payment delivered herewith:	US$	
[Value of _____ Shares delivered herewith[1]:	US$	_____]
[Value of _____ Shares pursuant to net exercise[2]:	US$	_____]
[Regulation T Program (cashless exercise[3]):	US$	_____]

[1] Shares must meet the public trading requirements set forth in the option. Shares must be valued in accordance with the terms of the option being exercised, and must be owned free and clear of any liens, claims, encumbrances or security interests. Certificates must be endorsed or accompanied by an executed assignment separate from certificate.
[2] The option must be a Nonstatutory Stock Option, and the Company must have established net exercise procedures at the time of exercise, in order to utilize this payment method.
[3] Shares must meet the public trading requirements set forth in the option.

By this exercise, I agree (i) to provide such additional documents as you may require pursuant to the terms of the Mirum Pharmaceuticals, Inc. 2019 Equity Incentive Plan, and (ii) to provide for the payment by me to you (in the manner designated by you) of the withholding obligation, if any, relating to the exercise of this option.

Very truly yours,

Exhibit 10.7

International - director

MIRUM PHARMACEUTICALS, INC.

DIRECTOR STOCK OPTION GRANT NOTICE - INTERNATIONAL
(2019 EQUITY INCENTIVE PLAN)

Mirum Pharmaceuticals, Inc. (the "***Company***"), pursuant to its 2019 Equity Incentive Plan (the "***Plan***"), hereby grants to Optionholder an option to purchase the number of shares of the Company's Common Stock set forth below. This option is subject to all of the terms and conditions as set forth in this Stock Option Grant Notice, in the Option Agreement, the Plan and the Notice of Exercise, all of which are attached hereto and incorporated herein in their entirety, including any special terms and conditions for the Optionholder's country set forth in the attached appendix (the "***Appendix***"). Capitalized terms not explicitly defined herein but defined in the Plan or the Option Agreement will have the same definitions as in the Plan or the Option Agreement. If there is any conflict between the terms in this Stock Option Grant Notice and the Plan, the terms of the Plan will control.

Optionholder:
Date of Grant:
Vesting Commencement Date:
Number of Shares Subject to Option:
Exercise Price (Per Share) (US$):
Total Exercise Price (US$):
Expiration Date:

Type of Grant: Nonstatutory Stock Option

Exercise Schedule: [Same as Vesting Schedule]

Vesting Schedule: **[**_____, subject to Optionholder's Continuous Service as of each such date.]

Payment: By one or a combination of the following items (described in the Option Agreement):

☐ By cash, check, bank draft or money order payable to the Company
☐ Pursuant to a Regulation T Program if the shares are publicly traded
☐ By delivery of already-owned shares if the shares are publicly traded
☐ Subject to the Company's consent at the time of exercise, by a "net exercise" arrangement

Additional Terms/Acknowledgements: Optionholder acknowledges receipt of, and understands and agrees to, this Stock Option Grant Notice, the Option Agreement (including the Appendix) and the Plan. Optionholder acknowledges and agrees that this Stock Option Grant Notice and the Option Agreement (including the Appendix) may not be modified, amended or revised except as provided in the Plan. Optionholder further acknowledges that as of the Date of Grant, this Stock Option Grant Notice, the Option Agreement (including the Appendix), and the Plan set forth the entire understanding between Optionholder and the Company regarding this option award and supersede all prior oral and written agreements, promises and/or representations on that subject with the exception of, if applicable, (i) equity awards previously granted and delivered to Optionholder, (ii) any compensation recovery policy that is adopted by the Company or is otherwise required by applicable law and (iii) any written employment agreement, severance agreement, offer letter or other written agreement entered into between the Company and Participant specifying the terms that should govern this specific option. By accepting this option, Optionholder consents to receive such documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

MIRUM PHARMACEUTICALS, INC.	**OPTIONHOLDER:**
By:	
Signature	Signature
Title:	Date:
Date:	

ATTACHMENTS: Option Agreement (including the Appendix), 2019 Equity Incentive Plan and Notice of Exercise

ATTACHMENT I
MIRUM PHARMACEUTICALS, INC.

OPTION AGREEMENT - INTERNATIONAL
(2019 EQUITY INCENTIVE PLAN)
(NONSTATUTORY STOCK OPTION)

Pursuant to your Stock Option Grant Notice ("***Grant Notice***") and this Option Agreement, including any special terms and conditions for your country set forth in the appendix attached hereto (the "***Appendix***") Mirum Pharmaceuticals, Inc. (the "***Company***") has granted you an option under its 2019 Equity Incentive Plan (the "***Plan***") to purchase the number of shares of the Company's Common Stock indicated in your Grant Notice at the exercise price indicated in your Grant Notice. The option is granted to you effective as of the date of grant set forth in the Grant Notice (the "***Date of Grant***"). If there is any conflict between the terms in this Option Agreement and the Plan, the terms of the Plan will control. Capitalized terms not explicitly defined in this Option Agreement or in the Grant Notice but defined in the Plan will have the same definitions as in the Plan.

The details of your option, in addition to those set forth in the Grant Notice and the Plan, are as follows:

1. VESTING.

(a) Subject to the provisions contained herein, your option will vest as provided in your Grant Notice. Vesting will cease upon the termination of your Continuous Service. Notwithstanding the foregoing, if a Change in Control occurs and your Continuous Service has not terminated as of immediately prior to such Change in Control, the vesting and exercisability of your option will be accelerated in full.

(b) If any payment or benefit you would receive from the Company or otherwise in connection with a Change in Control or other similar transaction (a "***280G Payment***") would (i) constitute a "parachute payment" within the meaning of Section 280G of the Code, and (ii) but for this sentence, be subject to the excise tax imposed by Section 4999 of the Code (the "***Excise Tax***"), then any such 280G Payment (a "***Payment***") shall be equal to the Reduced Amount. The "***Reduced Amount***" shall be either (x) the largest portion of the Payment that would result in no portion of the Payment (after reduction) being subject to the Excise Tax or (y) the largest portion, up to and including the total, of the Payment, whichever amount (i.e., the amount determined by clause (x) or by clause (y)), after taking into account all applicable federal, state and local employment taxes and social security, income taxes, and the Excise Tax (all computed at the highest applicable marginal rate), results in your receipt, on an after-tax basis, of the greater economic benefit notwithstanding that all or some portion of the Payment may be subject to the Excise Tax. If a reduction in a Payment is required pursuant to the preceding sentence and the Reduced Amount is determined pursuant to clause (x) of the preceding sentence, the reduction shall occur in the manner (the "***Reduction Method***") that results in the greatest economic benefit for you. If more than one method of reduction will result in the same economic benefit, the items so reduced will be reduced pro rata (the "***Pro Rata Reduction Method***").

Notwithstanding the foregoing, if you are subject to taxation in the United States and the Reduction Method or the Pro Rata Reduction Method would result in any portion of the Payment being subject to taxes pursuant to Section 409A of the Code that would not otherwise be subject to taxes pursuant to Section 409A of the Code, then the Reduction Method and/or the Pro Rata Reduction Method, as the case may be, shall be modified so as to avoid the imposition of taxes pursuant to Section 409A of the Code as follows: (A) as a first priority, the modification shall preserve to the greatest extent possible, the greatest economic benefit for you as determined on an after-tax basis; (B) as a second priority, Payments that are contingent

on future events (e.g., being terminated without cause), shall be reduced (or eliminated) before Payments that are not contingent on future events; and (C) as a third priority, Payments that are "deferred compensation" within the meaning of Section 409A of the Code shall be reduced (or eliminated) before Payments that are not deferred compensation within the meaning of Section 409A of the Code.

Unless you and the Company agree on an alternative accounting firm, the accounting firm engaged by the Company for general tax compliance purposes as of the day prior to the effective date of the change of control transaction triggering the Payment shall perform the foregoing calculations. If the accounting firm so engaged by the Company is serving as accountant or auditor for the individual, entity or group effecting the change of control transaction, the Company shall appoint a nationally recognized accounting firm to make the determinations required hereunder. The Company shall bear all expenses with respect to the determinations by such accounting firm required to be made hereunder. The Company shall use commercially reasonable efforts to cause the accounting firm engaged to make the determinations hereunder to provide its calculations, together with detailed supporting documentation, to you and the Company within fifteen (15) calendar days after the date on which your right to a 280G Payment becomes reasonably likely to occur (if requested at that time by you or the Company) or such other time as requested by you or the Company.

If you receive a Payment for which the Reduced Amount was determined pursuant to clause (x) of the first paragraph of this Section 1(b) and the Internal Revenue Service determines thereafter that some portion of the Payment is subject to the Excise Tax, you shall promptly return to the Company a sufficient amount of the Payment (after reduction pursuant to clause (x) of the first paragraph of this Section 1(b) so that no portion of the remaining Payment is subject to the Excise Tax. For the avoidance of doubt, if the Reduced Amount was determined pursuant to clause (y) in the first paragraph of this Section 1(b), you shall have no obligation to return any portion of the Payment pursuant to the preceding sentence.

2. NUMBER OF SHARES AND EXERCISE PRICE. The number of shares of Common Stock subject to your option and your exercise price per share in your Grant Notice will be adjusted for Capitalization Adjustments.

3. METHOD OF PAYMENT. You must pay the full amount of the exercise price for the shares you wish to exercise. You may pay the exercise price in cash or by check, bank draft or money order payable to the Company or in any other manner ***permitted by your Grant Notice,*** which may include one or more of the following:

(a) Provided that at the time of exercise the Common Stock is publicly traded, pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board that, prior to the issuance of Common Stock, results in either the receipt of cash (or check) by the Company or the receipt of irrevocable instructions to pay the aggregate exercise price to the Company from the sales proceeds. This manner of payment is also known as a "broker-assisted exercise", "same day sale", or "sell to cover".

(b) Provided that at the time of exercise the Common Stock is publicly traded, by delivery to the Company (either by actual delivery or attestation) of already-owned shares of Common Stock that are owned free and clear of any liens, claims, encumbrances or security interests, and that are valued at Fair Market Value on the date of exercise. "Delivery" for these purposes, in the sole discretion of the Company at the time you exercise your option, will include delivery to the Company of your attestation of ownership of such shares of Common Stock in a form approved by the Company. You may not exercise your option by delivery to the Company of Common Stock if doing so would violate the provisions of any law, regulation or agreement restricting the redemption of the Company's stock.

(c) Subject to the consent of the Company at the time of exercise, by a "net exercise" arrangement pursuant to which the Company will reduce the number of shares of Common Stock issued upon exercise of your option by the largest whole number of shares with a Fair Market Value that does not exceed the aggregate exercise price. You must pay any remaining balance of the aggregate exercise price not satisfied by the "net exercise" in cash or other permitted form of payment. Shares of Common Stock will no longer be outstanding under your option and will not be exercisable thereafter if those shares (i) are used to pay the exercise price pursuant to the "net exercise," (ii) are delivered to you as a result of such exercise, and (iii) are withheld to satisfy your tax and/or social security withholding obligations.

4. WHOLE SHARES. You may exercise your option only for whole shares of Common Stock.

5. SECURITIES LAW COMPLIANCE. In no event may you exercise your option unless the shares of Common Stock issuable upon exercise are then registered under the Securities Act or, if not registered, the Company has determined that your exercise and the issuance of the shares would be exempt from the registration requirements of the Securities Act. The exercise of your option also must comply with all other applicable laws and regulations governing your option, and you may not exercise your option if the Company determines that such exercise would not be in material compliance with such laws and regulations (including any restrictions on exercise required for compliance with Treas. Reg. 1.401(k)-1(d)(3), if applicable).

6. TERM. You may not exercise your option before the Date of Grant or after the expiration of the option's term. The term of your option expires, subject to the provisions of Section 5(h) of the Plan, upon the earliest of the following:

(a) immediately upon the termination of your Continuous Service for Cause;

(b) three (3) months after the termination of your Continuous Service for any reason other than Cause, your Disability or your death (except as otherwise provided in Section 6(d) below); *provided, however,* that if during any part of such three (3) month period your option is not exercisable solely because of the condition set forth in the section above regarding "Securities Law Compliance," your option will not expire until the earlier of the Expiration Date or until it has been exercisable for an aggregate period of three (3) months after the termination of your Continuous Service; *provided further,* if during any part of such three (3) month period, the sale of any Common Stock received upon exercise of your option would violate the Company's insider trading policy, then your option will not expire until the earlier of the Expiration Date or until it has been exercisable for an aggregate period of three (3) months after the termination of your Continuous Service during which the sale of the Common Stock received upon exercise of your option would not be in violation of the Company's insider trading policy;

(c) twelve (12) months after the termination of your Continuous Service due to your Disability (except as otherwise provided in Section 6(d)) below;

(d) eighteen (18) months after your death if you die either during your Continuous Service or within three (3) months after your Continuous Service terminates for any reason other than Cause;

(e) the Expiration Date indicated in your Grant Notice; or

(f) the day before the tenth (10th) anniversary of the Date of Grant.

7. EXERCISE.

(a) You may exercise the vested portion of your option (and the unvested portion of your option if your Grant Notice so permits) during its term by (i) delivering a Notice of Exercise (in a form designated by the Company) or completing such other documents and/or procedures designated by the Company for exercise and (ii) paying the exercise price and any applicable withholding taxes and/or social security to the Company's Secretary, stock plan administrator, or such other person as the Company may designate, together with such additional documents as the Company may then require.

(b) By exercising your option you agree that, as a condition to any exercise of your option, the Company may require you to enter into an arrangement providing for the payment by you to the Company of any tax and/or social security withholding obligation of the Company or any Affiliate arising by reason of (i) the exercise of your option, (ii) the lapse of any substantial risk of forfeiture to which the shares of Common Stock are subject at the time of exercise, or (iii) the disposition of shares of Common Stock acquired upon such exercise.

8. TRANSFER ABILITY. Your option is not transferable, except by will or by the laws of descent and distribution, and is exercisable during your life only by you.

9. OPTION NOT A SERVICE CONTRACT. Your option is not an employment or service contract, and nothing in your option will be deemed to create in any way whatsoever any obligation on your part to continue in the employ of the Company or an Affiliate, or of the Company or an Affiliate to continue your employment. In addition, nothing in your option will obligate the Company or an Affiliate, their respective stockholders, boards of directors, officers or employees to continue any relationship that you might have as a Director or Consultant for the Company or an Affiliate. By accepting your option you acknowledge, understand and agree that:

(a) the Plan is established voluntarily by the Company, it is discretionary in nature, and may be amended, suspended or terminated by the Company at any time, to the extent permitted under the Plan;

(b) the grant of your option is voluntary and occasional and does not create any contractual or other right to receive future grants of options (whether on the same or different terms), or benefits in lieu of options, even if options have been granted in the past;

(c) your option and any shares of Common Stock acquired under the Plan on exercise of your option, and the income and value of same, are not part of normal or expected compensation for any purpose, including, without limitation, calculating any severance, resignation, termination, redundancy, dismissal, end-of-service payments, holiday pay, bonuses, long-service awards, pension or retirement or welfare benefits or similar payments;

(d) the future value of the shares of Common Stock underlying the option is unknown, indeterminable, and cannot be predicted with certainty;

(e) neither the Company nor any Affiliate shall be liable for any foreign exchange rate fluctuation between your local currency and the United States Dollar that may affect the value of your option or of any amounts due to you pursuant to the exercise of your option or the subsequent sale of any shares of Common Stock received;

(f) for purposes of the option, your Continuous Service will be considered terminated as of the date you are no longer actively providing services to the Company or one of its Affiliates (regardless of the reason for such termination and whether or not later found to be invalid or in breach of employment laws in the jurisdiction where you are employed or the terms of your employment agreement, if any), and

unless otherwise expressly provided in this Option Agreement or determined by the Company, (i) your right to vest in the option under the Plan, if any, and (ii) the period (if any) during which you may exercise the option after such termination of Continuous Service will terminate as of such date and in each instance will not be extended by any notice period or any period of "garden leave" or similar period mandated under employment laws in the jurisdiction where you are employed or the terms of your employment agreement, if any); and the Plan Administrator shall have the exclusive discretion to determine when you are no longer actively providing services for purposes of the option (including whether you may still be considered to be providing services while on a leave of absence);

(g) no claim or entitlement to compensation or damages shall arise from forfeiture of this option resulting from the termination of your Continuous Service (for any reason whatsoever, whether or not later found to be invalid or in breach of employment laws in the jurisdiction where you are employed or the terms of your employment or service agreement, if any), and in consideration of the grant of this option to which you are otherwise not entitled, you irrevocably agree never to institute any claim against the Company or any Affiliate, waive your ability, if any, to bring any such claim, and release the Company and any Affiliate from any such claim; if, notwithstanding the foregoing, any such claim is allowed by a court of competent jurisdiction, then, by participating in the Plan, you shall be deemed irrevocably to have agreed not to pursue such claim and agree to execute any and all documents necessary to request dismissal or withdrawal of such claim.

10. WITHHOLDING OBLIGATIONS.

(a) At the time you exercise your option, in whole or in part, and at any time thereafter as requested by the Company, you hereby authorize withholding from payroll and any other amounts payable to you, and otherwise agree to make adequate provision for (including by means of a "same day sale" pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board to the extent permitted by the Company), any sums required to satisfy the federal, state, local and foreign tax and/or social security withholding obligations of the Company or an Affiliate, if any, which arise in connection with the exercise of your option.

(b) Upon your request and subject to approval by the Company, and compliance with any applicable legal conditions or restrictions, the Company may withhold from fully vested shares of Common Stock otherwise issuable to you upon the exercise of your option a number of whole shares of Common Stock having a Fair Market Value, determined by the Company as of the date of exercise, not in excess of the maximum amount of tax and/or social security required to be withheld by law (or such lower amount as may be necessary to avoid classification of your option as a liability for financial accounting purposes). Notwithstanding the filing of such election, shares of Common Stock shall be withheld solely from fully vested shares of Common Stock determined as of the date of exercise of your option that are otherwise issuable to you upon such exercise. Any adverse consequences to you arising in connection with such share withholding procedure shall be your sole responsibility.

(c) You may not exercise your option unless the tax and/or social security withholding obligations of the Company and/or any Affiliate are satisfied. Accordingly, you may not be able to exercise your option when desired even though your option is vested, and the Company will have no obligation to issue a certificate for such shares of Common Stock or release such shares of Common Stock from any escrow provided for herein, if applicable, unless such obligations are satisfied.

11. TAX CONSEQUENCES. You hereby agree that the Company does not have a duty to design or administer the Plan or its other compensation programs in a manner that minimizes your tax and/or social security liabilities. You will not make any claim against the Company, or any of its Officers, Directors,

Employees or Affiliates related to tax or social security liabilities arising from your option or your other compensation.

12. NOTICES. Any notices provided for in your option or the Plan will be given in writing (including electronically) and will be deemed effectively given upon receipt or, in the case of notices delivered by mail by the Company to you, five (5) days after deposit in the national mail, postage prepaid, addressed to you at the last address you provided to the Company. The Company may, in its sole discretion, decide to deliver any documents related to participation in the Plan and this option by electronic means or to request your consent to participate in the Plan by electronic means. By accepting this option, you consent to receive such documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

13. GOVERNING PLAN DOCUMENT. Your option is subject to all the provisions of the Plan, the provisions of which are hereby made a part of your option, and is further subject to all interpretations, amendments, rules and regulations, which may from time to time be promulgated and adopted pursuant to the Plan. If there is any conflict between the provisions of your option and those of the Plan, the provisions of the Plan will control. In addition, your option (and any compensation paid or shares issued under your option) is subject to recoupment in accordance with The Dodd–Frank Wall Street Reform and Consumer Protection Act and any implementing regulations thereunder, any clawback policy adopted by the Company and any compensation recovery policy otherwise required by applicable law.

14. OTHER DOCUMENTS. You hereby acknowledge receipt of and the right to receive a document providing the information required by Rule 428(b)(1) promulgated under the Securities Act, which includes the Plan prospectus. In addition, you acknowledge receipt of the Company's policy permitting certain individuals to sell shares only during certain "window" periods and the Company's insider trading policy, in effect from time to time.

15. EFFECT ON OTHER EMPLOYEE BENEFIT PLANS. The value of this option will not be included as compensation, earnings, salaries, or other similar terms used when calculating your benefits under any employee benefit plan sponsored by the Company or any Affiliate, except as such plan otherwise expressly provides. The Company expressly reserves its rights to amend, modify, or terminate any of the Company's or any Affiliate's employee benefit plans.

16. VOTING RIGHTS. You will not have voting or any other rights as a stockholder of the Company with respect to the shares to be issued pursuant to this option until such shares are issued to you. Upon such issuance, you will obtain full voting and other rights as a stockholder of the Company. Nothing contained in this option, and no action taken pursuant to its provisions, will create or be construed to create a trust of any kind or a fiduciary relationship between you and the Company or any other person.

17. SEVERABILITY. If all or any part of this Option Agreement or the Plan is declared by any court or governmental authority to be unlawful or invalid, such unlawfulness or invalidity will not invalidate any portion of this Option Agreement or the Plan not declared to be unlawful or invalid. Any Section of this Option Agreement (or part of such a Section) so declared to be unlawful or invalid shall, if possible, be construed in a manner which will give effect to the terms of such Section or part of a Section to the fullest extent possible while remaining lawful and valid.

18. MISCELLANEOUS.

(a) The rights and obligations of the Company under your option will be transferable to any one or more persons or entities, and all covenants and agreements hereunder will inure to the benefit of, and be enforceable by the Company's successors and assigns.

(b) You agree upon request to execute any further documents or instruments necessary or desirable in the sole determination of the Company to carry out the purposes or intent of your option.

(c) You acknowledge and agree that you have reviewed your option in its entirety, have had an opportunity to obtain the advice of counsel prior to executing and accepting your option, and fully understand all provisions of your option.

(d) This Option Agreement will be subject to all applicable laws, rules, and regulations, and to such approvals by any governmental agencies or national securities exchanges as may be required.

(e) All obligations of the Company under the Plan and this Option Agreement will be binding on any successor to the Company, whether the existence of such successor is the result of a direct or indirect purchase, merger, consolidation, or otherwise, of all or substantially all of the business and/or assets of the Company.

19. DATA PRIVACY.

(a) You explicitly and unambiguously acknowledge and consent to the collection, use and transfer, in electronic or other form, of your personal data as described in this document by and among, as applicable, your employer, the Company and its Affiliates for the exclusive purpose of implementing, administering and managing your participation in the Plan. You understand that the Company, its Affiliates and your employer hold certain personal information about you, including, but not limited to, name, home address and telephone number, date of birth, social security number (or other identification number), salary, nationality, job title, any shares of stock or directorships held in the Company, details of all options or any other entitlement to shares of stock awarded, canceled, purchased, exercised, vested, unvested or outstanding in your favor for the purpose of implementing, managing and administering the Plan ("***Data***"). You understand that the Data may be transferred to any third parties assisting in the implementation, administration and management of the Plan, that these recipients may be located in your country or elsewhere, in particular in the US, and that the recipient country may have different data privacy laws providing less protections of your personal data than your country. You may request a list with the names and addresses of any potential recipients of the Data by contacting the stock plan administrator at the Company (the "***Stock Plan Administrator***"). You acknowledge that the recipients may receive, possess, process, use, retain and transfer the Data, in electronic or other form, for the purposes of implementing, administering and managing your participation in the Plan, including any requisite transfer of such Data, as may be required to a broker or other third party with whom you may elect to deposit any shares of Common Stock acquired upon the exercise of your option. You understand that Data will be held only as long as is necessary to implement, administer and manage your participation in the Plan. You may, at any time, view the Data, request additional information about the storage and processing of the Data, require any necessary amendments to the Data or refuse or withdraw the consents herein, in any case without cost, by contacting the Stock Plan Administrator in writing.

(b) For the purposes of operating the Plan in the European Union, Switzerland and the United Kingdom, the Company will collect and process information relating to you in accordance with the privacy notice from time to time in force.

20. LANGUAGE. You acknowledge that you are sufficiently proficient in the English language, or have consulted with an advisor who is sufficiently proficient in English, so as to allow you to understand

the terms and conditions of this Option Agreement. If you have received this Option Agreement, or any other document related to this Option and/or the Plan translated into a language other than English and if the meaning of the translated version is different than the English version, the English version will control.

21. Insider Trading/Market Abuse. You acknowledge that, depending on your country, you may be subject to insider trading restrictions and/or market abuse laws, which may affect your ability to acquire or sell the shares of Common Stock or rights to the shares of Common Stock under the Plan during such times as you are considered to have "inside information" regarding the Company (as defined by the laws in your country). Any restrictions under these laws or regulations are separate from and in addition to any restrictions that may be imposed under any applicable Company insider trading policy. You acknowledge that it is your responsibility to comply with any applicable restrictions, and you are advised to speak to your personal advisor on this matter.

22. Foreign Asset/Account, Exchange Control and Tax Reporting. You may be subject to foreign asset/account, exchange control and/or tax reporting requirements as a result of the acquisition, holding and/or transfer of shares of Common Stock or cash (including dividends and the proceeds arising from the sale of shares of Common Stock) derived from your participation in the Plan in, to and/or from a brokerage/bank account or legal entity located outside your country. The applicable laws in your country may require that you report such accounts, assets and balances therein, the value thereof and/or the transactions related thereto to the applicable authorities in such country. You may also be required to repatriate sale proceeds or other funds received as a result of your participation in the Plan to your country through a designated bank or broker within a certain time after receipt. You acknowledge that it is your responsibility to be compliant with such regulations and you are encouraged to consult with your personal legal advisor for any details.

23. Imposition of Other Requirements. The Company reserves the right to impose other requirements on your participation in the Plan, and on any shares of Common Stock acquired under the Plan, to the extent the Company determines it is necessary or advisable for legal or administrative reasons, and to require you to sign any additional agreements or undertakings that may be necessary to accomplish the foregoing.

24. Choice of Law. The interpretation, performance and enforcement of this Option Agreement shall be governed by the laws of the State of Delaware without regard to that state's conflicts of laws rules.

25. Appendix. Notwithstanding any provisions in this Option Agreement, your Option shall be subject to the special terms and conditions for your country set forth in the Appendix attached hereto. Moreover, if you relocate to one of the countries included therein, the terms and conditions for such country will apply to you to the extent the Company determines that the application of such terms and conditions is necessary or advisable for legal or administrative reasons. The Appendix constitutes part of this Option Agreement.

* * *

This Option Agreement (including the Appendix) will be deemed to be signed by you upon the signing by you of the Stock Option Grant Notice to which it is attached.

Appendix

This Appendix includes special terms and conditions that govern the Option granted to you under the Plan if you reside and/or work in any country listed below.

The information contained herein is general in nature and may not apply to your particular situation, and you are advised to seek appropriate professional advice as to how the relevant laws in your country may apply to your situation. If you are a citizen or resident of a country other than the one in which you are currently working and/or residing, transfer employment and/or residency to another country after the date of grant, are a consultant, change employment status to a consultant position, or are considered a resident of another country for local law purposes, the Company shall, in its discretion, determine the extent to which the special terms and conditions contained herein shall be applicable to you. References to your employer shall include any entity that engages your services.

DENMARK

Stock Option Act. You acknowledge that you received the Employer Statement in Danish which sets forth additional terms of the option to the extent the Danish Stock Options Act applies.

Foreign Asset / Account Reporting Notification. If you establish an account holding cash or shares of Common Stock outside Denmark, you must report the account to the Danish Tax Administration.

FRANCE

Language Consent. The parties to the Option Agreement acknowledge that it is their express wish that the Option Agreement, as well as all documents, notices, and legal proceedings entered into, given or instituted pursuant hereto or relating directly or indirectly hereto, be drawn up in English.

Consentement relatif à la langue utilisée

Les parties reconnaissent avoir exigé que cette convention («Agreement») soit rédigée en anglais, ainsi que tous les documents, avis et procédures judiciaires, éxécutés, donnés ou intentés en vertu de, ou liés directement ou indirectement à la présente.

Term. Section 6(a) of the Option Agreement is deleted and replaced with the following:

6. TERM. You may not exercise your option before the Date of Grant or after the expiration of the option's term. Except as set forth in your Grant Notice and as set forth below under (a) to (e), the term of your option expires, subject to the provisions of Section 5(h) of the Plan, immediately upon the termination of your Continuous Service.

By exception, the term of your option expires upon the earliest of the following:

(a) three months after the termination of your Continuous Service in case of retirement or redundancy for economic reasons (except as otherwise provided in Section 6(d) below); *provided, however,* that if during any part of such three month period your option is not exercisable solely because of the condition set forth in the section above relating to "Securities Law Compliance," your option will not expire until the earlier of the Expiration Date or until it has been exercisable for an aggregate period of three months after the termination of your Continuous Service; *provided further,* if during any part of such three (3) month period, the sale of any Common Stock received upon exercise of your option would violate the Company's insider trading policy, then your option will not expire until the earlier of the Expiration Date

or until it has been exercisable for an aggregate period of three (3) months after the termination of your Continuous Service during which the sale of the Common Stock received upon exercise of your option would not be in violation of the Company's insider trading policy;

(b) 12 months after the termination of your Continuous Service due to your Disability (except as otherwise provided in Section 6(d)) below;

(c) 18 months after your death if you die either during your Continuous Service or during the three month period referred to at Section 6(a) above;

(d) the Expiration Date indicated in your Grant Notice; or

(e) the day before the 10th anniversary of the Date of Grant.

Tax Reporting. The gain realized on the exercise of the options will be subject to income tax and reporting obligations: as a standard salary income, the exercise gain will be subject to social security contributions and income tax withholding by your employer (if any). Any income tax withheld by your employer is not final. Your final income tax will be assessed based on the annual income tax return you will file the year following the exercise of your options.

The gain realized upon the sale of the shares of Common Stock – if any - will also be subject to income tax and reporting obligation: you will be responsible for reporting the capital gain, if any, realized upon the sale of the shares, on the annual income tax return you will file the year following the sale of your shares. You will be liable to pay the corresponding tax to the French tax authorities.

Foreign Asset/Account Reporting Information. If you are a French resident and maintain a foreign bank account, you must report such account to the French tax authorities when filing your annual tax return. Failure to comply with this requirement could trigger significant penalties and you should consult with your personal advisor to ensure proper compliance with applicable reporting requirements in France.

Exchange Control Information. Cross-border payments towards or from another EU member in excess of €10,000 must be reported to the French Custom Authorities. However, this reporting obligation does not apply to wire transfers made via banks or financial institutions. So, given that the Plan is established by a US company and that, in any case, all money transfers will be made through banks or financial institutions, this reporting obligation should not apply here.

GERMANY

Term. Section 6(a) of the Option Agreement is deleted and replaced with the following:

> **6. TERM.** You may not exercise your Option before the commencement of its term or after its term expires. The term of your Option commences on the Date of Grant and expires upon the earliest of the following:
>
> **(a)** immediately upon the termination of your Continuous Service for Cause; this does not apply in case termination of your Continuous Service does not comply with the principle of good faith. In such case, the provisions set forth under the remainder of this Section 6 shall apply.

Securities Disclaimer. The participation in the Plan is exempt or excluded from the requirement to publish a prospectus under the EU Prospectus Directive as implemented in Germany.

Exchange Control Information. Cross-border payments in excess of €12,500 must be reported monthly to the German Federal Bank (*Bundesbank*). In the event that you make or receive a payment in excess of this amount, you are required to report the payment to Bundesbank electronically using the "General Statistics Reporting Portal" ("*Allgemeines Meldeportal Statistik*") available via Bundesbank's website (www.bundesbank.de).

Tax Reporting. You must report and pay any capital gains tax liability that arises in connection with the sale of shares acquired under the Plan. In general the statutory deadline of filing annual income tax returns for taxpayers is 31 July of the calendar year following the respective fiscal year. Payment periods of due tax amounts are determined in view of the competent tax office. You should consult with your personal tax advisor to ensure that you are properly complying with applicable reporting requirements in Germany.

ITALY

Stock Option Exercises. Due to regulatory requirements, notwithstanding Section 3 of the Option Agreement, you will be required to exercise the option using a cashless sell-all exercise method, pursuant to which all shares of Common Stock subject to the exercised option will be sold immediately upon exercise and the proceeds of sale, less the exercise price, any federal, state, local and foreign tax and/or social security withholding obligations of the Company or an Affiliate and broker's fees or commissions, will be remitted to you in cash in accordance with any applicable exchange control laws and regulations. You will not be permitted to hold shares after exercise. The Company reserves the right to provide additional methods of exercise depending on the development of local laws.

Plan Acknowledgement. You acknowledge that you have read and specifically and expressly approve the following sections of the Option Agreement: (9) Nature of Grant; (10) Withholding Obligations; (11) Tax Consequences; (19) Data Privacy; (20) Language; (23) Imposition of Other Requirements; and (24) Choice of Law; and the Italy country-specific terms and conditions of this Appendix. **Foreign Asset/Account Reporting Information**. If you are an Italian resident and, during any fiscal year, hold investments or financial assets outside of Italy (*e.g.*, cash, shares of Common Stock) which may generate income taxable in Italy (or if you are the beneficial owner of such an investment or asset even if you do not directly hold the investment or asset), you are required to report such investments or assets on your annual tax return for such fiscal year (on UNICO Form, RW Schedule, or on a special form if you are not required to file a tax return).

Foreign Financial Assets Tax. The fair market value of any shares of Common Stock held outside of Italy is subject to a foreign assets tax. Financial assets include shares of Common Stock acquired under the Plan. The taxable amount will be the fair market value of the financial assets assessed at the end of the calendar year. You should consult with your personal tax advisor about the foreign financial assets tax.

NETHERLANDS

Prohibition Against Insider Trading. You should be aware of the European insider trading rules, which may affect the sale of shares of Common Stock acquired under the Plan. In particular, you may be prohibited from effecting certain share transactions if you have insider information regarding the Company. If it is uncertain whether the insider rules apply, the Company recommends that you consult with a legal advisor. The Company cannot be held liable if you violate the insider trading rules applicable in the Netherlands. You are responsible for ensuring your compliance with these rules.

Under Article 14 of the Market Abuse Regulation ((EU) Regulation 596/2014), anyone who has "inside information" related to an issuing company is prohibited from effectuating a transaction in securities in or from the Netherlands. "Inside information" is defined as knowledge of specific information concerning the

issuing company to which the securities relate or the trade in securities issued by such company, which has not been made public and which, if published, would reasonably be expected to affect the price of the securities, regardless of the development of the price. The insider could be any employee of the Company or an Affiliate in the Netherlands who has inside information as described herein. Given the broad scope of the definition of inside information, certain participants may have inside information and thus are prohibited from making a transaction in securities in the Netherlands at a time when they have such inside information. By entering into this Option Agreement and participating in the Plan, you acknowledge having read and understood the notification above and acknowledge that it is your responsibility to comply with the Dutch insider trading rules, as discussed herein.

Securities Disclaimer. The grant of the Option is exempt or excluded from the requirement to publish a prospectus under the Prospectus Regulation ((EU) Regulation 2017/1129). The Options are not transferable and are not deemed to qualify as an offering of securities in the Netherlands within the meaning of the Prospectus Regulation. To the extent that a supervisory body would qualify the offering of Awards or its underlying securities as an offering of securities within the meaning of the Prospectus Regulation, such offering will only be made in reliance on Article 1(4) of the Prospectus Regulation provided that no such offering of securities shall require Mirum Pharmaceuticals, Inc. to publish a prospectus pursuant to Article 3 of the Prospectus Regulation.

SPAIN

Nature of Grant. The following provision supplements Section 9 of the Option Agreement:

In accepting the option, you consent to participate in the Plan and acknowledge that the Plan was made available to you and that you read a copy of the Plan and you consent to the terms and conditions of the Agreement and acknowledge having received and read a copy of the Option Agreement.

You understand and agree that, as a condition of the option grant, your termination of employment for any reason (including for the reasons listed below) will automatically result in the forfeiture of the option and loss of the shares of Common Stock that may have been granted to you and that have not vested as of the date of your termination of employment.

In particular, you understand and agree that the option will be forfeited without entitlement to the underlying shares of Common Stock or to any amount as indemnification in the event of your termination of employment prior to vesting by reason of, including, but not limited to: resignation, disciplinary dismissal adjudged to be with cause, disciplinary dismissal adjudged or recognized to be without good cause (i.e., subject to a "*despido improcedente*"), individual or collective layoff on objective grounds, adjudged or recognized to be with or without good cause, material modification of the terms of employment under Article 41 of the Workers' Statute, relocation under Article 40 of the Workers' Statute, Article 50 of the Workers' Statute, unilateral withdrawal by the Company, and under Article 10.3 of Royal Decree 1382/1985.

Furthermore, you understand that the Company has unilaterally, gratuitously and discretionally decided to grant the options under the Plan to employees of the Company. The decision is a limited decision that is entered into upon the express assumption and condition that any grant will not economically or otherwise bind the Company on an ongoing basis. Consequently, you understand that the option is granted on the assumption and condition that the option and the shares of Common Stock underlying the option shall not become a part of any employment or service contract with the option and shall not be considered a mandatory benefit, salary for any purposes (including severance compensation) or any other right whatsoever. In addition, you understand that the option would not be granted to you but for the assumptions and conditions referred to above; thus, you acknowledge and freely accept that should any or all of the

assumptions be mistaken or should any of the conditions not be met for any reason, then any option granted to you shall be null and void.

Securities Law Information. The option described in the Option Agreement does not qualify as a security under Spanish regulations. No "offer of securities to the public," within the meaning of Spanish law, has taken place or will take place in the Spanish territory. The Option Agreement and any other documents evidencing the option have not been, nor will they be, registered with the *Comisión Nacional del Mercado de Valores* (Spanish Securities Exchange Commission), and none of these documents constitutes a public offering prospectus.

Exchange Control Information. The acquisition, ownership and sale of shares of Common Stock under the Plan must be declared for statistical purposes to the Spanish *Dirección General de Comercio e Inversiones* (the "***DGCI***"), the Bureau for Commerce and Investments, which is a department of the Ministry of Economy and Competitiveness. You also must declare ownership of any shares of Common Stock as of December 31 of the prior year with the Directorate of Foreign Transactions each January. In addition, if the acquisition or sale of any shares of Common Stock exceeds certain thresholds, it must be declared to the DGCI within 1 month after the sale.

When receiving foreign currency payments derived from the ownership of shares of Common Stock (i.e., sale proceeds), you must inform the financial institution receiving the payment of the basis upon which such payment is made if the payment exceeds €50,000. You will need to provide the following information: (i) your name, address, and fiscal identification number; (ii) the name and corporate domicile of the Company; (iii) the amount of the payment and the currency used; (iv) the country of origin; (v) the reasons for the payment; and (vi) further information that may be required.

In addition, you may be required to declare electronically to the Bank of Spain any foreign accounts (including brokerage accounts held abroad), any foreign instruments (including any shares of Common Stock acquired under the Plan) and any transactions with non-Spanish residents (including any payments of shares of Common Stock made to you by the Company) depending on the value of such accounts and instruments and the amount of the transactions during the relevant year as of December 31 of the relevant year.

SWITZERLAND

Sole Contact and Contractual Partner Information. You acknowledge that the option, this Option Agreement including the Appendix and, the Annexes and your participation in the Plan do not create any claims against the employer, either directly or indirectly. Your sole contract and sole contractual partner regarding the Plan and the option is the Company and the option does not form part of your contractual compensation.

Continuous Service. Notwithstanding anything else in the Plan or the Option Agreement, the Continuous Service will be deemed to end on the date when a termination notice is issued, regardless of whether the cessation of the employment was lawful, and shall not include any period notice of termination of employment or any period of salary continuance or deemed employment. As a result, if you receive notice of termination your Continuous Service will end on the date you receive such notice.

Securities Law Information. The option grant is not intended to be publicly offered in or from Switzerland. Because it is considered a private offering, it is not subject to securities registration in Switzerland. Neither this document nor any other materials relating to the option (i) constitutes a prospectus as such term is understood pursuant to article 35 of the Swiss Federal Act on Financial Services (FinSa)), (ii) may be publicly distributed nor otherwise made publicly available in Switzerland or (iii) has been or

will be filed with, approved or supervised by any Swiss regulatory authority, including the Swiss Financial Market Supervisory Authority (FINMA).

Grant of the Option. The option is a voluntary gratuity (*Gratifikation; gratification*) within the meaning of Article 322d Swiss Code of Obligations (CO) as determined at the Company's sole discretion which you have no entitlement to and which does not constitute an entitlement for a grant of further options or other equities in the future.

Vesting. You acknowledge and confirm that the option grant is fully discretionary and that before any option has vested you shall not have any right in regard to such option.

Disability. For the avoidance of any doubt, "***Disability***" shall include, but not be limited to, any permanent disability as per the social security laws of Switzerland.

Social Security and Tax. You herewith directly authorize your employer to make all (if any) applicable social security, insurance and tax deductions resulting from the grant and/or vesting of the option or the sale of shares of Common Stock from any compensation owed to you by your employer, subject to any statutory limitations. If your compensation shall not be sufficient to cover such social security, insurance and tax liabilities, you will indemnify the employer upon first demand.

Cause. "***Cause***" shall include, but not be limited to, all reasons entitling to a summary dismissal pursuant to article 337 of the Swiss Code of Obligations (CO) and all justified reasons pursuant to article 340c para. 2 CO, without limiting the definition of Cause as outlined in the Option Agreement.

Language Acknowledgement. You confirm having read and understood the documents relating to the Plan, including the Option Agreement, with all terms and conditions included therein, which were provided in the English language only. You confirm that you have sufficient language capabilities to understand these terms and conditions in full.

Sie bestätigen, dass Sie den Plan sowie die dazugehörigen Dokumente, inklusive der Vereinbarung, mit all den darin enthaltenen Bedingungen und Voraussetzungen, welche in englischer Sprache verfasst sind, gelesen und verstanden haben. Sie bestätigen, dass Ihre Sprachkenntnisse genügend sind, um die Bedingungen und Voraussetzungen zu verstehen.

Vous confirmez que vous avez lu et compris les documents relatifs au plan, y compris la convention d'attribution, avec toutes les conditions qui y sont incluses, qui ont été fournies en langue anglaise uniquement. Vous confirmez que vous avez des capacités linguistiques suffisantes pour comprendre ces termes et conditions dans leur intégralité.

No Right against Employer. You expressly acknowledge that you shall not have any right or claim under this option grant or the Option Agreement against your employer. You expressly acknowledge and agree that you may only have any right and claim against the Company under this option grant and the Option Agreement.

Governing Law and Jurisdiction. You expressly acknowledge and agree to the Choice of Law clause in the Plan and the Option Agreement and accept that Swiss law does not apply and that Swiss courts do not have any jurisdiction in regard to any claims under the Plan or the Option Agreement.

UNITED KINGDOM

Option Not a Service Contract. The following supplements Section 9 of the Option Agreement:

You waive all rights to compensation or damages in consequence of the termination of your office or employment with the Company or any Affiliate for any reason whatsoever (whether lawful or unlawful and including, without prejudice to the foregoing, in circumstances giving rise to a claim for wrongful dismissal) in so far as those rights arise or may arise from you ceasing to hold or being able to vest your Option, or from the loss or diminution in value of any rights or entitlements in connection with the Plan.

Withholding Obligations. The following supplements Section 10 of the Option Agreement:

As a condition of the vesting of your Option, you unconditionally and irrevocably agree:

(i) to place the Company in funds and indemnify the Company in respect of (1) all liability to UK income tax which the Company is liable to account for on your behalf directly to HM Revenue & Customs; (2) all liability to national insurance contributions which the Company is liable to account for on your behalf to HM Revenue & Customs (including, to the extent permitted by law, secondary class 1 (employer's) national insurance contributions for which you are liable and hereby agree to bear); and (3) all liability to national insurance contributions for which the Company is liable and which are formally transferred to you, which arises as a consequence of or in connection with the exercise of your Option (the "***UK Tax Liability***"); or

(ii) to permit the Company to sell at the best price which it can reasonably obtain such number of shares of Common Stock allocated or allotted to you following exercise as will provide the Company with an amount equal to the UK Tax Liability; and to permit the Company to withhold an amount not exceeding the UK Tax Liability from any payment made to you (including, but not limited to salary); and

(iii) if so required by the Company, and, to the extent permitted by law, to enter into a joint election or other arrangements under which the liability for all or part of such employer's national insurance contributions liability is transferred to you; and

(iv) if so required by the Company, to enter into a joint election within Section 431 of (UK) Income Tax (Earnings and Pensions) Act 2003 ("***ITEPA***") in respect of computing any tax charge on the acquisition of "restricted securities" (as defined in Section 423 and 424 of ITEPA); and

(v) to sign, promptly, all documents required by the Company to effect the terms of this provision, and references in this provision to "the Company" shall, if applicable, be construed as also referring to any Affiliate.

Clawback/Recovery. By executing the Option Agreement, you expressly consent in writing to the application of the right of recoupment to your option in accordance with the terms of Section 8(l) of the Plan and Section 13 of the Option Agreement.

ATTACHMENT II

2019 EQUITY INCENTIVE PLAN

ATTACHMENT III

NOTICE OF EXERCISE - INTERNATIONAL

MIRUM PHARMACEUTICALS, INC.

Date of Exercise:

This constitutes notice to Mirum Pharmaceuticals, Inc. (the "***Company***") under my stock option that I elect to purchase the below number of shares of Common Stock of the Company (the "***Shares***") for the price set forth below.

Type of option:	Nonstatutory	
Stock option dated:		
Number of Shares as to which option is exercised:		
Certificates to be issued in name of:		
Total exercise price:	US $	
Cash payment delivered herewith:	US $	
[Value of _____ Shares delivered herewith[1]:	US $	]
[Value of _____ Shares pursuant to net exercise[2]:	US $	]
[Regulation T Program (cashless exercise[3]):	US $	]

[1] Shares must meet the public trading requirements set forth in the option. Shares must be valued in accordance with the terms of the option being exercised, and must be owned free and clear of any liens, claims, encumbrances or security interests. Certificates must be endorsed or accompanied by an executed assignment separate from certificate.

[2] The Company must have established net exercise procedures at the time of exercise, in order to utilize this payment method.

[3] Shares must meet the public trading requirements set forth in the option.

By this exercise, I agree (i) to provide such additional documents as you may require pursuant to the terms of the Mirum Pharmaceuticals, Inc. 2019 Equity Incentive Plan, Stock Option Grant Notice, Option Agreement and the Appendix thereto, and (ii) to provide for the payment by me (in the manner designated by you) of the withholding obligation, if any, relating to the exercise of this option.

Very truly yours,

Exhibit 10.8

International

MIRUM PHARMACEUTICALS, INC.

RESTRICTED STOCK UNIT GRANT NOTICE - INTERNATIONAL
(2019 EQUITY INCENTIVE PLAN)

Mirum Pharmaceuticals, Inc. (the "***Company***"), pursuant to its 2019 Equity Incentive Plan (the "***Plan***"), hereby awards to Participant a Restricted Stock Unit Award for the number of shares of the Company's Common Stock ("***Restricted Stock Units***") set forth below (the "***Award***"). The Award is subject to all of the terms and conditions as set forth in this notice of grant (this "***Restricted Stock Unit Grant Notice***"), and in the Plan and the Restricted Stock Unit Award Agreement (the "***Award Agreement***") (the definition of which shall include any special terms and conditions for the Participant's country set out in the attached appendix (the "***Appendix***")), both of which are attached hereto and incorporated herein in their entirety. Capitalized terms not explicitly defined herein shall have the meanings set forth in the Plan or the Award Agreement. In the event of any conflict between the terms in this Restricted Stock Unit Grant Notice or the Award Agreement and the Plan, the terms of the Plan shall control.

Participant:
Date of Grant:
Vesting Commencement Date:
Number of Restricted Stock Units:

Vesting Schedule: [_____, subject to Participant's Continuous Service through each such vesting date.]

Issuance Schedule: Subject to any Capitalization Adjustment, one share of Common Stock (or its cash equivalent, at the discretion of the Company) will be issued for each Restricted Stock Unit that vests at the time set forth in Section 6 of the Award Agreement.

Additional Terms/Acknowledgements: Participant acknowledges receipt of, and understands and agrees to, this Restricted Stock Unit Grant Notice, the Award Agreement and the Plan. Participant further acknowledges that as of the Date of Grant, this Restricted Stock Unit Grant Notice, the Award Agreement and the Plan set forth the entire understanding between Participant and the Company regarding the acquisition of the Common Stock pursuant to the Award specified above and supersede all prior oral and written agreements on the terms of this Award, with the exception, if applicable, of (i) restricted stock unit awards or options previously granted and delivered to Participant, (ii) the written employment agreement, offer letter or other written agreement entered into between the Company and Participant specifying the terms that should govern this specific Award, and (iii) any compensation recovery policy that is adopted by the Company or is otherwise required by applicable law.

By accepting this Award, Participant acknowledges having received and read the Restricted Stock Unit Grant Notice, the Award Agreement and the Plan and agrees to all of the terms and conditions set forth in these documents. Participant consents to receive Plan documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

MIRUM PHARMACEUTICALS, INC.	**PARTICIPANT**
By:	
Signature	Signature
Title:	Date:
Date:	

ATTACHMENTS: Award Agreement (including the Appendix) and 2019 Equity Incentive Plan

ATTACHMENT I

MIRUM PHARMACEUTICALS, INC.

2019 EQUITY INCENTIVE PLAN
RESTRICTED STOCK UNIT AWARD AGREEMENT

Pursuant to the Restricted Stock Unit Grant Notice (the "***Grant Notice***") and this Restricted Stock Unit Award Agreement (the "***Agreement***", the definition of which shall include any special terms and conditions for your country set out in the attached appendix (the "***Appendix***")), Mirum Pharmaceuticals, Inc. (the "***Company***") has awarded you ("***Participant***") a Restricted Stock Unit Award (the "***Award***") pursuant to the Company's 2019 Equity Incentive Plan (the "***Plan***") for the number of Restricted Stock Units/shares indicated in the Grant Notice. Capitalized terms not explicitly defined in this Agreement or the Grant Notice shall have the same meanings given to them in the Plan. The terms of your Award, in addition to those set forth in the Grant Notice, are as follows.

1. GRANT OF THE AWARD. This Award represents the right to be issued on a future date one (1) share of Common Stock for each Restricted Stock Unit that vests on the applicable vesting date(s) (subject to any adjustment under Section 3 below) as indicated in the Grant Notice. As of the Date of Grant, the Company will credit to a bookkeeping account maintained by the Company for your benefit (the "***Account***") the number of Restricted Stock Units/shares of Common Stock subject to the Award. Notwithstanding the foregoing, the Company reserves the right to issue you the cash equivalent of Common Stock, in part or in full satisfaction of the delivery of Common Stock in connection with the vesting of the Restricted Stock Units, and, to the extent applicable, references in this Agreement and the Grant Notice to Common Stock issuable in connection with your Restricted Stock Units will include the potential issuance of its cash equivalent pursuant to such right. This Award was granted in consideration of your services to the Company.

2. VESTING. Subject to the limitations contained herein, your Award will vest, if at all, in accordance with the vesting schedule provided in the Grant Notice. Vesting will cease upon the termination of your Continuous Service and the Restricted Stock Units credited to the Account that were not vested on the date of such termination will be forfeited at no cost to the Company and you will have no further right, title or interest in or to such Award or the shares of Common Stock to be issued in respect of such portion of the Award.

3. NUMBER OF SHARES. The number of Restricted Stock Units subject to your Award may be adjusted from time to time for Capitalization Adjustments, as provided in the Plan. Any additional Restricted Stock Units, shares, cash or other property that becomes subject to the Award pursuant to this Section 3, if any, shall be subject, in a manner determined by the Board, to the same forfeiture restrictions, restrictions on transferability, and time and manner of delivery as applicable to the other Restricted Stock Units and shares covered by your Award. Notwithstanding the provisions of this Section 3, no fractional shares or rights for fractional shares of Common Stock shall be created pursuant to this Section 3. Any fraction of a share will be rounded down to the nearest whole share.

4. SECURITIES LAW COMPLIANCE. You may not be issued any Common Stock under your Award unless the shares of Common Stock underlying the Restricted Stock Units are either (i) then registered under the Securities Act, or (ii) the Company has determined that such issuance would be exempt from the registration requirements of the Securities Act. Your Award must also comply with other applicable laws and regulations governing the Award, and you shall not receive such Common Stock if the Company determines that such receipt would not be in material compliance with such laws and regulations.

5. TRANSFER RESTRICTIONS. Prior to the time that shares of Common Stock have been delivered to you, you may not transfer, pledge, sell or otherwise dispose of this Award or the shares issuable in respect of your Award, except as expressly provided in this Section 5. For example, you may not use shares that may be issued in respect of your Restricted Stock Units as security for a loan. The restrictions on transfer set forth herein will lapse upon delivery to you of shares in respect of your vested Restricted Stock Units.

(a) Death. Your Award is not transferable other than to your personal representative on your death. At your death, vesting of your Award will cease and your personal representative shall be entitled to receive any Common Stock or other consideration that vested but was not issued before your death.

6. DATE OF ISSUANCE.

(a) If you are subject to taxation in the United States, the issuance of shares in respect of the Restricted Stock Units is intended to comply with Treasury Regulations Section 1.409A-1(b)(4) and will be construed and administered in such a manner. Subject to the satisfaction of the Withholding Obligation set forth in Section 11 of this Agreement, in the event one or more Restricted Stock Units vests, the Company shall issue to you one (1) share of Common Stock for each Restricted Stock Unit that vests on the applicable vesting date(s) (subject to any adjustment under Section 3 above, and subject to any different provisions in the Grant Notice). Each issuance date determined by this paragraph is referred to as an "***Original Issuance Date***".

(b) If the Original Issuance Date falls on a date that is not a business day, delivery shall instead occur on the next following business day. In addition, if:

(i) the Original Issuance Date does not occur (1) during an "open window period" applicable to you, as determined by the Company in accordance with the Company's then-effective policy on trading in Company securities, or (2) on a date when you are otherwise permitted to sell shares of Common Stock on an established stock exchange or stock market (including but not limited to under a previously established written trading plan that meets the requirements of Rule 10b5-1 under the Exchange Act and was entered into in compliance with the Company's policies (a "***10b5-1 Arrangement***")), *and*

(ii) either (1) a Withholding Obligation does not apply, or (2) the Company decides, prior to the Original Issuance Date, (A) not to satisfy the Withholding Obligation by withholding shares of Common Stock from the shares otherwise due, on the Original Issuance Date, to you under this Award, and (B) not to permit you to enter into a "same day sale" commitment with a broker-dealer pursuant to Section 11 of this Agreement (including but not limited to a commitment under a 10b5-1 Arrangement) and (C) not to permit you to pay your Withholding Obligation in cash,

then the shares that would otherwise be issued to you on the Original Issuance Date will not be delivered on such Original Issuance Date and will instead be delivered on the first business day when you are not prohibited from selling shares of the Company's Common Stock in the open public market, but, if you are subject to taxation in the United States, in no event later than December 31 of the calendar year in which the Original Issuance Date occurs (that is, the last day of your taxable year in which the Original Issuance Date occurs), or, if and only if permitted in a manner that complies with Treasury Regulations Section 1.409A-1(b)(4), no later than the date that is the 15th day of the third calendar month of the applicable year following the year in which the shares of Common Stock under this Award are no longer subject to a "substantial risk of forfeiture" within the meaning of Treasury Regulations Section 1.409A-1(d).

(c) The form of delivery (*e.g.*, a stock certificate or electronic entry evidencing such shares) shall be determined by the Company.

7. DIVIDENDS. You shall receive no benefit or adjustment to your Award with respect to any cash dividend, stock dividend or other distribution that does not result from a Capitalization Adjustment; provided, however, that this sentence will not apply with respect to any shares of Common Stock that are delivered to you in connection with your Award after such shares have been delivered to you.

8. RESTRICTIVE LEGENDS. The shares of Common Stock issued in respect of your Award shall be endorsed with appropriate legends as determined by the Company.

9. EXECUTION OF DOCUMENTS. You hereby acknowledge and agree that the manner selected by the Company by which you indicate your consent to your Grant Notice is also deemed to be your execution of your Grant Notice and of this Agreement. You further agree that such manner of indicating consent may be relied upon as your signature for establishing your execution of any documents to be executed in the future in connection with your Award.

10. AWARD NOT A SERVICE CONTRACT.

(a) Nothing in this Agreement (including, but not limited to, the vesting of your Award or the issuance of the shares in respect of your Award), the Plan or any covenant of good faith and fair dealing that may be found implicit in this Agreement or the Plan shall: (i) confer upon you any right to continue in the employ or service of, or affiliation with, the Company or an Affiliate; (ii) constitute any promise or commitment by the Company or an Affiliate regarding the fact or nature of future positions, future work assignments, future compensation or any other term or condition of employment or affiliation; (iii) confer any right or benefit under this Agreement or the Plan unless such right or benefit has specifically accrued under the terms of this Agreement or Plan; or (iv) deprive the Company or any Affiliate of the right to terminate you in accordance with applicable laws and your employment or engagement agreement, if any, without regard to any future vesting opportunity that you may have.

(b) By accepting this Award, you acknowledge and agree that the right to continue vesting in the Award pursuant to the vesting schedule provided in the Grant Notice may not be earned unless (in addition to any other conditions described in the Grant Notice and this Agreement) you continue as an employee, director or consultant at the will of the Company and affiliate, as applicable (not through the act of being hired, being granted this Award or any other award or benefit) and that the Company has the right to reorganize, sell, spin-out or otherwise restructure one or more of its businesses or Affiliates at any time or from time to time, as it deems appropriate (a "reorganization"). You acknowledge and agree that such a reorganization could result in the termination of your Continuous Service, or the termination of Affiliate status of your employer and the loss of benefits available to you under this Agreement, including but not limited to, the termination of the right to continue vesting in the Award. You further acknowledge and agree that this Agreement, the Plan, the transactions contemplated hereunder and the vesting schedule set forth herein or any covenant of good faith and fair dealing that may be found implicit in any of them do not constitute an express or implied promise of continued engagement as an employee or consultant for the term of this Agreement, for any period, or at all, and shall not interfere in any way with the Company's right to terminate your Continuous Service in accordance with applicable law and your employment or engagement agreement, if any, or to conduct a reorganization.

(c) By accepting your Award, you acknowledge, understand and agree that:

(i) the Plan is established voluntarily by the Company, it is discretionary in nature, and may be amended, suspended or terminated by the Company at any time, to the extent permitted under the Plan;

(ii) the grant of your Award is voluntary and occasional and does not create any contractual or other right to receive future grants of awards (whether on the same or different terms), or benefits in lieu of awards, even if awards have been granted in the past;

(iii) your Award and any shares of Common Stock acquired under the Plan, and the income and value of same, are not part of normal or expected compensation for any purpose, including, without limitation, calculating any severance, resignation, termination, redundancy, dismissal, end-of-service payments, bonuses, holiday pay, long-service awards, pension or retirement or welfare benefits or similar payments;

(iv) the future value of the shares of Common Stock underlying the Award is unknown, indeterminable, and cannot be predicted with certainty;

(v) neither the Company nor any Affiliate shall be liable for any foreign exchange rate fluctuation between your local currency and the United States Dollar that may affect the value of your Award or of any amounts due to you pursuant to the vesting of your Award or the subsequent sale of any shares of Common Stock received;

(vi) for the purposes of your Award, your Continuous Service will be considered terminated as of the date you are no longer actively providing services to the Company or one of its Affiliates (regardless of the reason for such termination and whether or not later found to be invalid or in breach of employment laws in the jurisdiction where you are employed or the terms of your employment agreement, if any), and unless otherwise expressly provided in this Agreement or determined by the Company, your right to vest in the Award under the Plan, if any, and the Board shall have the exclusive discretion to determine when you are no longer actively providing services for purposes of the Award (including whether you may still be considered to be providing services while on a leave of absence);

(vii) no claim or entitlement to compensation or damages shall arise from forfeiture of this Award resulting from the termination of your Continuous Service (for any reason whatsoever, whether or not later found to be invalid or in breach of employment laws in the jurisdiction where you are employed or the terms of your employment or service agreement, if any), and in consideration of the grant of this Award to which you are otherwise not entitled, you irrevocably agree never to institute any claim against the Company or any Affiliate, waive your ability, if any, to bring any such claim, and release the Company and any Affiliate from any such claim; if, notwithstanding the foregoing, any such claim is allowed by a court of competent jurisdiction, then, by participating in the Plan, you shall be deemed irrevocably to have agreed not to pursue such claim and agree to execute any and all documents necessary to request dismissal or withdrawal of such claim.

11. WITHHOLDING OBLIGATION.

(a) On each vesting date, and on or before the time you receive a distribution of the shares of Common Stock in respect of your Restricted Stock Units, and at any other time as reasonably requested by the Company in accordance with applicable tax and social security laws, you hereby authorize any required withholding from the Common Stock issuable to you and/or otherwise agree to make adequate provision, including in cash, for any sums required to satisfy the federal, state, local and foreign tax and

social security withholding obligations of the Company or any Affiliate that arise in connection with your Award (the "***Withholding Obligation***").

(b) By accepting this Award, you acknowledge and agree that the Company or any Affiliate may, in its sole discretion, satisfy all or any portion of the Withholding Obligation relating to your Restricted Stock Units by any of the following means or by a combination of such means: (i) causing you to pay any portion of the Withholding Obligation in cash; (ii) withholding from any compensation otherwise payable to you by the Company; (iii) withholding shares of Common Stock from the shares of Common Stock issued or otherwise issuable to you in connection with the Award with a Fair Market Value (measured as of the date shares of Common Stock are issued pursuant to Section 6) equal to the amount of such Withholding Obligation; provided, however, that the number of such shares of Common Stock so withheld will not exceed the amount necessary to satisfy the Withholding Obligation using the maximum statutory withholding rates for federal, state, local and foreign tax and social security purposes, including payroll taxes, that are applicable to supplemental taxable income; and *provided*, further, that to the extent necessary to qualify for an exemption from application of Section 16(b) of the Exchange Act, if applicable, such share withholding procedure will be subject to the express prior approval of the Board or the Company's Compensation Committee; and/or (iv) permitting or requiring you to enter into a "same day sale" commitment, if applicable, with a broker-dealer that is a member of the Financial Industry Regulatory Authority (a "***FINRA Dealer***"), pursuant to this authorization and without further consent, whereby you irrevocably elect to sell a portion of the shares to be delivered in connection with your Restricted Stock Units to satisfy the Withholding Obligation and whereby the FINRA Dealer irrevocably commits to forward the proceeds necessary to satisfy the Withholding Obligation directly to the Company and/or its Affiliates. Unless the Withholding Obligation is satisfied, the Company shall have no obligation to deliver to you any Common Stock or any other consideration pursuant to this Award.

(c) In the event the Withholding Obligation arises prior to the delivery to you of Common Stock or it is determined after the delivery of Common Stock to you that the amount of the Withholding Obligation was greater than the amount withheld by the Company, you agree to indemnify and hold the Company harmless from any failure by the Company to withhold the proper amount.

12. TAX CONSEQUENCES. The Company has no duty or obligation to minimize the tax or social security consequences to you of this Award and shall not be liable to you for any adverse tax or social security consequences to you arising in connection with this Award. You are hereby advised to consult with your own personal tax, financial and/or legal advisors regarding the tax and social security consequences of this Award and by signing the Grant Notice, you have agreed that you have done so or knowingly and voluntarily declined to do so. You understand that you (and not the Company or any Affiliate) shall be responsible for your own tax and social security liability that may arise as a result of this investment or the transactions contemplated by this Agreement.

13. UNSECURED OBLIGATION. Your Award is unfunded, and as a holder of a vested Award, you shall be considered an unsecured creditor of the Company with respect to the Company's obligation, if any, to issue shares or other property pursuant to this Agreement. You shall not have voting or any other rights as a stockholder of the Company with respect to the shares to be issued pursuant to this Agreement until such shares are issued to you pursuant to Section 6 of this Agreement. Upon such issuance, you will obtain full voting and other rights as a stockholder of the Company. Nothing contained in this Agreement, and no action taken pursuant to its provisions, shall create or be construed to create a trust of any kind or a fiduciary relationship between you and the Company or any other person.

14. NOTICES. Any notice or request required or permitted hereunder shall be given in writing (including electronically) and will be deemed effectively given upon receipt or, in the case of notices delivered by mail by the Company to you, five (5) days after deposit in the national mail, postage prepaid,

addressed to you at the last address you provided to the Company. The Company may, in its sole discretion, decide to deliver any documents related to participation in the Plan and this Award by electronic means or to request your consent to participate in the Plan by electronic means. By accepting this Award, you consent to receive such documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

15. HEADINGS. The headings of the Sections in this Agreement are inserted for convenience only and shall not be deemed to constitute a part of this Agreement or to affect the meaning of this Agreement.

16. MISCELLANEOUS.

(a) The rights and obligations of the Company under your Award shall be transferable by the Company to any one or more persons or entities, and all covenants and agreements hereunder shall inure to the benefit of, and be enforceable by, the Company's successors and assigns.

(b) You agree upon request to execute any further documents or instruments necessary or desirable in the sole determination of the Company to carry out the purposes or intent of your Award.

(c) You acknowledge and agree that you have reviewed your Award in its entirety, have had an opportunity to obtain the advice of counsel prior to executing and accepting your Award and fully understand all provisions of your Award.

(d) This Agreement shall be subject to all applicable laws, rules, and regulations, and to such approvals by any governmental agencies or national securities exchanges as may be required.

(e) All obligations of the Company under the Plan and this Agreement shall be binding on any successor to the Company, whether the existence of such successor is the result of a direct or indirect purchase, merger, consolidation, or otherwise, of all or substantially all of the business and/or assets of the Company.

17. GOVERNING PLAN DOCUMENT. Your Award is subject to all the provisions of the Plan, the provisions of which are hereby made a part of your Award, and is further subject to all interpretations, amendments, rules and regulations which may from time to time be promulgated and adopted pursuant to the Plan. Your Award (and any compensation paid or shares issued under your Award) is subject to recoupment in accordance with The Dodd–Frank Wall Street Reform and Consumer Protection Act and any implementing regulations thereunder, any clawback policy adopted by the Company and any compensation recovery policy otherwise required by applicable law. No recovery of compensation under such a clawback policy will be an event giving rise to a right to voluntarily terminate employment upon a resignation for "good reason," or for a "constructive termination" or any similar term under any plan of or agreement with the Company.

18. EFFECT ON OTHER EMPLOYEE BENEFIT PLANS. The value of the Award subject to this Agreement shall not be included as compensation, earnings, salaries, or other similar terms used when calculating benefits under any employee benefit plan (other than the Plan) sponsored by the Company or any Affiliate except as such plan otherwise expressly provides. The Company expressly reserves its rights to amend, modify, or terminate any or all of the employee benefit plans of the Company or any Affiliate.

19. SEVERABILITY. If all or any part of this Agreement or the Plan is declared by any court or governmental authority to be unlawful or invalid, such unlawfulness or invalidity shall not invalidate any portion of this Agreement or the Plan not declared to be unlawful or invalid. Any Section of this Agreement

(or part of such a Section) so declared to be unlawful or invalid shall, if possible, be construed in a manner which will give effect to the terms of such Section or part of a Section to the fullest extent possible while remaining lawful and valid.

20. OTHER DOCUMENTS. You hereby acknowledge receipt or the right to receive a document providing the information required by Rule 428(b)(1) promulgated under the Securities Act. In addition, you acknowledge receipt of the Company's policy permitting certain individuals to sell shares only during certain "window" periods and the Company's insider trading policy, in effect from time to time.

21. AMENDMENT. This Agreement may not be modified, amended or terminated except by an instrument in writing, signed by you and by a duly authorized representative of the Company. Notwithstanding the foregoing, this Agreement may be amended solely by the Board by a writing which specifically states that it is amending this Agreement, so long as a copy of such amendment is delivered to you, and provided that, except as otherwise expressly provided in the Plan, no such amendment materially adversely affecting your rights hereunder may be made without your written consent. Without limiting the foregoing, the Board reserves the right to change, by written notice to you, the provisions of this Agreement in any way it may deem necessary or advisable to carry out the purpose of the Award as a result of any change in applicable laws or regulations or any future law, regulation, ruling, or judicial decision, provided that any such change shall be applicable only to rights relating to that portion of the Award which is then subject to restrictions as provided herein.

22. COMPLIANCE WITH SECTION 409A OF THE CODE. If you are subject to taxation in the United States, this Award is intended to be exempt from the application of Section 409A of the Code, including but not limited to by reason of complying with the "short-term deferral" rule set forth in Treasury Regulation Section 1.409A-1(b)(4) and any ambiguities herein shall be interpreted accordingly. Notwithstanding the foregoing, if it is determined that the Award fails to satisfy the requirements of the short-term deferral rule and is otherwise not exempt from, and determined to be deferred compensation subject to Section 409A of the Code, this Award shall comply with Section 409A to the extent necessary to avoid adverse personal tax consequences and any ambiguities herein shall be interpreted accordingly. If it is determined that the Award is deferred compensation subject to Section 409A and you are a "Specified Employee" (within the meaning set forth in Section 409A(a)(2)(B)(i) of the Code) as of the date of your "Separation from Service" (as defined in Section 409A), then the issuance of any shares that would otherwise be made upon the date of your Separation from Service or within the first six (6) months thereafter will not be made on the originally scheduled date(s) and will instead be issued in a lump sum on the date that is six (6) months and one day after the date of the Separation from Service, with the balance of the shares issued thereafter in accordance with the original vesting and issuance schedule set forth above, but if and only if such delay in the issuance of the shares is necessary to avoid the imposition of adverse taxation on you in respect of the shares under Section 409A of the Code. Each installment of shares that vests is intended to constitute a "separate payment" for purposes of Treasury Regulation Section 1.409A-2(b)(2).

23. DATA PRIVACY.

(a) You explicitly and unambiguously acknowledge and consent to the collection, use and transfer, in electronic or other form, of your personal data as described in this document by and among, as applicable, your employer, the Company and its Affiliates for the exclusive purpose of implementing, administering and managing your participation in the Plan. You understand that the Company, its Affiliates and your employer hold certain personal information about you, including, but not limited to, name, home address and telephone number, date of birth, social security number (or other identification number), salary, nationality, job title, any shares of stock or directorships held in the Company, details of all options or any other entitlement to shares of stock awarded, canceled, purchased, exercised, vested, unvested or outstanding in your favor for the purpose of implementing, managing and administering the Plan ("***Data***").

You understand that the Data may be transferred to any third parties assisting in the implementation, administration and management of the Plan, that these recipients may be located in your country or elsewhere, in particular in the US, and that the recipient country may have different data privacy laws providing less protections of your personal data than your country. You may request a list with the names and addresses of any potential recipients of the Data by contacting the stock plan administrator at the Company (the "***Stock Plan Administrator***"). You acknowledge that the recipients may receive, possess, process, use, retain and transfer the Data, in electronic or other form, for the purposes of implementing, administering and managing your participation in the Plan, including any requisite transfer of such Data, as may be required to a broker or other third party with whom you may elect to deposit any shares of Common Stock acquired upon the vesting of your Award. You understand that Data will be held only as long as is necessary to implement, administer and manage your participation in the Plan. You may, at any time, view the Data, request additional information about the storage and processing of the Data, require any necessary amendments to the Data or refuse or withdraw the consents herein, in any case without cost, by contacting the Stock Plan Administrator in writing.

(b) For the purposes of operating the Plan in the European Union, Switzerland and the United Kingdom, the Company will collect and process information relating to you in accordance with the privacy notice from time to time in force.

24. NO ADVICE REGARDING GRANT. The Company is not providing any tax, legal or financial advice, nor is the Company making any recommendations regarding your participation in the Plan, or your acquisition or sale of the underlying shares of Common Stock. You should consult with your own personal tax, legal and financial advisors regarding your participation in the Plan before taking any action related to the Plan.

25. LANGUAGE. You acknowledge that you are sufficiently proficient in the English language, or have consulted with an advisor who is sufficiently proficient in English, so as to allow you to understand the terms and conditions of this Agreement. If you have received this Agreement, or any other document related to this Award and/or the Plan translated into a language other than English and if the meaning of the translated version is different than the English version, the English version will control.

26. INSIDER TRADING/MARKET ABUSE. You acknowledge that, depending on your country, you may be subject to insider trading restrictions and/or market abuse laws, which may affect your ability to acquire or sell the shares of Common Stock or rights to the shares of Common Stock under the Plan during such times as you are considered to have "inside information" regarding the Company (as defined by the laws in your country). Any restrictions under these laws or regulations are separate from and in addition to any restrictions that may be imposed under any applicable Company insider trading policy. You acknowledge that it is your responsibility to comply with any applicable restrictions, and you are advised to speak to your personal advisor on this matter.

27. FOREIGN ASSET/ACCOUNT, EXCHANGE CONTROL AND TAX REPORTING. You may be subject to foreign asset/account, exchange control and/or tax reporting requirements as a result of the acquisition, holding and/or transfer of shares of Common Stock or cash (including dividends and the proceeds arising from the sale of shares of Common Stock) derived from your participation in the Plan in, to and/or from a brokerage/bank account or legal entity located outside your country. The applicable laws in your country may require that you report such accounts, assets and balances therein, the value thereof and/or the transactions related thereto to the applicable authorities in such country. You may also be required to repatriate sale proceeds or other funds received as a result of your participation in the Plan to your country through a designated bank or broker within a certain time after receipt. You acknowledge that it is your responsibility to be compliant with such regulations and you are encouraged to consult with your personal legal advisor for any details.

28. Choice of Law. The interpretation, performance and enforcement of this Agreement shall be governed by the laws of the State of Delaware without regard to that state's conflicts of laws rules.

29. Appendix. Notwithstanding any provisions in this Agreement, your Award shall be subject to the special terms and conditions for your country set forth in the Appendix attached hereto. Moreover, if you relocate to one of the countries included therein, the terms and conditions for such country will apply to you to the extent the Company determines that the application of such terms and conditions is necessary or advisable for legal or administrative reasons. The Appendix constitutes part of this Agreement.

* * * * *

This Restricted Stock Unit Award Agreement shall be deemed to be signed by the Company and the Participant upon the signing by the Participant of the Restricted Stock Unit Grant Notice to which it is attached.

APPENDIX

This Appendix includes special terms and conditions that govern the Award granted to you under the Plan if you reside and/or work in any country listed below.

The information contained herein is general in nature and may not apply to your particular situation, and you are advised to seek appropriate professional advice as to how the relevant laws in your country may apply to your situation. If you are a citizen or resident of a country other than the one in which you are currently working and/or residing, transfer employment and/or residency to another country after the date of grant, are a consultant, change employment status to a consultant position, or are considered a resident of another country for local law purposes, the Company shall, in its discretion, determine the extent to which the special terms and conditions contained herein shall be applicable to you. References to your employer shall include any entity that engages your services.

DENMARK

Stock Option Act. You acknowledge that you received the Employer Statement in Danish which sets forth additional terms of the Award to the extent the Danish Stock Options Act applies.

Foreign Asset / Account Reporting. If you establish an account holding cash or shares of Common Stock outside Denmark, you must report the account to the Danish Tax Administration.

GERMANY

Securities Disclaimer. Participation in the Plan is exempt or excluded from the requirement to publish a prospectus under the EU Prospectus Directive as implemented in Germany.

Exchange Control Information. Cross-border payments in excess of EUR €12,500 must be reported monthly to the German Federal Bank (*Bundesbank*). In the event that you make or receive a payment in excess of this amount, you are required to report the payment to Bundesbank electronically using the "General Statistics Reporting Portal" ("*Allgemeines Meldeportal Statistik*") available via Bundesbank's website (www.bundesbank.de).

Tax Reporting. You must report and pay any capital gains tax liability that arises in connection with the sale of shares of Common Stock acquired under the Plan. In general the statutory deadline of filing annual income tax returns for taxpayers is July 31 of the calendar year following the respective fiscal year. Payment periods of due tax amounts are determined in view of the competent tax office. You should consult with your personal tax advisor to ensure that you are properly complying with applicable reporting requirements in Germany.

ITALY

Plan Document Acknowledgement. By accepting the Award, you acknowledge that you have received a copy of the Plan, have reviewed the Plan and the Agreement in their entirety and fully understand and accept all provisions of the Plan and the Agreement.

You acknowledge that you have read and specifically and expressly approve the following sections of the Agreement and this Appendix, including: (10) Award Not a Service Contract; (11) Withholding Obligations; 12 (Tax Consequences); (23) Data Privacy; (24) Language; and (28) Choice of Law.

Foreign Asset/Account Reporting Information. If you are an Italian resident and, during any fiscal year, hold investments or financial assets outside of Italy (*e.g.*, cash, shares of Common Stock) which may generate income taxable in Italy (or if you are the beneficial owner of such an investment or asset even if you do not directly hold the investment or asset), you are required to report such investments or assets on your annual tax return for such fiscal year (on UNICO Form, RW Schedule, or on a special form if you are not required to file a tax return).

Foreign Financial Assets Tax. The fair market value of any shares of Common Stock held outside of Italy is subject to a foreign assets tax. Financial assets include shares of Common Stock acquired under the Plan. The taxable amount will be the fair market value of the financial assets assessed at the end of the calendar year. *You should consult with your personal tax advisor about the foreign financial assets tax.*

NETHERLANDS

Prohibition Against Insider Trading. You should be aware of the European insider trading rules, which may affect the sale of shares of Common Stock acquired under the Plan. In particular, you may be prohibited from effecting certain share transactions if you have insider information regarding the Company. If it is uncertain whether the insider rules apply, the Company recommends that you consult with a legal advisor. The Company cannot be held liable if you violate the insider trading rules applicable in the Netherlands. You are responsible for ensuring your compliance with these rules.

Under Article 14 of the Market Abuse Regulation ((EU) Regulation 596/2014), anyone who has "inside information" related to an issuing company is prohibited from effectuating a transaction in securities in or from the Netherlands. "Inside information" is defined as knowledge of specific information concerning the issuing company to which the securities relate or the trade in securities issued by such company, which has not been made public and which, if published, would reasonably be expected to affect the price of the securities, regardless of the development of the price. The insider could be any employee of the Company or an Affiliate in the Netherlands who has inside information as described herein.

Given the broad scope of the definition of inside information, certain participants may have inside information and thus are prohibited from making a transaction in securities in the Netherlands at a time when they have such inside information. By entering into this Agreement and participating in the Plan, you acknowledge having read and understood the notification above and acknowledge that it is your responsibility to comply with the Dutch insider trading rules, as discussed herein.

Securities Disclaimer. The grant of the Award is exempt or excluded from the requirement to publish a prospectus under the Prospectus Regulation ((EU) Regulation 2017/1129). The Awards are not transferable and are not deemed to qualify as an offering of securities in the Netherlands within the meaning of the Prospectus Regulation. To the extent that a supervisory body would qualify the offering of Awards or its underlying securities as an offering of securities within the meaning of the Prospectus Regulation, such offering will only be made in reliance on Article 1(4) of the Prospectus Regulation provided that no such offering of securities shall require Mirum Pharmaceuticals, Inc. to publish a prospectus pursuant to Article 3 of the Prospectus Regulation.

SPAIN

No Entitlement for Claims or Compensation. The following provision supplements Section 10 of the Agreement that clarifies that the grant, vesting, or settlement of Award does not give you a right to continued service/employment:

By accepting the grant of Award, you consent to participation in the Plan and acknowledge that you have received a copy of the Plan document.

You understand that the Company has unilaterally, gratuitously and in its sole discretion decided to make grants of Awards under the Plan to individuals who may be Consultants, Directors, and Employees throughout the world. The decision is limited and entered into based upon the express assumption and condition that any Awards will not economically or otherwise bind the Company or any Affiliate, including the employer, on an ongoing basis, other than as expressly set forth in the Agreement. Consequently, you understand that the grant of Awards is made on the assumption and condition that the Awards shall not become part of any employment contract (whether with the Company or any Affiliate, including the employer) and shall not be considered a mandatory benefit, salary for any purpose (including severance compensation) or any other right whatsoever. Furthermore, you understand and freely accept that there is no guarantee that any benefit whatsoever shall arise from the Awards, which is gratuitous and discretionary, since the future value of the Awards and the underlying shares of Common Stock is unknown and unpredictable.

You understand and agree that, as a condition of the grant of the Awards, your termination of Continuous Service for any reason (including for the reasons listed below) will automatically result in the cancellation and loss of any Awards that may have been granted to you and that were not fully vested on the date of termination of your Continuous Service. In particular, you understand and agree that, unless otherwise expressly provided for by the Company at the Date of Grant, the Awards will be cancelled without entitlement to the shares of Common Stock or to any amount as indemnification if you terminate employment by reason of, including, but not limited to: resignation, death, disability, retirement, disciplinary dismissal adjudged to be with cause, disciplinary dismissal adjudged or recognized to be without cause, individual or collective layoff on objective grounds, whether adjudged to be with cause or adjudged or recognized to be without cause, material modification of the terms of employment under Article 41 of the Workers' Statute, relocation under Article 40 of the Workers' Statute, Article 50 of the Workers' Statute, unilateral withdrawal by the employer, and under Article 10.3 of Royal Decree 1382/1985.

You also understand that this grant of Awards would not be made but for the assumptions and conditions set forth hereinabove; thus, you understand, acknowledge and freely accept that, should any or all of the assumptions be mistaken or any of the conditions not be met for any reason, the grant, the Awards and any right to the underlying shares of Common Stock shall be null and void.

Securities Law Information. The Award described in the Agreement and this Appendix do not qualify under Spanish regulations as securities. No "offer of securities to the public", as defined under Spanish law, has taken place or will take place in the Spanish territory. The Agreement (including this Appendix) has not been nor will it be registered with the *Comisión Nacional del Mercado de Valores*, and does not constitute a public offering prospectus.

Foreign Assets Reporting. You may be subject to certain tax reporting requirements with respect to assets or rights that you hold outside of Spain, including bank accounts, securities and real estate if the aggregate value for particular category of assets exceeds EUR €50,000 as of December 31 each year. Shares of Common Stock acquired under the Plan or other equity programs offered by the Company constitute

securities for purposes of this requirement, but unvested awards (e.g., RSU Awards, etc.) are not considered assets or rights for purposes of this reporting requirement.

If applicable, you must report the assets on Form 720 by no later than March 31 following the end of the relevant year. After the rights and/or assets are initially reported, the reporting obligation will only apply if: (i) the value of previously-reported rights or assets increases by more than EUR €20,000 as of each subsequent December 31, or (ii) upon disposition of the previously-reported rights or assets. You are encouraged to consult with your personal advisor to determine any obligations in this respect.

Share Reporting Requirement. You must declare the acquisition, ownership and disposition of shares of Common Stock to the Spanish *Dirección General de Comercio e Inversiones* (the "***DGCI***") of the Ministry of Economy and Competitiveness on a Form D-6. Generally, the declaration must be made in January for shares of Common Stock owned as of December 31 of the prior year and/or shares of Common Stock acquired or disposed of during the prior year; however, if the value of the shares of Common Stock acquired or the amount of the sale proceeds exceeds EUR €1,502,530 (or you hold 10% or more of the share capital of the Company or other such amount that would entitle you to join the Board), the declaration must be filed within one month of the acquisition or disposition, as applicable. You should consult with your personal advisor to determine your obligations in this respect.

Foreign Assets and Transaction Reporting. You may be required to electronically declare to the Bank of Spain any foreign accounts (including brokerage accounts held abroad), any foreign instruments (*e.g.*, shares of Common Stock) and any transactions with non-Spanish residents (including any payments of cash or shares made to you by the Company or a U.S. brokerage account) if the balances in such accounts together with the value of such instruments as of December 31, or the volume of transactions with non-Spanish residents during the prior or current year, exceed EUR €1,000,000. Once the EUR €1,000,000 threshold has been surpassed in either respect, you will generally be required to report all of your foreign accounts, foreign instruments and transactions with non-Spanish residents, even if the relevant threshold has not been crossed for an individual item. You will generally only be required to report on an annual basis (by January 20 of each year); however, if the balances in your foreign accounts together with value of your foreign instruments or the volume of transactions with non-Spanish residents exceed EUR €100,000,000, you acknowledge that more frequent reporting will be required.

SWITZERLAND

Sole Contact and Contractual Partner Information. You acknowledge that the Award, this Agreement including the Appendix and, the Annexes and your participation in the Plan do not create any claims against the employer, either directly or indirectly. Your sole contract and sole contractual partner regarding the Plan and the Award is the Company and the Award does not form part of your contractual compensation.

Continuous Service. Notwithstanding anything else in the Plan or the Agreement, the Continuous Service will be deemed to end on the date when a termination notice is issued, regardless of whether the cessation of the employment was lawful, and shall not include any period notice of termination of employment or any period of salary continuance or deemed employment. As a result, if you receive notice of termination your Continuous Service will end on the date you receive such notice.

Securities Law Information. The Award is not intended to be publicly offered in or from Switzerland. Because it is considered a private offering, it is not subject to securities registration in Switzerland. Neither this document nor any other materials relating to the Restricted Stock Units and/or the underlying shares of Common Stock: (i) constitutes a prospectus according to articles 35 et seq. of the Swiss Federal Act on Financial Services ("***FinSA***"); (ii) may be publicly distributed or otherwise made publicly available in

Switzerland to any person other than a Participant; or (iii) has been or will be filed with, approved or supervised by any Swiss reviewing body according to article 51 FinSA or any Swiss regulatory authority, including the Swiss Financial Market Supervisory Authority ("***FINMA***").

Grant of the Award. The Award is a voluntary gratuity (*Gratifikation; gratification*) within the meaning of Article 322d Swiss Code of Obligations (CO) as determined at the Company's sole discretion which you have no entitlement to and which does not constitute an entitlement for a grant of further Awards or other equities in the future.

Vesting. You acknowledge and confirm that the Award is fully discretionary and that before the Restricted Stock Units have vested you shall not have any right in regard to such Restricted Stock Units.

Disability. For the avoidance of any doubt, "***Disability***" shall include, but not be limited to, any permanent disability as per the social security laws of Switzerland.

Social Security and Tax. You herewith directly authorize your employer to make all (if any) applicable social security, insurance and tax deductions resulting from the grant and/or vesting of the Award or the sale of shares of Common Stock from any compensation owed to you by your employer, subject to any statutory limitations. If your compensation shall not be sufficient to cover such social security, insurance and tax liabilities, you will indemnify the employer upon first demand.

Cause. "***Cause***" shall include, but not be limited to, all reasons entitling to a summary dismissal pursuant to article 337 of the Swiss Code of Obligations (CO) and all justified reasons pursuant to article 340c para. 2 CO, without limiting the definition of Cause as outlined in the Plan. You expressly acknowledge that the definition of Cause as per the Plan shall include any crime or felony under Swiss laws and any breaches against your duties and in respect of the employer, and not only in respect of the Company.

Language Acknowledgement. You confirm that you have read and understood the documents relating to the Plan, including the Agreement, with all terms and conditions included therein, which were provided in the English language only. You confirm that you have sufficient language capabilities to understand these terms and conditions in full.

Sie bestätigen, dass Sie den Plan sowie die dazugehörigen Dokumente, inklusive der Vereinbarung, mit all den darin enthaltenen Bedingungen und Voraussetzungen, welche in englischer Sprache verfasst sind, gelesen und verstanden haben. Sie bestätigen, dass Ihre Sprachkenntnisse genügend sind, um die Bedingungen und Voraussetzungen zu verstehen.

Vous confirmez que vous avez lu et compris les documents relatifs au plan, y compris la convention d'attribution, avec toutes les conditions qui y sont incluses, qui ont été fournies en langue anglaise uniquement. Vous confirmez que vous avez des capacités linguistiques suffisantes pour comprendre ces termes et conditions dans leur intégralité.

No Right against Employer. You expressly acknowledge that you shall not have any right or claim under the Restricted Stock Units, the Award, the Plan or the Agreement against your employer. You expressly acknowledge and agree that you only have any right and claim against the Company as set out under the Plan and the Agreement.

Governing Law and Jurisdiction. You expressly acknowledge and agree to the Choice of Law clause in the Plan and the Agreement and accept that Swiss law does not apply and that Swiss courts do not have any jurisdiction in regard to any claims under the Plan or the Agreement.

UNITED KINGDOM

No Cash Settlement. Notwithstanding any provision of the Plan or the Agreement, the Award may not be settled in cash.

Award Not a Service Contract. The following supplements Section 10 of the Agreement:

You waive all rights to compensation or damages in consequence of the termination of your office or employment with the Company or any Affiliate for any reason whatsoever (whether lawful or unlawful and including, without prejudice to the foregoing, in circumstances giving rise to a claim for wrongful dismissal) in so far as those rights arise or may arise from you ceasing to hold or being able to vest your Award, or from the loss or diminution in value of any rights or entitlements in connection with the Plan.

Withholding Obligations. The following supplements Section 11 of the Agreement:

As a condition of the vesting of your Award, you unconditionally and irrevocably agree:

(i) to place the Company in funds and indemnify the Company in respect of: (1) all liability to UK income tax which the Company is liable to account for on your behalf directly to HM Revenue & Customs; (2) all liability to national insurance contributions which the Company is liable to account for on your behalf to HM Revenue & Customs (including, to the extent permitted by law, secondary class 1 (employer's) national insurance contributions for which you are liable and hereby agree to bear); and (3) all liability to national insurance contributions for which the Company is liable and which are formally transferred to you, which arises as a consequence of or in connection with your Award (the "***UK Tax Liability***"); or

(ii) to permit the Company to sell at the best price which it can reasonably obtain such number of shares of Common Stock allocated or allotted to you following vesting as will provide the Company with an amount equal to the UK Tax Liability; and to permit the Company to withhold an amount not exceeding the UK Tax Liability from any payment made to you (including, but not limited to salary); and

(iii) if so required by the Company, and, to the extent permitted by law, to enter into a joint election or other arrangements under which the liability for all or part of such employer's national insurance contributions liability is transferred to you; and

(iv) if so required by the Company, to enter into a joint election within Section 431 of (UK) Income Tax (Earnings and Pensions) Act 2003 ("***ITEPA***") in respect of computing any tax charge on the acquisition of "restricted securities" (as defined in Section 423 and 424 of ITEPA); and

(v) to sign, promptly, all documents required by the Company to effect the terms of this provision, and references in this provision to "the Company" shall, if applicable, be construed as also referring to any Affiliate.

Clawback/Recovery. By executing the Agreement, you expressly consent in writing to the application of the right of recoupment to your Award in accordance with the terms of Section 8(l) of the Plan and Section 17 of the Agreement.

ATTACHMENT II

2019 EQUITY INCENTIVE PLAN

MIRUM PHARMACEUTICALS, INC.

2021 EMPLOYEE STOCK PURCHASE PLAN

TERMS AND CONDITIONS – NON-US PARTICIPANTS

By enrolling in the 2019 Employee Stock Purchase Plan (the "***Plan***") through an online enrollment procedure or such other enrollment procedure as may be established by the Company or by a third party designated by the Company, the Participant agrees to the terms and conditions of the Plan, and these Terms and Conditions, including any special terms and conditions for the Participant's country set forth in the appendix to these Terms and Conditions (the "***Appendix***"). Certain capitalized terms used but not defined in these Terms and Conditions have the meanings set forth in the Plan.

Part A

TERMS AND CONDITIONS FOR ALL PARTICIPANTS OUTSIDE THE UNITED STATES

This Part A includes special terms and conditions that govern your participation in the Plan if you reside and/or work outside the United States, and is subject to Part B.

1. RESPONSIBILITY FOR TAXES. You acknowledge that, regardless of any action taken by the Company or your employer, the ultimate liability for all income tax, social insurance, payroll tax, fringe benefit tax, payment on account or other tax-related items related to your participation in the Plan and legally applicable to you ("***Tax-Related Items***") is and remains your responsibility and may exceed the amount actually withheld by the Company or your employer. You further acknowledge that the Company and/or your employer (a) make no representations or undertakings regarding the treatment of any Tax-Related Items in connection with any aspect of the Plan, including, without limitation, the grant of the Purchase Rights, the purchase and issuance of shares of Common Stock, the subsequent sale of shares of Common Stock or the receipt of any dividends; and (b) do not commit to and are under no obligation to structure the terms of the grant of the Purchase Rights or any aspect of the Plan to reduce or eliminate your liability for Tax-Related Items or to achieve any particular tax result. Further, if you are subject to Tax-Related Items in more than one jurisdiction, you acknowledge that the Company and/or your employer (or former employer, as applicable) may be required to withhold or account for Tax-Related Items in more than one jurisdiction.

Prior to the relevant taxable or tax withholding event, as applicable, you agree to make adequate arrangements satisfactory to the Company and/or your employer to satisfy all Tax-Related Items. In this regard, you authorize the Company and/or your employer to satisfy any withholding obligations with regard to Tax-Related Items by one or a combination of the following: (i) withholding from your wages or compensation payable to you by the Company and/or your employer; (ii) withholding from the proceeds from the sale of shares of Common Stock acquired under the Plan, either through a voluntary sale or through a mandatory sale arranged by the Company (on your behalf pursuant to this authorization without further consent); or (iii) withholding in shares to be issued to you at purchase**.**

Depending on the withholding method, the Company may withhold or account for Tax-Related Items by considering applicable minimum statutory withholding amounts or other applicable withholding rates, including maximum applicable rates, in which case you will receive a refund of any over-withheld amount in cash and will have no entitlement to the equivalent amount in shares. If the obligation for Tax-Related Items is satisfied by withholding in shares, for tax purposes, you will be deemed to have been issued the full number of shares subject to the exercised Purchase Rights, notwithstanding that some shares are held back solely for the purpose of paying the Tax-Related Items.

Finally, you agree to pay to the Company or your employer any amount of Tax-Related Items that the Company or your employer may be required to withhold or account for as a result of your participation in the Plan and that cannot be satisfied by the means previously described. The Company may refuse to issue or deliver the shares of Common Stock or the proceeds from the sale of shares of Common Stock if you fail to comply with your obligations in connection with the Tax-Related Items.

2. NATURE OF THE PLAN. By enrolling and participating in the Plan, you acknowledge, understand and agree that:

(a)the Plan is established voluntarily by the Company, is discretionary in nature and may be amended, modified, suspended or terminated by the Company at any time, to the extent permitted in the Plan;

(b)the grant of the Purchase Rights is voluntary and occasional and does not create any contractual or other right to receive future grants of purchase rights, or benefits in lieu of purchase rights, even if purchase rights have been granted in the past;

(c)all decisions with respect to future grants of purchase rights or other grants, if any, will be at the sole discretion of the Company;

(d)the grant of the Purchase Rights and your participation in the Plan shall not create a right to employment or be interpreted as forming an employment or service contract with the Company or any Related Corporation, and shall not interfere with the ability of the Company or any Related Corporation, including your employer, to terminate your employment at any time. Notwithstanding the foregoing, references to "at will" employment in the Plan shall be disregarded;

(e)you are voluntarily participating in the Plan;

(f)the Plan and the shares of Common Stock purchased under the Plan, and the income and value of same, are not intended to replace any pension rights or compensation;

(g)the Plan and the shares of Common Stock purchased under the Plan, and the income and value of same, are not part of normal or expected compensation for any purposes including, without limitation, calculating any severance, resignation, termination, redundancy, dismissal, end-of-service payments, bonuses, long-service awards, pension, holiday pay or retirement or welfare benefits or similar payments;

(h)the future value of the shares of Common Stock subject to the Purchase Rights is unknown, indeterminable, and cannot be predicted with certainty;

(i)the value of the shares of Common Stock purchased under the Plan may increase or decrease in the future, even below the purchase price of the shares;

(j)no claim or entitlement to compensation or damages shall arise from forfeiture of the Purchase Rights under the Plan resulting from termination of your employment (for any reason whatsoever, whether or not later found to be invalid or in breach of employment laws in the jurisdiction where you are employed or the terms of your employment agreement, if any) and in consideration of the grant of the Purchase Rights and the issuance of shares of Common Stock at purchase under the Plan to which you are otherwise not entitled, you irrevocably agree never to institute any claim against the Company or any Related Corporation, including your employer, waive your ability, if any, to bring any such claim, and release the Company and any Related Corporation, including your employer, from any such claim; if, notwithstanding the foregoing, any such claim is allowed by a court of competent jurisdiction, then, by participating in the Plan, you shall be deemed irrevocably to have agreed not to pursue such claim and agree to execute any and all documents necessary to request dismissal or withdrawal of such claim;

(k)for purposes of your participation in the Plan, your employment will be considered terminated as of the date you are no longer actively employed by the Company or a Related Corporation (regardless of the reason for such termination and regardless of whether later found to be invalid or in breach of employment laws in the jurisdiction where you are employed or the terms of your employment agreement, if any), and your right to participate in the Plan and your Purchase Rights, if any, will terminate effective as of your last day of active employment and will not be extended by any notice period (*e.g.*, active employment would not include any contractual notice or any period of "garden leave" or similar period mandated under employment laws in the jurisdiction where you are employed or the terms of your employment agreement, if any); the Board shall have exclusive discretion to determine when you are no longer actively employed for purposes of your participation in the Plan (including whether you are actively employed while on a leave of absence);

(l)unless otherwise provided in the Plan or by the Company in its discretion, the Purchase Rights do not create any entitlement to have the Plan or any Purchase Rights, transferred to, or assumed by, another company nor to be exchanged, cashed out or substituted for, in connection with any corporate transaction affecting the shares of Common Stock;

(m)neither the Company nor any Related Corporation, including your employer, shall be liable for any foreign exchange rate fluctuation between your local currency and the United States Dollar that may affect the value of the shares of Common Stock or any amounts due pursuant to your participation in the Plan; and

(n)Purchase Rights personal to you are exercisable during your lifetime only by you. Purchase Rights are only transferable on your death to your personal representative.

3. NO ADVICE REGARDING GRANT. Neither the Company nor any Related Corporation is providing any tax, legal or financial advice, nor is the Company or any Related Corporation making any recommendations regarding your participation in the Plan, or your purchase or sale of the underlying shares of Common Stock. You are hereby advised to consult with your own personal tax, legal and financial advisors regarding your participation in the Plan before taking any action related to the Plan.

4. ELECTRONIC DELIVERY AND ACCEPTANCE. The Company may, in its sole discretion, decide to deliver any documents related to current or future participation in the Plan by electronic means. You hereby consent to receive such documents by electronic delivery and agree to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

5. INSIDER-TRADING/MARKET-ABUSE LAWS. You understand that, depending on your country, you may be subject to insider-trading restrictions and/or market-abuse laws, which may affect your ability to purchase or sell shares of Common Stock under the Plan during such times as you are considered to have "inside information" regarding the Company (as defined by the laws in your country). Any restrictions under these laws or regulations are separate from and in addition to any restrictions that may be imposed under any applicable Company insider-trading policy. You are responsible for complying with any applicable restrictions, so you are advised to speak to your personal legal advisor for further details regarding any applicable insider-trading and/or market-abuse laws in your country.

6. DATA PRIVACY.

(a)You explicitly and unambiguously acknowledge and consent to the collection, use and transfer, in electronic or other form, of your personal data as described in this document by and among, as applicable, your employer, the Company and its Related Corporations for the exclusive purpose of implementing, administering and managing your participation in the Plan. You understand that the Company, its Related Corporations and your employer hold certain personal information about you, including, but not limited to, name, home address and telephone number, date of birth, social security number (or other identification number), salary, nationality, job title, any shares of stock or directorships held in the Company, details of all options or any other entitlement to shares of stock awarded, canceled, purchased, exercised, vested, unvested or outstanding in your favor for the purpose of implementing, managing and administering the Plan ("***Data***"). You understand that the Data may be transferred to any third parties assisting in the implementation, administration and management of the Plan, that these recipients may be located in your country or elsewhere, in particular in the US, and that the recipient country may have different data privacy laws providing less protections of your personal data than your country. You may request a list with the names and addresses of any potential recipients of the Data by contacting the stock plan administrator at the Company (the "***Stock Plan Administrator***"). You acknowledge that the recipients may receive, possess, process, use, retain and transfer the Data, in electronic or other form, for the purposes of implementing, administering and managing your participation in the Plan, including any requisite transfer of such Data, as may be required to a broker or other third party with whom you may elect to deposit any shares of Common Stock acquired upon the exercise of your option. You understand that Data will be held only as long as is necessary to implement, administer and manage your participation in the Plan. You may, at any time, view the Data, request additional information about the storage and processing of the Data, require any necessary amendments to the Data or refuse or withdraw the consents herein, in any case without cost, by contacting the Stock Plan Administrator in writing.

(b)For the purposes of operating the Plan in the European Union and the United Kingdom, the Company will collect and process information relating to you in accordance with the privacy notice from time to time in force.

7. LANGUAGE. You acknowledge that you are sufficiently proficient in the English language, or have consulted with an advisor who is sufficiently proficient in English, so as to allow you to understand these Terms and Conditions and those of the Plan. If you have received these Terms and Conditions, or any other document related to the Plan translated into a language other than English and if the meaning of the translated version is different than the English version, the English version will control.

8. FOREIGN ASSET/ACCOUNT, EXCHANGE CONTROL AND TAX REPORTING. You may be subject to foreign asset/account, exchange control and/or tax reporting requirements as a result of the acquisition, holding and/or transfer of shares of Common Stock or cash (including dividends and the proceeds arising from the sale of shares of Common Stock) derived from your participation in the Plan in, to and/or from a brokerage/bank account or legal entity located outside your country. The applicable laws in your country may require that you report such accounts, assets and balances therein, the

value thereof and/or the transactions related thereto to the applicable authorities in such country. You may also be required to repatriate sale proceeds or other funds received as a result of your participation in the Plan to your country through a designated bank or broker within a certain time after receipt. You acknowledge that it is your responsibility to be compliant with such regulations and you are encouraged to consult with your personal legal advisor for any details.

9. CHOICE OF LAW. The interpretation, performance and enforcement of these Terms and Conditions shall be governed by the laws of the State of Delaware without regard to that state's conflicts of laws rules.

Part B

Additional Terms and Conditions for Participants Resident in the Jurisdictions listed Below

This Part B includes special terms and conditions and notification obligations that govern your participation in the Plan if you reside and/or work in one of the jurisdictions listed below.

Denmark

Stock Option Act. You acknowledge receipt of the employer statement in Danish (a copy of which is appended hereto at Part C) which sets forth additional terms of the Purchase Rights to the extent the Danish Stock Options Act applies.

Foreign Asset / Account Reporting. If you establish an account holding cash or shares of Common Stock outside Denmark, you must report the account to the Danish Tax Administration.

France

Eligibility. The following amends Section 5(a) of the Plan:

(a) Purchase Rights may be granted only to Employees of the Company or, as the Board may designate in accordance with Section 2(b), to Employees of a Related Corporation or an Affiliate. Except as provided in Section 5(b), an Employee will not be eligible to be granted Purchase Rights unless, on the Offering Date, the Employee has been in the employ of the Company, the Related Corporation or the Affiliate, as the case may be, for such continuous period preceding such Offering Date as the Board may require, but in no event will the required period of continuous employment be equal to or greater than two years.

Language Acknowledgement. You confirm having read and understood the documents relating to the Plan, the Offering Document and the Terms and Conditions, including all terms and conditions included therein, which were provided in the English language. You accept the terms of those documents accordingly.

Consentement Relatif à la Langue Utilisée. *Le Participant confirme avoir lu et compris le Plan et cette convention («Offering Document») et les Terms et Conditions, incluant tous leurs terms et conditions, qui ont été transmis en langue anglaise. Le Participant accepte les dispositions de ces documents en connaissance de cause*.

Foreign Asset/Account Reporting Information. If you are a French resident and maintain a foreign bank account, you must report such account to the French tax authorities when filing your annual tax return. Failure to comply with this requirement could trigger significant penalties and you should consult with your personal advisor to ensure proper compliance with applicable reporting requirements in France.

Exchange Control Information. Cross-border payments towards or from another EU member in excess of EUR €10,000 must be reported to the French Custom Authorities. However, this reporting obligation does not apply to wire transfers made via banks or financial institutions. So, given that the Plan is established by a US company and that, in any case, all money transfers will be made through banks or financial institutions, this reporting obligation should not apply here.

Germany

Exchange Control Information. Cross-border payments in excess of EUR €12,500 must be reported monthly to the German Federal Bank (*Bundesbank*). In case of payments in connection with securities (including proceeds realized upon the sale of shares of Common Stock or the receipt of dividends), the report must be made by the 5th day of the month following the month in which the payment was received. The report must be filed electronically and the form of report (*Allgemeine Meldeportal Statistik*) can be accessed via the Bundesbank's website (www.bundesbank.de), in both German and English. You are responsible for filing this report.

Tax Reporting. You must report and pay any capital gains tax liability that arises in connection with the sale of shares of Common Stock acquired under the Plan. In general the statutory deadline of filing annual income tax returns for taxpayers is July 31 of the calendar year following the respective fiscal year. Payment periods of due tax amounts are determined in view of the competent tax office. You should consult with your personal tax advisor to ensure that you are properly complying with applicable reporting requirements in Germany.

Italy

Plan Acknowledgement. You acknowledge that you have read and specifically and expressly approve the following sections of the Terms and Conditions: Responsibility for Taxes; Nature of the Plan; No Advice Regarding Grant; Data Privacy; Language; Choice of Law; and the Italy country-specific terms and conditions of this Appendix.

Foreign Asset/Account Reporting Information. If you are an Italian resident and at any time during the fiscal year hold investments or financial assets outside of Italy (e.g., cash or shares of Common Stock) which may generate income taxable in Italy (or, under Italian money laundering provisions if you are the beneficial owner of such an investment or asset, even if you do not directly hold the investment or asset), you are required to report such investments or assets on your annual tax return for such fiscal year (on UNICO Form, RW Schedule) or on a special form if you are not required to file a tax return.

Foreign Financial Assets Tax. The fair market value of any shares of Common Stock held outside of Italy is subject to a foreign assets tax. Financial assets include shares of Common Stock acquired under the Plan. The taxable amount will be the fair market value of the financial assets assessed at the end of the calendar year. You should consult with your personal tax advisor about the foreign financial assets tax.

Netherlands

Securities Law Information. The grants under the ESPP are exempt or excluded from the requirement to publish a prospectus under the EU Prospectus Regulation (2017/1129) that has direct effect in the Netherlands. The grants fall outside the supervision of the Authority for the Financial Markets ("***AFM***") and no prospectus is required for this activity.

Spain

Nature of Participation. The following provision supplements Section 2 of the Terms and Conditions:

In accepting the Purchase Rights, you consent to participate in the Plan and acknowledge that the Plan was made available to you and that you read a copy of the Plan and you consent to the Terms and Conditions and acknowledge having received and read a copy of the Terms and Conditions.

You understand and agree that, as a condition of participating in the Plan, if you are no longer an Employee for any reason or for no reason (subject to any post-employment participation period required by law) or (ii) are otherwise no longer eligible to participate (including for the reasons listed below) your participation will automatically result in the forfeiture of the Purchase Rights as of such date.

In particular, you understand and agree that the Purchase Rights, will be forfeited without entitlement to the underlying shares of Common Stock or to any amount as indemnification in the event you are no longer an Employee for any reason or for no reason (subject to any post-employment participation period required by law) or (ii) are otherwise no longer eligible to participate prior to purchase by reason of, including, but not limited to: resignation, disciplinary dismissal adjudged to be with cause, disciplinary dismissal adjudged or recognized to be without good cause (i.e., subject to a "*despido improcedente*"), individual or collective layoff on objective grounds, adjudged or recognized to be with or without good cause, material modification of the terms of employment under Article 41 of the Workers' Statute, relocation under Article 40 of the Workers' Statute, Article 50 of the Workers' Statute, unilateral withdrawal by the Company, and under Article 10.3 of Royal Decree 1382/1985.

Furthermore, you understand that the Company has unilaterally, gratuitously and discretionally decided to offer Purchase Rights under the Plan to Eligible Employees. The decision is a limited decision that is entered into upon the express assumption and condition that any grant will not economically or otherwise bind the Company on an ongoing basis. Consequently, you understand that the Purchase Rights are granted on the assumption and condition that the Purchase Rights, and the shares of Common Stock underlying the Purchase Right, shall not become a part of any employment or service contract with the Purchase Right, and shall not be considered a mandatory benefit, salary for any purposes (including severance compensation) or any other right whatsoever. In addition, you understand that the Purchase Rights would not be granted to you but for the assumptions and conditions referred to above; thus, you acknowledge and freely accept that should any or all of the assumptions be mistaken or should any of the conditions not be met for any reason, then any Purchase Rights granted to you shall be null and void.

Exchange Control Information. The acquisition, ownership and sale of shares of Common Stock under the Plan must be declared for statistical purposes to the Spanish *Dirección General de Comercio e Inversiones* (the "***DGCI***"), the Bureau for Commerce and Investments, which is a department of the Ministry of Industry, Tourism and Commerce. Generally, the declaration must be made in January for shares owned as of December 31 of the prior year and/or shares acquired or disposed of during the prior year; however, if the value of shares acquired or disposed of or the amount of the sale proceeds exceeds EUR €1,502,530 (or if you hold 10% or more of the share capital of the Company), the declaration must be filed within one month of the acquisition or disposition, as applicable.

In addition, you may be required to electronically declare to the Bank of Spain any foreign accounts (including brokerage accounts held abroad), any foreign instruments (including shares of Common Stock acquired under the Plan), and any transactions with non-Spanish residents (including any payments of shares of Common Stock made pursuant to the Plan), depending on the balances in such accounts together with the value of such instruments as of December 31 of the relevant year, or the volume of transactions with non-Spanish residents during the relevant year.

Foreign Asset/Account Reporting Information. To the extent that you hold rights or assets (i.e., cash or shares of Common Stock held in a bank or brokerage account) outside Spain with a value in excess of EUR €50,000 per type of right or asset (e.g., shares of Common Stock, cash, etc.) as of December 31 each year, you are required to report information on such rights and assets on your tax return for such year. After such rights or assets are initially reported, the reporting obligation will only apply for subsequent years if the value of any previously-reported rights or assets increases by more than EUR €20,000. You should consult with your personal tax and legal advisors to ensure that you are properly complying with your reporting obligations.

Securities Law Information. No "offer of securities to the public", as defined under Spanish law, has taken place or will take place in the Spanish territory in connection with the Offering of the Purchase Rights. Neither the Plan nor any related Plan document has been nor will it be registered with the *Comisión Nacional del Mercado de Valores*, and does not constitute a public offering prospectus.

Switzerland

Sole Contact and Contractual Partner Information. You acknowledge that the Offering, the Offering Document and the Plan and your participation in the Plan does not create any claims against the Affiliate employing you, either directly or indirectly. Your sole contract and sole contractual partner regarding Offering, the Offering Document and the Plan is the Company and the granted Purchase Rights and shares of Common Stock do not form part of your contractual compensation.

Contributions. You herewith explicitly authorize the Affiliate employing you to deduct the agreed Contributions from any compensation owed to you by such Affiliate. You further acknowledge that such funds are not held separately from any other funds of the Company and/or such Affiliate.

Securities Law Information. The Offering of Purchase Rights and the purchase of shares of Common Stock is not intended to be publicly offered in or from Switzerland. Because it is considered a private offering, it is not subject to securities registration in Switzerland. Neither this document nor any other materials relating to the Purchase Rights: (i) constitutes a prospectus according to articles 35 et seq. of the Swiss Federal Act on Financial Services ("***FinSA***"); (ii) may be publicly distributed or otherwise made publicly available in Switzerland to any person other than a participant; or (iii) has been or will be filed with, approved or supervised by any Swiss reviewing body according to article 51 FinSA or any Swiss regulatory authority, including the Swiss Financial Market Supervisory Authority ("***FINMA***").

Grant. The grant of the Purchase Rights and shares of Common Stock is made on a voluntary gratuity (*Gratifikation; gratification*) within the meaning of Article 322d Swiss Code of Obligations (CO) as determined at the Company's sole discretion which you have no entitlement to and which does not constitute an entitlement for a grant of further Purchase Rights, shares of Common Stock other equities in the future.

Purchase Date. You acknowledge and confirm that the Purchase Rights are fully discretionary and that before any

Purchase Date no accrued Contributions will be used to purchase shares of Common Stock. If you cease to be employed by the Affiliate employing you on the Purchase Date, no purchase of shares of Common Stock will occur.

Social Security and Tax: You herewith directly authorize the Affiliate employing you to make all (if any) applicable social security, insurance and tax deductions resulting from the grant of Purchase Rights, the purchase of shares of Common Stock and/or the sale of shares of Common Stock from any compensation owed to you by your employer, subject to any statutory limitations. If your compensation shall not be sufficient to cover such social security, insurance and tax liabilities, you will indemnify the Affiliate employing you upon first demand.

Language Acknowledgement. You confirm that you have read and understood the Offering, the Offering Document and the Plan and the documents relating to the Plan, with all terms and conditions included therein, which were provided in the English language only. You confirm that you have sufficient language capabilities to understand these terms and conditions in full.

Sie bestätigen, dass Sie das Angebot, die Angebotsunterlagen, den Plan sowie die dazugehörigen Dokumente, mit all den darin enthaltenen Bedingungen und Voraussetzungen, welche in englischer Sprache verfasst sind, gelesen und verstanden haben. Sie bestätigen, dass Ihre Sprachkenntnisse genügend sind, um diese Bedingungen und Voraussetzungen zu verstehen.

Vous confirmez avoir lu et compris l'offre, le document d'offre et le plan ainsi que les documents relatifs au plan, avec toutes les conditions qui y sont incluses, qui ont été fournies en langue anglaise uniquement. Vous confirmez que vous avez des capacités linguistiques suffisantes pour comprendre l'intégralité de ces termes et conditions.

No Right against Employer. You expressly acknowledge that you shall not have any right or claim under the Offering, the Offering Document or the Plan against the Affiliate employing you. You expressly acknowledge and agree that you only have any right and claim against the Company as set out under the Plan and the Offering Document.

Governing Law and Jurisdiction. You expressly acknowledge and agree to the Governing Law and Jurisdiction clause in the Plan and the Offering Document and accept that Swiss law does not apply and that Swiss courts do not have any jurisdiction in regard to any claims under the Plan or the Offering Document.

United Kingdom

Responsibility for Taxes. The following supplements Section 1 of the Terms and Conditions:

As a condition of your participation in the Plan, you unconditionally and irrevocably agree:

(a)to place the Company in funds and indemnify the Company in respect of (1) all liability to UK income tax which the Company is liable to account for on your behalf directly to HM Revenue & Customs; (2) all liability to national insurance contributions which the Company is liable to account for on your behalf to HM Revenue & Customs (including, to the extent permitted by law, secondary class 1 (employer's) national insurance contributions for which you are liable and hereby agree to bear); and (3) all liability to national insurance contributions for which the Company is liable and which are formally transferred to you, which arises as a consequence of or in connection with the acquisition by you of shares of Common Stock under the Plan (the "***UK Tax Liability***"); or

(b)to permit the Company to sell at the best price which it can reasonably obtain such number of shares of Common Stock allocated or allotted to you under the Plan as will provide the Company with an amount equal to the UK Tax Liability; and to permit the Company to withhold an amount not exceeding the UK Tax Liability from any payment made to you (including, but not limited to salary); and

(c)if so required by the Company, and, to the extent permitted by law, to enter into a joint election or other arrangements under which the liability for all or part of such employer's national insurance contributions liability is transferred to you; and

(d)if so required by the Company, to enter into a joint election within Section 431 of (UK) Income Tax (Earnings and Pensions) Act 2003 ("***ITEPA***") in respect of computing any tax charge on the acquisition of "restricted securities" (as defined in Section 423 and 424 of ITEPA); and

(e)to sign, promptly, all documents required by the Company to effect the terms of this provision, and references in this provision to "the Company" shall, if applicable, be construed as also referring to any your employer or any other relevant affiliate of the Company.

PART C

DANISH EMPLOYER STATEMENT

ARBEJDSGIVERERKLÆRING/EMPLOYER STATEMENT

I henhold til § 3, stk. 1, i lov om brug af køberet eller tegningsret m.v. i ansættelsesforhold ("Aktieoptionsloven") er du berettiget til at modtage følgende oplysninger om Mirum Pharmaceuticals, Inc.'s ("Selskabet") 2021 Employee Stock Purchase Plan ("Aktiekøbsprogrammet") i en særskilt skriftlig erklæring. Tildelingen af Aktiekøbsrettigheder sker som følge af din ansættelse i [employer name].

Pursuant to section 3(1) of the Danish Act on the Use of Rights to Purchase or Subscribe for Shares etc. in Employment Relationships (the "Stock Option Act"), you are entitled to receive the following information regarding Mirum Pharmaceuticals, Inc.'s (the "Company") 2019 Employee Stock Purchase Plan (the "Plan") in a separate written statement. The Purchase Right is given due to your employment at [employer name].

Denne erklæring indeholder kun de oplysninger som er krævet i henhold til Aktieoptionsloven. Øvrige betingelser for dine Aktiekøbsrettigheder er detaljeret beskrevet i Aktiekøbsprogrammet. Hvis der er uoverensstemmelser mellem indholdet af denne erklæring og Aktiekøbsprogrammet, finder Aktiekøbsprogrammet anvendelse.

This statement contains the information mentioned in the Stock Option Act only, while the other conditions of the Purchase Right are described in detail in the Plan. In the event that there is any discrepancy between the substance put forward in this statement and the Plan, the Plan shall apply.

1. **TIDSPUNKTET FOR TILDELING AF RETTEN TIL AT KØBE AKTIER**

TIME OF GRANT OF THE RIGHT TO PURCHASE SHARES

Tidspunktet for Tildelingsdatoen og Købsdatoen fastsættes af Bestyrelsen i Tildelingsdokumentet.

The Offering Date and the Purchase Date shall be selected of the Board in the Offering Document.

2. **KRITERIER ELLER BETINGELSER FOR TILDELING AF RETTEN TIL SENERE AT KØBE AKTIER**

TERMS OR CONDITIONS FOR GRANT OF A RIGHT TO FUTURE PURCHASE OF STOCK

Aktiekøbsprogrammet tilbydes efter Selskabets bestyrelses frie skøn.

The Plan is offered at the discretion of the Company's Board of Directors.

3. **KØBSTIDSPUNKTET**

PURCHASE DATE

Hvis du har ret til at købe aktier i Selskabet på en Købsdato vil aktier automatisk blive købt til dig ved anvendelse af dine opsparede lønfradrag.

If you have a right to purchase shares of stock in the Company on the Purchase Date, shares of Common Stock will automatically be purchased for you with the amount of your accumulated payroll deductions.

Hvis der købes aktier for dig ved udgangen af en købsperiode, vil antallet af aktier afhænge af købsprisen og det beløb, du har opsparet ved dine lønfradrag.

The number of shares purchased will depend on the Purchase Price and the amount of your accumulated payroll deductions.

Du vil umiddelbart blive ejer af de aktier, du har købt for dine opsparede lønfradrag, og du kan sælge de aktier, du har købt i henhold til Aktieprogrammet når som helst.

You will be the immediate owner of the common stock purchased with your accumulated payroll deductions and you may sell your shares of common stock purchased under the Plan at any time.

4. **KØBSPRIS**

Købsprisen pr. aktie udstedt i henhold til udnyttelsen af en option vil ikke være mindre end det mindste af:

i.et beløb svarende til 85% af Markedsværdien af aktierne på Tildelingstidspunktet; eller
ii.et beløb svarende til 85% af Markedsprisen af aktierne på Købstidspunktet.

PURCHASE PRICE

The purchase price of stock issued pursuant to the exercise of an option will be not less than the lesser of:

i.an amount equal to 85% of the Fair Market Value of the shares of Common Stock on the Offering Date; or
ii.an amount equal to 85% of the Fair Market Value of the shares of Common Stock on the applicable Purchase Date.

5. **DIN RETSSTILLING I FORBINDELSE MED FRATRÆDEN**

Ved din fratræden vil dine Aktiekøbsrettigheder blive behandlet som beskrevet i Aktiekøbsprogrammet. Aktiekøbsrettigheder vil bortfalde med omgående virkning i forbindelse med fratræden.

YOUR RIGHTS UPON TERMINATION OF EMPLOYMENT

The terms which regulate the treatment of your Purchase Right upon termination of employment are set out in the Plan. Upon your termination of employment for any reason, any Purchase Right shall terminate and be forfeited immediately.

6. **DE ØKONOMISKE ASPEKTER AF DELTAGELSE I AKTIEKØBSPROGRAMMET**

Udover de lønfradrag der vil blive foretaget, når du deltager i Aktiekøbsprogrammet, har Aktiekøbsprogrammet ingen umiddelbare økonomiske konsekvenser for dig. Værdien af køberettighederne og værdien af de aktier, der købes til dig i henhold til Aktiekøbsprogrammet indgår ikke i beregningen af feriepenge, pensionsbidrag eller øvrige vederlagsafhængige ydelser.

Aktier er et finansielt instrument, og investering i aktier vil altid være forbundet med en risiko. Muligheden for gevinst på det tidspunkt, hvor du sælger dine aktier, vil ikke kun være afhængig af Selskabets økonomiske udvikling, men bl.a. også af den generelle udvikling på aktiemarkedet. Efter du har købt aktier, kan værdien af aktierne endvidere falde, endda til en værdi, der er lavere end din købspris.

FINANCIAL ASPECTS OF PARTICIPATING IN THE PLAN

Aside from the payroll deductions which will start after you enrol in the Plan, the Plan offering has no immediate financial consequences for you. The value of the purchase rights and the value of the shares purchased for you under the Plan are not taken into account when calculating holiday allowances, pension contributions or other statutory consideration calculated on the basis of salary.

Shares of stock are financial instruments and investing in stocks will always have financial risk. The possibility of profit at the time you sell your shares will not only be dependent on the Company's financial development, but inter alia also on the general development on the stock market. In addition, after you purchase shares, the shares could decrease in value even below the purchase price.

Sted/Place:
Dato/Date:

_____

På vegne af Selskabet/On behalf of the Company

Exhibit 10.14

MIRUM PHARMACEUTICALS, INC.

STOCK OPTION GRANT NOTICE - INTERNATIONAL
(2020 INDUCEMENT PLAN)

Mirum Pharmaceuticals, Inc. (the "***Company***"), pursuant to its 2020 Inducement Plan (the "***Plan***"), hereby grants to Optionholder an option to purchase the number of shares of the Company's Common Stock set forth below. This option is subject to all of the terms and conditions as set forth in this Stock Option Grant Notice, in the Option Agreement, the Plan and the Notice of Exercise, all of which are attached hereto and incorporated herein in their entirety, including any special terms and conditions for the Optionholder's country set forth in the attached appendix (the "Appendix"). Capitalized terms not explicitly defined herein but defined in the Plan or the Option Agreement will have the same definitions as in the Plan or the Option Agreement. If there is any conflict between the terms in this Stock Option Grant Notice and the Plan, the terms of the Plan will control.

Optionholder:	_____
Date of Grant:	_____
Vesting Commencement Date:	_____
Number of Shares Subject to Option:	_____
Exercise Price (Per Share) (US$):	_____
Total Exercise Price (US$):	_____
Expiration Date:	_____

Type of Grant: Nonstatutory Stock Option

Exercise Schedule: [Same as Vesting Schedule]

Vesting Schedule: **[**_____, subject to Optionholder's Continuous Service as of each such date.]

Payment: By one or a combination of the following items (described in the Option Agreement):

☐ By cash, check, bank draft or money order payable to the Company
☐ Pursuant to a Regulation T Program if the shares are publicly traded
☐ By delivery of already-owned shares if the shares are publicly traded
☐ Subject to the Company's consent at the time of exercise, by a "net exercise" arrangement

Additional Terms/Acknowledgements: Optionholder acknowledges receipt of, and understands and agrees to, this Stock Option Grant Notice, the Option Agreement (including the Appendix) and the Plan. Optionholder acknowledges and agrees that this Stock Option Grant Notice and the Option Agreement (including the Appendix) may not be modified, amended or revised except as provided in the Plan. Optionholder further acknowledges that as of the Date of Grant, this Stock Option Grant Notice, the Option Agreement (including the Appendix), and the Plan set forth the entire understanding between Optionholder and the Company regarding this option award and supersede all prior oral and written agreements, promises and/or representations on that subject with the exception of, if applicable, (i) equity awards previously granted and delivered to Optionholder, (ii) any compensation recovery policy that is adopted by the Company or is otherwise required by applicable law and (iii) any written employment agreement, severance agreement, offer letter or other written agreement entered into between the Company and Participant specifying the terms that should govern this specific option. By accepting this option, Optionholder consents to receive such documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

MIRUM PHARMACEUTICALS, INC.	**OPTIONHOLDER:**
By: _____	_____
Signature	Signature
Title: _____	Date: _____
Date: _____	

ATTACHMENTS: Option Agreement (including the Appendix), 2020 Inducement Plan and Notice of Exercise

not exercise your option by delivery to the Company of Common Stock if doing so would violate the provisions of any law, regulation or agreement restricting the redemption of the Company's stock.

(c) Subject to the consent of the Company at the time of exercise, by a "net exercise" arrangement pursuant to which the

your option and will not be exercisable thereafter if those shares (i) are used to pay the exercise price pursuant to the "net exercise," (ii) are delivered to you as a result of such exercise, and (iii) are withheld to satisfy your tax and/or social security withholding obligations.

4. WHOLE SHARES. You may exercise your option only for whole shares of Common Stock.

5. SECURITIES LAW COMPLIANCE. In no event may you exercise your option unless the shares of Common Stock issuable upon

exercise would not be in material compliance with such laws and regulations (including any restrictions on exercise required for compliance with Treas. Reg. 1.401(k)-1(d)(3), if applicable).

6. TERM. You may not exercise your option before the Date of Grant or after the expiration of the option's term. The term of your

(a) immediately upon the termination of your Continuous Service for Cause;

(b) three (3) months after the termination of your Continuous Service for any reason other than Cause, your Disability or your death (except as otherwise provided in Section 6(d) below); *provided, however,* that if during any part of such three (3) month period

the termination of your Continuous Service; *provided further,* if during any part of such three (3) month period, the sale of any Common Stock received upon exercise of your option would violate the Company's insider trading policy, then your option will not expire until the earlier of the Expiration Date or until it has been exercisable for an aggregate period of three (3) months after the termination of your Continuous Service during which the sale of the Common Stock received upon exercise of your option would not be in violation of the

(c) twelve (12) months after the termination of your Continuous Service due to your Disability (except as otherwise

(d) eighteen (18) months after your death if you die either during your Continuous Service or within three (3) months after

(e) the Expiration Date indicated in your Grant Notice; or

(f) the day before the tenth (10th) anniversary of the Date of Grant.

7. EXERCISE.

(a) You may exercise the vested portion of your option (and the unvested portion of your option if your Grant Notice so

additional documents as the Company may then require.

(b) By exercising your option you agree that, as a condition to any exercise of your option, the Company may require you

8. TRANSFERABILITY. Your option is not transferable, except by will or by the laws of descent and distribution, and is exercisable

9. OPTION NOT A SERVICE CONTRACT. Your option is not an employment or service contract, and nothing in your option will be

Consultant for the Company or an Affiliate. By accepting your option you acknowledge, understand and agree that:

(a) the Plan is established voluntarily by the Company, it is discretionary in nature, and may be amended, suspended or

(b) the grant of your option is voluntary and occasional and does not create any contractual or other right to receive future

(c) your option and any shares of Common Stock acquired under the Plan on exercise of your option, and the income and

(d) the future value of the shares of Common Stock underlying the option is unknown, indeterminable, and cannot be

(e) neither the Company nor any Affiliate shall be liable for any foreign exchange rate fluctuation between your local

option or of any amounts due to you pursuant to the exercise of your option or the subsequent sale of any shares of Common Stock received;

(f) for purposes of the option, your Continuous Service will be considered terminated as of the date you are no longer

under the Plan, if any, and (ii) the period (if any) during which you may exercise the option after such termination of Continuous Service will terminate as of such date and in each instance will not be extended by any notice period or any period of "garden leave" or similar period mandated under employment laws in the jurisdiction where you are employed or the terms of your employment agreement, if any); and the Plan Administrator shall have the exclusive discretion to determine when you are no longer actively providing services for purposes of the

(g) no claim or entitlement to compensation or damages shall arise from forfeiture of this option resulting from the

waive your ability, if any, to bring any such claim, and release the Company and any Affiliate from any such claim; if, notwithstanding the foregoing, any such claim is allowed by a court of competent jurisdiction, then, by participating in the Plan, you shall be deemed irrevocably to have agreed not to pursue such claim and agree to execute any and all documents necessary to request dismissal or withdrawal of such claim.

10. WITHHOLDING OBLIGATIONS.

(a) At the time you exercise your option, in whole or in part, and at any time thereafter as requested by the Company, you

withholding obligations of the Company or an Affiliate, if any, which arise in connection with the exercise of your option.

(b) Upon your request and subject to approval by the Company, and compliance with any applicable legal conditions or

avoid classification of your option as a liability for financial accounting purposes). Notwithstanding the filing of such election, shares of Common Stock shall be withheld solely from fully vested shares of Common Stock determined as of the date of exercise of your option that are otherwise issuable to you upon such exercise. Any adverse consequences to you arising in connection with such share withholding procedure shall be your sole responsibility.

(c) You may not exercise your option unless the tax and/or social security withholding obligations of the Company and/or

11. TAX CONSEQUENCES. You hereby agree that the Company does not have a duty to design or administer the Plan or its other

12. NOTICES. Any notices provided for in your option or the Plan will be given in writing (including electronically) and will be

participate in the Plan by electronic means. By accepting this option, you consent to receive such documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

13. GOVERNING PLAN DOCUMENT. Your option is subject to all the provisions of the Plan, the provisions of which are hereby made

recoupment in accordance with The Dodd–Frank Wall Street Reform and Consumer Protection Act and any implementing regulations thereunder, any clawback policy adopted by the Company and any compensation recovery policy otherwise required by applicable law.

14. OTHER DOCUMENTS. You hereby acknowledge receipt of and the right to receive a document providing the information

15. EFFECT ON OTHER EMPLOYEE BENEFIT PLANS. The value of this option will not be included as compensation, earnings,

16. VOTING RIGHTS. You will not have voting or any other rights as a stockholder of the Company with respect to the shares to be

Moreover, if you relocate to one of the countries included therein, the terms and conditions for such country will apply to you to the extent

Appendix constitutes part of this Option Agreement.

* * *

This Option Agreement (including the Appendix) will be deemed to be signed by you upon the signing by you of the Stock Option Grant Notice to which it is attached.

Appendix

This Appendix includes special terms and conditions that govern the Option granted to you under the Plan if you reside and/or work in any country listed below.

The information contained herein is general in nature and may not apply to your particular situation, and you are advised to seek appropriate professional advice as to how the relevant laws in your country may apply to your situation. If you are a citizen or resident of a country other than the one in which you are currently working and/or residing, transfer employment and/or residency to another country after the date of grant, are a consultant, change employment status to a consultant position, or are considered a resident of another country for local law purposes, the Company shall, in its discretion, determine the extent to which the special terms and conditions contained herein shall be applicable to you. References to your employer shall include any entity that engages your services.

DENMARK

Stock Option Act. You acknowledge that you received the Employer Statement in Danish which sets forth additional terms of the option to the extent the Danish Stock Options Act applies.

Foreign Asset / Account Reporting Notification. If you establish an account holding cash or shares of Common Stock outside Denmark, you must report the account to the Danish Tax Administration.

FRANCE

Language Consent. The parties to the Option Agreement acknowledge that it is their express wish that the Option Agreement, as well as all documents, notices, and legal proceedings entered into, given or instituted pursuant hereto or relating directly or indirectly hereto, be drawn up in English.

Consentement relatif à la langue utilisée

Les parties reconnaissent avoir exigé que cette convention («Agreement») soit rédigée en anglais, ainsi que tous les documents, avis et procédures judiciaires, éxécutés, donnés ou intentés en vertu de, ou liés directement ou indirectement à la présente.

Term. Section 6(a) of the Option Agreement is deleted and replaced with the following:

6. TERM. You may not exercise your option before the Date of Grant or after the expiration of the option's term. Except as set forth in your Grant Notice and as set forth below under (a) to (e), the term of your option expires, subject to the provisions of Section 5(h) of the Plan, immediately upon the termination of your Continuous Service.

By exception, the term of your option expires upon the earliest of the following:

(a)three months after the termination of your Continuous Service in case of retirement or redundancy for economic reasons (except as otherwise provided in Section 6(d) below); *provided, however,* that if during any part of such three month period your option is not

Continuous Service; *provided further,* if during any part of such three (3) month period, the sale of any Common Stock received upon exercise of your option would violate

the Company's insider trading policy, then your option will not expire until the earlier of the Expiration Date or until it has been exercisable

received upon exercise of your option would not be in violation of the Company's insider trading policy;

(b) 12 months after the termination of your Continuous Service due to your Disability (except as otherwise provided in

(c) 18 months after your death if you die either during your Continuous Service or during the three month period referred to

(d) the Expiration Date indicated in your Grant Notice; or

(e) the day before the 10th anniversary of the Date of Grant.

Tax Reporting. The gain realized on the exercise of the options will be subject to income tax and reporting obligations: as a standard salary income, the exercise gain will be subject to social security contributions and income tax withholding by your employer (if any). Any income tax withheld by your employer is not final. Your final income tax will be assessed based on the annual income tax return you will file the year following the exercise of your options.

The gain realized upon the sale of the shares of Common Stock – if any - will also be subject to income tax and reporting obligation: you will be responsible for reporting the capital gain, if any, realized upon the sale of the shares, on the annual income tax return you will file the year following the sale of your shares. You will be liable to pay the corresponding tax to the French tax authorities.

Foreign Asset/Account Reporting Information. If you are a French resident and maintain a foreign bank account, you must report such account to the French tax authorities when filing your annual tax return. Failure to comply with this requirement could trigger significant penalties and you should consult with your personal advisor to ensure proper compliance with applicable reporting requirements in France.

Exchange Control Information. Cross-border payments towards or from another EU member in excess of €10,000 must be reported to the French Custom Authorities. However, this reporting obligation does not apply to wire transfers made via banks or financial institutions. So, given that the Plan is established by a US company and that, in any case, all money transfers will be made through banks or financial institutions, this reporting obligation should not apply here.

GERMANY

Term. Section 6(a) of the Option Agreement is deleted and replaced with the following:

> **6. TERM.** You may not exercise your Option before the commencement of its term or after its term expires. The term of your Option commences on the Date of Grant and expires upon the earliest of the following:
>
> **(a)** immediately upon the termination of your Continuous Service for Cause; this does not apply in case termination of your Continuous Service does not comply with the principle of good faith. In such case, the provisions set forth under the remainder of this Section 6 shall apply.

Securities Disclaimer. The participation in the Plan is exempt or excluded from the requirement to publish a prospectus under the EU Prospectus Directive as implemented in Germany.

Exchange Control Information. Cross-border payments in excess of €12,500 must be reported monthly to the German Federal Bank (*Bundesbank*). In the event that you make or receive a payment in excess of this amount, you are required to report the payment to Bundesbank electronically using the "General Statistics Reporting Portal" ("*Allgemeines Meldeportal Statistik*") available via Bundesbank's website (www.bundesbank.de).

Tax Reporting. You must report and pay any capital gains tax liability that arises in connection with the sale of shares acquired under the Plan. In general the statutory deadline of filing annual income tax returns for taxpayers is 31 July of the calendar year following the respective fiscal year. Payment periods of due tax amounts are determined in view of the competent tax office. You should consult with your personal tax advisor to ensure that you are properly complying with applicable reporting requirements in Germany.

ITALY

Stock Option Exercises. Due to regulatory requirements, notwithstanding Section 3 of the Option Agreement, you will be required to exercise the option using a cashless sell-all exercise method, pursuant to which all shares of Common Stock subject to the exercised option will be sold immediately upon exercise and the proceeds of sale, less the exercise price, any federal, state, local and foreign tax and/or social security withholding obligations of the Company or an Affiliate and broker's fees or commissions, will be remitted to you in cash in accordance with any applicable exchange control laws and regulations. You will not be permitted to hold shares after exercise. The Company reserves the right to provide additional methods of exercise depending on the development of local laws.

Plan Acknowledgement. You acknowledge that you have read and specifically and expressly approve the following sections of the Option Agreement: (9) Nature of Grant; (10) Withholding Obligations; (11) Tax Consequences; (19) Data Privacy; (20) Language; (23) Imposition of Other Requirements; and (24) Choice of Law; and the Italy country-specific terms and conditions of this Appendix.

Foreign Asset/Account Reporting Information. If you are an Italian resident and, during any fiscal year, hold investments or financial assets outside of Italy (*e.g.*, cash, shares of Common Stock) which may generate income taxable in Italy (or if you are the beneficial owner of such an investment or asset even if you do not directly hold the investment or asset), you are required to report such investments or assets on your annual tax return for such fiscal year (on UNICO Form, RW Schedule, or on a special form if you are not required to file a tax return).

Foreign Financial Assets Tax. The fair market value of any shares of Common Stock held outside of Italy is subject to a foreign assets tax. Financial assets include shares of Common Stock acquired under the Plan. The taxable amount will be the fair market value of the financial assets assessed at the end of the calendar year. You should consult with your personal tax advisor about the foreign financial assets tax.

NETHERLANDS

Prohibition Against Insider Trading. You should be aware of the European insider trading rules, which may affect the sale of shares of Common Stock acquired under the Plan. In particular, you may be prohibited from effecting certain share transactions if you have insider information regarding the Company. If it is uncertain whether the insider rules apply, the Company recommends that you consult with a legal advisor.

The Company cannot be held liable if you violate the insider trading rules applicable in the Netherlands. You are responsible for ensuring your compliance with these rules.

Under Article 14 of the Market Abuse Regulation ((EU) Regulation 596/2014), anyone who has "inside information" related to an issuing company is prohibited from effectuating a transaction in securities in or from the Netherlands. "Inside information" is defined as knowledge of specific information concerning the issuing company to which the securities relate or the trade in securities issued by such company, which has not been made public and which, if published, would reasonably be expected to affect the price of the securities, regardless of the development of the price. The insider could be any employee of the Company or an Affiliate in the Netherlands who has inside information as described herein.

Given the broad scope of the definition of inside information, certain participants may have inside information and thus are prohibited from making a transaction in securities in the Netherlands at a time when they have such inside information. By entering into this Option Agreement and participating in the Plan, you acknowledge having read and understood the notification above and acknowledge that it is your responsibility to comply with the Dutch insider trading rules, as discussed herein.

Securities Disclaimer. The grant of the Option is exempt or excluded from the requirement to publish a prospectus under the Prospectus Regulation ((EU) Regulation 2017/1129). The Options are not transferable and are not deemed to qualify as an offering of securities in the Netherlands within the meaning of the Prospectus Regulation. To the extent that a supervisory body would qualify the offering of Awards or its underlying securities as an offering of securities within the meaning of the Prospectus Regulation, such offering will only be made in reliance on Article 1(4) of the Prospectus Regulation provided that no such offering of securities shall require Mirum Pharmaceuticals, Inc. to publish a prospectus pursuant to Article 3 of the Prospectus Regulation.

SPAIN

Nature of Grant. The following provision supplements Section 9 of the Option Agreement:

In accepting the option, you consent to participate in the Plan and acknowledge that the Plan was made available to you and that you read a copy of the Plan and you consent to the terms and conditions of the Agreement and acknowledge having received and read a copy of the Option Agreement.

You understand and agree that, as a condition of the option grant, your termination of employment for any reason (including for the reasons listed below) will automatically result in the forfeiture of the option and loss of the shares of Common Stock that may have been granted to you and that have not vested as of the date of your termination of employment.

In particular, you understand and agree that the option will be forfeited without entitlement to the underlying shares of Common Stock or to any amount as indemnification in the event of your termination of employment prior to vesting by reason of, including, but not limited to: resignation, disciplinary dismissal adjudged to be with cause, disciplinary dismissal adjudged or recognized to be without good cause (i.e., subject to a "*despido improcedente*"), individual or collective layoff on objective grounds, adjudged or recognized to be with or without good cause, material modification of the terms of employment under Article 41 of the Workers' Statute, relocation under Article 40 of the Workers' Statute, Article 50 of the Workers' Statute, unilateral withdrawal by the Company, and under Article 10.3 of Royal Decree 1382/1985.

Furthermore, you understand that the Company has unilaterally, gratuitously and discretionally decided to grant the options under the Plan to employees of the Company. The decision is a limited decision that is entered into upon the express assumption and condition that any grant will not economically or otherwise

bind the Company on an ongoing basis. Consequently, you understand that the option is granted on the assumption and condition that the option and the shares of Common Stock underlying the option shall not become a part of any employment or service contract with the option and shall not be considered a mandatory benefit, salary for any purposes (including severance compensation) or any other right whatsoever. In addition, you understand that the option would not be granted to you but for the assumptions and conditions referred to above; thus, you acknowledge and freely accept that should any or all of the assumptions be mistaken or should any of the conditions not be met for any reason, then any option granted to you shall be null and void.

Securities Law Information. The option described in the Option Agreement does not qualify as a security under Spanish regulations. No "offer of securities to the public," within the meaning of Spanish law, has taken place or will take place in the Spanish territory. The Option Agreement and any other documents evidencing the option have not been, nor will they be, registered with the *Comisión Nacional del Mercado de Valores* (Spanish Securities Exchange Commission), and none of these documents constitutes a public offering prospectus.

Exchange Control Information. The acquisition, ownership and sale of shares of Common Stock under the Plan must be declared for statistical purposes to the Spanish *Dirección General de Comercio e Inversiones* (the "***DGCI***"), the Bureau for Commerce and Investments, which is a department of the Ministry of Economy and Competitiveness. You also must declare ownership of any shares of Common Stock as of December 31 of the prior year with the Directorate of Foreign Transactions each January. In addition, if the acquisition or sale of any shares of Common Stock exceeds certain thresholds, it must be declared to the DGCI within 1 month after the sale.

When receiving foreign currency payments derived from the ownership of shares of Common Stock (i.e., sale proceeds), you must inform the financial institution receiving the payment of the basis upon which such payment is made if the payment exceeds €50,000. You will need to provide the following information: (i) your name, address, and fiscal identification number; (ii) the name and corporate domicile of the Company; (iii) the amount of the payment and the currency used; (iv) the country of origin; (v) the reasons for the payment; and (vi) further information that may be required.

In addition, you may be required to declare electronically to the Bank of Spain any foreign accounts (including brokerage accounts held abroad), any foreign instruments (including any shares of Common Stock acquired under the Plan) and any transactions with non-Spanish residents (including any payments of shares of Common Stock made to you by the Company) depending on the value of such accounts and instruments and the amount of the transactions during the relevant year as of December 31 of the relevant year.

SWITZERLAND

Sole Contact and Contractual Partner Information. You acknowledge that the option, this Option Agreement including the Appendix and, the Annexes and your participation in the Plan do not create any claims against the employer, either directly or indirectly. Your sole contract and sole contractual partner regarding the Plan and the option is the Company and the option does not form part of your contractual compensation.

Continuous Service. Notwithstanding anything else in the Plan or the Option Agreement, the Continuous Service will be deemed to end on the date when a termination notice is issued, regardless of whether the cessation of the employment was lawful, and shall not include any period notice of termination of

employment or any period of salary continuance or deemed employment. As a result, if you receive notice of termination your Continuous Service will end on the date you receive such notice.

Securities Law Information. The option grant is not intended to be publicly offered in or from Switzerland. Because it is considered a private offering, it is not subject to securities registration in Switzerland. Neither this document nor any other materials relating to the option (i) constitutes a prospectus as such term is understood pursuant to article 35 of the Swiss Federal Act on Financial Services (FinSa)), (ii) may be publicly distributed nor otherwise made publicly available in Switzerland or (iii) has been or will be filed with, approved or supervised by any Swiss regulatory authority, including the Swiss Financial Market Supervisory Authority (FINMA).
Grant of the Option. The option is a voluntary gratuity (*Gratifikation; gratification*) within the meaning of Article 322d Swiss Code of Obligations (CO) as determined at the Company's sole discretion which you have no entitlement to and which does not constitute an entitlement for a grant of further options or other equities in the future.

Vesting. You acknowledge and confirm that the option grant is fully discretionary and that before any option has vested you shall not have any right in regard to such option.

Disability. For the avoidance of any doubt, "***Disability***" shall include, but not be limited to, any permanent disability as per the social security laws of Switzerland.

Social Security and Tax. You herewith directly authorize your employer to make all (if any) applicable social security, insurance and tax deductions resulting from the grant and/or vesting of the option or the sale of shares of Common Stock from any compensation owed to you by your employer, subject to any statutory limitations. If your compensation shall not be sufficient to cover such social security, insurance and tax liabilities, you will indemnify the employer upon first demand.

Cause. "***Cause***" shall include, but not be limited to, all reasons entitling to a summary dismissal pursuant to article 337 of the Swiss Code of Obligations (CO) and all justified reasons pursuant to article 340c para. 2 CO, without limiting the definition of Cause as outlined in the Option Agreement.

Language Acknowledgement. You confirm having read and understood the documents relating to the Plan, including the Option Agreement, with all terms and conditions included therein, which were provided in the English language only. You confirm that you have sufficient language capabilities to understand these terms and conditions in full.

Sie bestätigen, dass Sie den Plan sowie die dazugehörigen Dokumente, inklusive der Vereinbarung, mit all den darin enthaltenen Bedingungen und Voraussetzungen, welche in englischer Sprache verfasst sind, gelesen und verstanden haben. Sie bestätigen, dass Ihre Sprachkenntnisse genügend sind, um die Bedingungen und Voraussetzungen zu verstehen.

Vous confirmez que vous avez lu et compris les documents relatifs au plan, y compris la convention d'attribution, avec toutes les conditions qui y sont incluses, qui ont été fournies en langue anglaise uniquement. Vous confirmez que vous avez des capacités linguistiques suffisantes pour comprendre ces termes et conditions dans leur intégralité.

No Right against Employer. You expressly acknowledge that you shall not have any right or claim under this option grant or the Option Agreement against your employer. You expressly acknowledge and agree that you may only have any right and claim against the Company under this option grant and the Option Agreement.

Governing Law and Jurisdiction. You expressly acknowledge and agree to the Choice of Law clause in the Plan and the Option Agreement and accept that Swiss law does not apply and that Swiss courts do not have any jurisdiction in regard to any claims under the Plan or the Option Agreement.

UNITED KINGDOM

Option Not a Service Contract. The following supplements Section 9 of the Option Agreement:

You waive all rights to compensation or damages in consequence of the termination of your office or employment with the Company or any Affiliate for any reason whatsoever (whether lawful or unlawful and including, without prejudice to the foregoing, in circumstances giving rise to a claim for wrongful dismissal) in so far as those rights arise or may arise from you ceasing to hold or being able to vest your Option, or from the loss or diminution in value of any rights or entitlements in connection with the Plan.

Withholding Obligations. The following supplements Section 10 of the Option Agreement:

As a condition of the vesting of your Option, you unconditionally and irrevocably agree:

(i)to place the Company in funds and indemnify the Company in respect of (1) all liability to UK income tax which the Company is

contributions for which the Company is liable and which are formally transferred to you, which arises as a consequence of or in connection with the exercise of your Option (the "***UK Tax Liability***"); or

(ii)to permit the Company to sell at the best price which it can reasonably obtain such number of shares of Common Stock allocated

(iii)if so required by the Company, and, to the extent permitted by law, to enter into a joint election or other arrangements under

(iv)if so required by the Company, to enter into a joint election within Section 431 of (UK) Income Tax (Earnings and Pensions) Act 2003 ("***ITEPA***") in respect of computing any tax charge on the acquisition of "restricted securities" (as defined in Section 423 and 424 of

(v)to sign, promptly, all documents required by the Company to effect the terms of this provision, and references in this provision to

Clawback/Recovery. By executing the Option Agreement, you expressly consent in writing to the application of the right of recoupment to your option in accordance with the terms of Section 8(k) of the Plan and Section 13 of the Option Agreement.

ATTACHMENT II

2020 INDUCEMENT PLAN

ATTACHMENT III

NOTICE OF EXERCISE

MIRUM PHARMACEUTICALS, INC.

Date of Exercise:

This constitutes notice to Mirum Pharmaceuticals, Inc. (the "***Company***") under my stock option that I elect to purchase the below number of shares of Common Stock of the Company (the "***Shares***") for the price set forth below.

Type of option:	Nonstatutory
Stock option dated:	
Number of Shares as to which option is exercised:	
Certificates to be issued in name of:	
Total exercise price:	US$
Cash payment delivered herewith:	US$
[Value of _____ Shares delivered herewith[1]:	US$
[Value of _____ Shares pursuant to net exercise[2]:	US$
[Regulation T Program (cashless exercise[3]):	US$

[1] Shares must meet the public trading requirements set forth in the option. Shares must be valued in accordance with the terms of the option being exercised, and must be owned free and clear of any liens, claims, encumbrances or security interests. Certificates must be endorsed or accompanied by an executed assignment separate from certificate.

[2] The Company must have established net exercise procedures at the time of exercise, in order to utilize this payment method.

[3] Shares must meet the public trading requirements set forth in the option.

By this exercise, I agree (i) to provide such additional documents as you may require pursuant to the terms of the Mirum Pharmaceuticals, Inc. 2020 Inducement Plan, Stock Option Grant Notice, Option Agreement and the Appendix thereto and (ii) to provide for the payment by me to you (in the manner designated by you) of your withholding obligation, if any, relating to the exercise of this option.

Very truly yours,

Exhibit 10.15

International

MIRUM PHARMACEUTICALS, INC.

RESTRICTED STOCK UNIT GRANT NOTICE - INTERNATIONAL
(2020 INDUCEMENT PLAN)

Mirum Pharmaceuticals, Inc. (the "***Company***"), pursuant to its 2020 Inducement Plan (the "***Plan***"), hereby awards to Participant a Restricted Stock Unit Award for the number of shares of the Company's Common Stock ("***Restricted Stock Units***") set forth below (the "***Award***"). The Award is subject to all of the terms and conditions as set forth in this notice of grant (this "***Restricted Stock Unit Grant Notice***"), and in the Plan and the Restricted Stock Unit Award Agreement (the "***Award Agreement***") (the definition of which shall include any special terms and conditions for the Participant's country set out in the attached appendix (the "***Appendix***")), both of which are attached hereto and incorporated herein in their entirety. Capitalized terms not explicitly defined herein shall have the meanings set forth in the Plan or the Award Agreement. In the event of any conflict between the terms in this Restricted Stock Unit Grant Notice or the Award Agreement and the Plan, the terms of the Plan shall control.

Participant:
Date of Grant:
Vesting Commencement Date:
Number of Restricted Stock Units:

Vesting Schedule: [_____, subject to Participant's Continuous Service through each such vesting date.]

Issuance Schedule: Subject to any Capitalization Adjustment, one share of Common Stock (or its cash equivalent, at the discretion of the Company) will be issued for each Restricted Stock Unit that vests at the time set forth in Section 6 of the Award Agreement.

Additional Terms/Acknowledgements: Participant acknowledges receipt of, and understands and agrees to, this Restricted Stock Unit Grant Notice, the Award Agreement and the Plan. Participant further acknowledges that as of the Date of Grant, this Restricted Stock Unit Grant Notice, the Award Agreement and the Plan set forth the entire understanding between Participant and the Company regarding the acquisition of the Common Stock pursuant to the Award specified above and supersede all prior oral and written agreements on the terms of this Award, with the exception, if applicable, of (i) restricted stock unit awards or options previously granted and delivered to Participant, (ii) the written employment agreement, offer letter or other written agreement entered into between the Company and Participant specifying the terms that should govern this specific Award, and (iii) any compensation recovery policy that is adopted by the Company or is otherwise required by applicable law.

By accepting this Award, Participant acknowledges having received and read the Restricted Stock Unit Grant Notice, the Award Agreement and the Plan and agrees to all of the terms and conditions set forth in these documents. Participant consents to receive Plan documents by electronic delivery and to participate

in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

MIRUM PHARMACEUTICALS, INC.	**PARTICIPANT**
By:	
Signature	Signature
Title:	Date:
Date:	

ATTACHMENTS: Award Agreement (including the Appendix) and 2020 Inducement Plan

ATTACHMENT I

MIRUM PHARMACEUTICALS, INC.

2020 INDUCEMENT PLAN RESTRICTED STOCK UNIT AWARD AGREEMENT

Pursuant to the Restricted Stock Unit Grant Notice (the "***Grant Notice***") and this Restricted Stock Unit Award Agreement (the "***Agreement***", the definition of which shall include any special terms and conditions for your country set out in the attached appendix (the "***Appendix***")), Mirum Pharmaceuticals, Inc. (the "***Company***") has awarded you ("***Participant***") a Restricted Stock Unit Award (the "***Award***") pursuant to the Company's 2020 Inducement Plan (the "***Plan***") for the number of Restricted Stock Units/shares indicated in the Grant Notice. The Award is granted in compliance with Nasdaq Marketplace Rule 5635(c)(4) as a material inducement to you entering into employment with the Company. Capitalized terms not explicitly defined in this Agreement or the Grant Notice shall have the same meanings given to them in the Plan. The terms of your Award, in addition to those set forth in the Grant Notice, are as follows.

1.GRANT OF THE AWARD. This Award represents the right to be issued on a future date one (1) share of Common Stock for each

"***Account***") the number of Restricted Stock Units/shares of Common Stock subject to the Award. Notwithstanding the foregoing, the Company reserves the right to issue you the cash equivalent of Common Stock, in part or in full satisfaction of the delivery of Common Stock in connection with the vesting of the Restricted Stock Units, and, to the extent applicable, references in this Agreement and the Grant Notice to Common Stock issuable in connection with your Restricted Stock Units will include the potential issuance of its cash equivalent pursuant to such right. This Award was granted in consideration of your services to the Company.

2.VESTING. Subject to the limitations contained herein, your Award will vest, if at all, in accordance with the vesting schedule

3.NUMBER OF SHARES. The number of Restricted Stock Units subject to your Award may be adjusted from time to time for

covered by your Award. Notwithstanding the provisions of this Section 3, no fractional shares or rights for fractional shares of Common Stock shall be created pursuant to this Section 3. Any fraction of a share will be rounded down to the nearest whole share.

4.SECURITIES LAW COMPLIANCE. You may not be issued any Common Stock under your Award unless the shares of Common

Section 1.409A-1(b)(4), no later than the date that is the 15th day of the third calendar month of the applicable year following the year in which the shares of Common Stock under this Award are no longer subject to a "substantial risk of forfeiture" within the meaning of Treasury Regulations Section 1.409A1(d).

(c) The form of delivery (*e.g.*, a stock certificate or electronic entry evidencing such shares) shall be determined by the

7. DIVIDENDS. You shall receive no benefit or adjustment to your Award with respect to any cash dividend, stock dividend or other

8. RESTRICTIVE LEGENDS. The shares of Common Stock issued in respect of your Award shall be endorsed with appropriate

9. EXECUTION OF DOCUMENTS. You hereby acknowledge and agree that the manner selected by the Company by which you

executed in the future in connection with your Award.

10. AWARD NOT A SERVICE CONTRACT.

(a) Nothing in this Agreement (including, but not limited to, the vesting of your Award or the issuance of the shares in

compensation or any other term or condition of employment or affiliation; (iii) confer any right or benefit under this Agreement or the Plan unless such right or benefit has specifically accrued under the terms of this Agreement or Plan; or (iv) deprive the Company or any Affiliate of the right to terminate you in accordance with applicable laws and your employment or engagement agreement, if any, without regard to any future vesting opportunity that you may have.

(b) By accepting this Award, you acknowledge and agree that the right to continue vesting in the Award pursuant to the vesting schedule provided in the Grant Notice may not be earned unless (in addition to any other conditions described in the Grant Notice and this Agreement) you continue as an employee, director or consultant at the will of the Company and affiliate, as applicable (not through the act of being hired, being granted this Award or any other award or benefit) and that the Company has the right to reorganize, sell, spin-out or otherwise restructure one or more of its businesses or Affiliates at any time or from time to time, as it deems appropriate (a "***reorganization***"). You acknowledge and agree that such a reorganization could result in the termination of your Continuous Service, or the termination of Affiliate status of your employer and the loss of benefits available to you under this Agreement, including but not limited to, the termination of the right to continue vesting in the Award. You further acknowledge and agree that this Agreement, the Plan, the transactions contemplated hereunder and the vesting schedule set forth herein or any covenant of good faith and fair dealing that may be found implicit in any of them do not constitute an express or implied promise of continued engagement as an employee or consultant for the term of this Agreement, for any period, or at all, and shall not interfere in any way with the Company's

right to terminate your Continuous Service in accordance with applicable law and your employment or engagement agreement, if any, or to conduct a reorganization.

(c) By accepting your Award, you acknowledge, understand and agree that:

(i) the Plan is established voluntarily by the Company, it is discretionary in nature, and may be amended, suspended or terminated by the Company at any time, to the extent permitted under the Plan;

(ii) the grant of your Award is voluntary and occasional and does not create any contractual or other right to receive future grants of awards (whether on the same or different terms), or benefits in lieu of awards, even if awards have been granted in the past;

(iii) your Award and any shares of Common Stock acquired under the Plan, and the income and value of same, are not part of normal or expected compensation for any purpose, including, without limitation, calculating any severance, resignation, termination, redundancy, dismissal, end-of-service payments, bonuses, holiday pay, long-service awards, pension or retirement or welfare benefits or similar payments;

(iv) the future value of the shares of Common Stock underlying the Award is unknown, indeterminable, and cannot be predicted with certainty;

(v) neither the Company nor any Affiliate shall be liable for any foreign exchange rate fluctuation between your local currency and the United States Dollar that may affect the value of your Award or of any amounts due to you pursuant to the vesting of your Award or the subsequent sale of any shares of Common Stock received;

(vi) for the purposes of your Award, your Continuous Service will be considered terminated as of the date you are no longer actively providing services to the Company or one of its Affiliates (regardless of the reason for such termination and whether or not later found to be invalid or in breach of employment laws in the jurisdiction where you are employed or the terms of your employment agreement, if any), and unless otherwise expressly provided in this Agreement or determined by the Company, your right to vest in the Award under the Plan, if any, and the Board shall have the exclusive discretion to determine when you are no longer actively providing services for purposes of the Award (including whether you may still be considered to be providing services while on a leave of absence);

(vii) no claim or entitlement to compensation or damages shall arise from forfeiture of this Award resulting from the termination of your Continuous Service (for any reason whatsoever, whether or not later found to be invalid or in breach of employment laws in the jurisdiction where you are employed or the terms of your employment or service agreement, if any), and in consideration of the grant of this Award to which you are otherwise not entitled, you irrevocably agree never to institute any claim against the Company or any Affiliate, waive your ability, if any, to bring any such claim, and release the Company and any Affiliate from any such claim; if, notwithstanding the foregoing, any such claim is allowed by a court of competent jurisdiction, then, by participating in the Plan, you shall be deemed irrevocably to have agreed not to pursue such claim and agree to execute any and all documents necessary to request dismissal or withdrawal of such claim.

11. WITHHOLDING OBLIGATION.

(a) On each vesting date, and on or before the time you receive a distribution of the shares of Common Stock in respect of your Restricted Stock Units, and at any other time as reasonably requested by the Company in accordance with applicable tax and social security laws, you hereby authorize any required withholding from the Common Stock issuable to you and/or otherwise agree to make adequate provision, including in cash, for any sums required to satisfy the federal, state, local and foreign tax and social security withholding obligations of the Company or any Affiliate that arise in connection with your Award (the "***Withholding Obligation***").

(c) In the event the Withholding Obligation arises prior to the delivery to you of Common Stock or it is determined after the

12. TAX CONSEQUENCES. The Company has no duty or obligation to minimize the tax or social security consequences to you of this

understand that you (and not the Company or any Affiliate) shall be responsible for your own tax and social security liability that may arise as a result of this investment or the transactions contemplated by this Agreement.

13. UNSECURED OBLIGATION. Your Award is unfunded, and as a holder of a vested Award, you shall be considered an unsecured

rights as a stockholder of the Company with respect to the shares to be issued pursuant to this Agreement until such shares are issued to you

or a fiduciary relationship between you and the Company or any other person.

14.NOTICES. Any notice or request required or permitted hereunder shall be given in writing (including electronically) and will be

participate in the Plan by electronic means. By accepting this Award, you consent to receive such documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

15.HEADINGS. The headings of the Sections in this Agreement are inserted for convenience only and shall not be deemed to

16.MISCELLANEOUS.

(a) The rights and obligations of the Company under your Award shall be transferable by the Company to any one or more persons or entities, and all covenants and agreements hereunder shall inure to the benefit of, and be enforceable by, the Company's successors and assigns.

(b) You agree upon request to execute any further documents or instruments necessary or desirable in the sole determination of the Company to carry out the purposes or intent of your Award.

(c) You acknowledge and agree that you have reviewed your Award in its entirety, have had an opportunity to obtain the advice of counsel prior to executing and accepting your Award and fully understand all provisions of your Award.

(d) This Agreement shall be subject to all applicable laws, rules, and regulations, and to such approvals by any governmental agencies or national securities exchanges as may be required.

(e) All obligations of the Company under the Plan and this Agreement shall be binding on any successor to the Company, whether the existence of such successor is the result of a direct or indirect purchase, merger, consolidation, or otherwise, of all or substantially all of the business and/or assets of the Company.

17.GOVERNING PLAN DOCUMENT. Your Award is subject to all the provisions of the Plan, the provisions of which are hereby

thereunder, any clawback policy adopted by the Company and any compensation recovery policy otherwise required by applicable law. No recovery of compensation under

if and only if such delay in the issuance of the shares is necessary to avoid the imposition of adverse taxation on you in respect of the shares Regulation Section 1.409A-2(b)(2).

23.DATA PRIVACY.

(a) You explicitly and unambiguously acknowledge and consent to the collection, use and transfer, in electronic or other form, of your personal data as described in this document by and among, as applicable, your employer, the Company and its Affiliates for the exclusive purpose of implementing, administering and managing your participation in the Plan. You understand that the Company, its Affiliates and your employer hold certain personal information about you, including, but not limited to, name, home address and telephone number, date of birth, social security number (or other identification number), salary, nationality, job title, any shares of stock or directorships held in the Company, details of all options or any other entitlement to shares of stock awarded, canceled, purchased, exercised, vested, unvested or outstanding in your favor for the purpose of implementing, managing and administering the Plan ("***Data***"). You understand that the Data may be transferred to any third parties assisting in the implementation, administration and management of the Plan, that these recipients may be located in your country or elsewhere, in particular in the US, and that the recipient country may have different data privacy laws providing less protections of your personal data than your country. You may request a list with the names and addresses of any potential recipients of the Data by contacting the stock plan administrator at the Company (the "***Stock Plan Administrator***"). You acknowledge that the recipients may receive, possess, process, use, retain and transfer the Data, in electronic or other form, for the purposes of implementing, administering and managing your participation in the Plan, including any requisite transfer of such Data, as may be required to a broker or other third party with whom you may elect to deposit any shares of Common Stock acquired upon the vesting of your Award. You understand that Data will be held only as long as is necessary to implement, administer and manage your participation in the Plan. You may, at any time, view the Data, request additional information about the storage and processing of the Data, require any necessary amendments to the Data or refuse or withdraw the consents herein, in any case without cost, by contacting the Stock Plan Administrator in writing.

(b) For the purposes of operating the Plan in the European Union, Switzerland and the United Kingdom, the Company will collect and process information relating to you in accordance with the privacy notice from time to time in force.

24.NO ADVICE REGARDING GRANT. The Company is not providing any tax, legal or financial advice, nor is the Company making

25.LANGUAGE. You acknowledge that you are sufficiently proficient in the English language, or have consulted with an advisor

26.INSIDER TRADING/MARKET ABUSE. You acknowledge that, depending on your country, you may be subject to insider trading

during such times as you are considered to have "inside information" regarding the Company (as defined by the laws in your country). Any

advised to speak to your personal advisor on this matter.

27.FOREIGN ASSET/ACCOUNT, EXCHANGE CONTROL AND TAX REPORTING. You may be subject to foreign asset/account,

report such accounts, assets and balances therein, the value thereof and/or the transactions related thereto to the applicable authorities in such country. You may also be required to repatriate sale proceeds or other funds received as a result of your participation in the Plan to your country through a designated bank or broker within a certain time after receipt. You acknowledge that it is your responsibility to be compliant with such regulations and you are encouraged to consult with your personal legal advisor for any details.

28.CHOICE OF LAW. The interpretation, performance and enforcement of this Agreement shall be governed by the laws of the State

29.APPENDIX. Notwithstanding any provisions in this Agreement, your Award shall be subject to the special terms and conditions

* * * * *

This Restricted Stock Unit Award Agreement shall be deemed to be signed by the Company and the Participant upon the signing by the Participant of the Restricted Stock Unit Grant Notice to which it is attached.

APPENDIX

This Appendix includes special terms and conditions that govern the Award granted to you under the Plan if you reside and/or work in any country listed below.

The information contained herein is general in nature and may not apply to your particular situation, and you are advised to seek appropriate professional advice as to how the relevant laws in your country may apply to your situation. If you are a citizen or resident of a country other than the one in which you are currently working and/or residing, transfer employment and/or residency to another country after the date of grant, are a consultant, change employment status to a consultant position, or are considered a resident of another country for local law purposes, the Company shall, in its discretion, determine the extent to which the special terms and conditions contained herein shall be applicable to you. References to your employer shall include any entity that engages your services.

DENMARK

Stock Option Act. You acknowledge that you received the Employer Statement in Danish which sets forth additional terms of the Award to the extent the Danish Stock Options Act applies.

Foreign Asset / Account Reporting. If you establish an account holding cash or shares of Common Stock outside Denmark, you must report the account to the Danish Tax Administration.

GERMANY

Securities Disclaimer. Participation in the Plan is exempt or excluded from the requirement to publish a prospectus under the EU Prospectus Directive as implemented in Germany.

Exchange Control Information. Cross-border payments in excess of EUR €12,500 must be reported monthly to the German Federal Bank (*Bundesbank*). In the event that you make or receive a payment in excess of this amount, you are required to report the payment to Bundesbank electronically using the "General Statistics Reporting Portal" ("*Allgemeines Meldeportal Statistik*") available via Bundesbank's website (www.bundesbank.de).

Tax Reporting. You must report and pay any capital gains tax liability that arises in connection with the sale of shares of Common Stock acquired under the Plan. In general the statutory deadline of filing annual income tax returns for taxpayers is July 31 of the calendar year following the respective fiscal year. Payment periods of due tax amounts are determined in view of the competent tax office. You should consult with your personal tax advisor to ensure that you are properly complying with applicable reporting requirements in Germany.

ITALY

Plan Document Acknowledgement. By accepting the Award, you acknowledge that you have received a copy of the Plan, have reviewed the Plan and the Agreement in their entirety and fully understand and accept all provisions of the Plan and the Agreement.

You acknowledge that you have read and specifically and expressly approve the following sections of the Agreement and this Appendix, including: (10) Award Not a Service Contract; (11) Withholding Obligations; 12 (Tax Consequences); (23) Data Privacy; (24) Language; and (28) Choice of Law.

Foreign Asset/Account Reporting Information. If you are an Italian resident and, during any fiscal year, hold investments or financial assets outside of Italy (*e.g.*, cash, shares of Common Stock) which may generate income taxable in Italy (or if you are the beneficial owner of such an investment or asset even if you do not directly hold the investment or asset), you are required to report such investments or assets on your annual tax return for such fiscal year (on UNICO Form, RW Schedule, or on a special form if you are not required to file a tax return).

Foreign Financial Assets Tax. The fair market value of any shares of Common Stock held outside of Italy is subject to a foreign assets tax. Financial assets include shares of Common Stock acquired under the Plan. The taxable amount will be the fair market value of the financial assets assessed at the end of the calendar year. *You should consult with your personal tax advisor about the foreign financial assets tax.*

NETHERLANDS

Prohibition Against Insider Trading. You should be aware of the European insider trading rules, which may affect the sale of shares of Common Stock acquired under the Plan. In particular, you may be prohibited from effecting certain share transactions if you have insider information regarding the Company. If it is uncertain whether the insider rules apply, the Company recommends that you consult with a legal advisor. The Company cannot be held liable if you violate the insider trading rules applicable in the Netherlands. You are responsible for ensuring your compliance with these rules.

Under Article 14 of the Market Abuse Regulation ((EU) Regulation 596/2014), anyone who has "inside information" related to an issuing company is prohibited from effectuating a transaction in securities in or from the Netherlands. "Inside information" is defined as knowledge of specific information concerning the issuing company to which the securities relate or the trade in securities issued by such company, which has not been made public and which, if published, would reasonably be expected to affect the price of the securities, regardless of the development of the price. The insider could be any employee of the Company or an Affiliate in the Netherlands who has inside information as described herein.

Given the broad scope of the definition of inside information, certain participants may have inside information and thus are prohibited from making a transaction in securities in the Netherlands at a time when they have such inside information. By entering into this Agreement and participating in the Plan, you acknowledge having read and understood the notification above and acknowledge that it is your responsibility to comply with the Dutch insider trading rules, as discussed herein.

Securities Disclaimer. The grant of the Award is exempt or excluded from the requirement to publish a prospectus under the Prospectus Regulation ((EU) Regulation 2017/1129). The Awards are not transferable and are not deemed to qualify as an offering of securities in the Netherlands within the meaning of the Prospectus Regulation. To the extent that a supervisory body would qualify the offering of Awards or its underlying securities as an offering of securities within the meaning of the Prospectus Regulation, such offering will only be made in reliance on Article 1(4) of the Prospectus Regulation provided that no such offering of securities shall require Mirum Pharmaceuticals, Inc. to publish a prospectus pursuant to Article 3 of the Prospectus Regulation.

SPAIN

No Entitlement for Claims or Compensation. The following provision supplements Section 10 of the Agreement that clarifies that the grant, vesting, or settlement of Award does not give you a right to continued service/employment:

By accepting the grant of Award, you consent to participation in the Plan and acknowledge that you have received a copy of the Plan document.

You understand that the Company has unilaterally, gratuitously and in its sole discretion decided to make grants of Awards under the Plan to individuals who may be Consultants, Directors, and Employees throughout the world. The decision is limited and entered into based upon the express assumption and condition that any Awards will not economically or otherwise bind the Company or any Affiliate, including the employer, on an ongoing basis, other than as expressly set forth in the Agreement. Consequently, you understand that the grant of Awards is made on the assumption and condition that the Awards shall not become part of any employment contract (whether with the Company or any Affiliate, including the employer) and shall not be considered a mandatory benefit, salary for any purpose (including severance compensation) or any other right whatsoever. Furthermore, you understand and freely accept that there is no guarantee that any benefit whatsoever shall arise from the Awards, which is gratuitous and discretionary, since the future value of the Awards and the underlying shares of Common Stock is unknown and unpredictable.

You understand and agree that, as a condition of the grant of the Awards, your termination of Continuous Service for any reason (including for the reasons listed below) will automatically result in the cancellation and loss of any Awards that may have been granted to you and that were not fully vested on the date of termination of your Continuous Service. In particular, you understand and agree that, unless otherwise expressly provided for by the Company at the Date of Grant, the Awards will be cancelled without entitlement to the shares of Common Stock or to any amount as indemnification if you terminate employment by reason of, including, but not limited to: resignation, death, disability, retirement, disciplinary dismissal adjudged to be with cause, disciplinary dismissal adjudged or recognized to be without cause, individual or collective layoff on objective grounds, whether adjudged to be with cause or adjudged or recognized to be without cause, material modification of the terms of employment under Article 41 of the Workers' Statute, relocation under Article 40 of the Workers' Statute, Article 50 of the Workers' Statute, unilateral withdrawal by the employer, and under Article 10.3 of Royal Decree 1382/1985.

You also understand that this grant of Awards would not be made but for the assumptions and conditions set forth hereinabove; thus, you understand, acknowledge and freely accept that, should any or all of the assumptions be mistaken or any of the conditions not be met for any reason, the grant, the Awards and any right to the underlying shares of Common Stock shall be null and void.

Securities Law Information. The Award described in the Agreement and this Appendix do not qualify under Spanish regulations as securities. No "offer of securities to the public", as defined under Spanish law, has taken place or will take place in the Spanish territory. The Agreement (including this Appendix) has not been nor will it be registered with the *Comisión Nacional del Mercado de Valores*, and does not constitute a public offering prospectus.

Foreign Assets Reporting. You may be subject to certain tax reporting requirements with respect to assets or rights that you hold outside of Spain, including bank accounts, securities and real estate if the aggregate value for particular category of assets exceeds EUR €50,000 as of December 31 each year. Shares of

Common Stock acquired under the Plan or other equity programs offered by the Company constitute securities for purposes of this requirement, but unvested awards (e.g., RSU Awards, etc.) are not considered assets or rights for purposes of this reporting requirement.

If applicable, you must report the assets on Form 720 by no later than March 31 following the end of the relevant year. After the rights and/or assets are initially reported, the reporting obligation will only apply if: (i) the value of previously-reported rights or assets increases by more than EUR €20,000 as of each subsequent December 31, or (ii) upon disposition of the previously-reported rights or assets. You are encouraged to consult with your personal advisor to determine any obligations in this respect.

Share Reporting Requirement. You must declare the acquisition, ownership and disposition of shares of Common Stock to the Spanish *Dirección General de Comercio e Inversiones* (the "***DGCI***") of the Ministry of Economy and Competitiveness on a Form D-6. Generally, the declaration must be made in January for shares of Common Stock owned as of December 31 of the prior year and/or shares of Common Stock acquired or disposed of during the prior year; however, if the value of the shares of Common Stock acquired or the amount of the sale proceeds exceeds EUR €1,502,530 (or you hold 10% or more of the share capital of the Company or other such amount that would entitle you to join the Board), the declaration must be filed within one month of the acquisition or disposition, as applicable. You should consult with your personal advisor to determine your obligations in this respect.

Foreign Assets and Transaction Reporting. You may be required to electronically declare to the Bank of Spain any foreign accounts (including brokerage accounts held abroad), any foreign instruments (*e.g.*, shares of Common Stock) and any transactions with non-Spanish residents (including any payments of cash or shares made to you by the Company or a U.S. brokerage account) if the balances in such accounts together with the value of such instruments as of December 31, or the volume of transactions with nonSpanish residents during the prior or current year, exceed EUR €1,000,000. Once the EUR €1,000,000 threshold has been surpassed in either respect, you will generally be required to report all of your foreign accounts, foreign instruments and transactions with non-Spanish residents, even if the relevant threshold has not been crossed for an individual item. You will generally only be required to report on an annual basis (by January 20 of each year); however, if the balances in your foreign accounts together with value of your foreign instruments or the volume of transactions with non-Spanish residents exceed EUR €100,000,000, you acknowledge that more frequent reporting will be required.

SWITZERLAND

Sole Contact and Contractual Partner Information. You acknowledge that the Award, this Agreement including the Appendix and, the Annexes and your participation in the Plan do not create any claims against the employer, either directly or indirectly. Your sole contract and sole contractual partner regarding the Plan and the Award is the Company and the Award does not form part of your contractual compensation.

Continuous Service. Notwithstanding anything else in the Plan or the Agreement, the Continuous Service will be deemed to end on the date when a termination notice is issued, regardless of whether the cessation of the employment was lawful, and shall not include any period notice of termination of employment or any period of salary continuance or deemed employment. As a result, if you receive notice of termination your Continuous Service will end on the date you receive such notice.

Securities Law Information. The Award is not intended to be publicly offered in or from Switzerland. Because it is considered a private offering, it is not subject to securities registration in Switzerland. Neither this document nor any other materials relating to the Restricted Stock Units and/or the underlying shares of

Common Stock: (i) constitutes a prospectus according to articles 35 et seq. of the Swiss Federal Act on Financial Services ("***FinSA***"); (ii) may be publicly distributed or otherwise made publicly available in Switzerland to any person other than a Participant; or (iii) has been or will be filed with, approved or supervised by any Swiss reviewing body according to article 51 FinSA or any Swiss regulatory authority, including the Swiss Financial Market Supervisory Authority ("***FINMA***").

Grant of the Award. The Award is a voluntary gratuity (*Gratifikation; gratification*) within the meaning of Article 322d Swiss Code of Obligations (CO) as determined at the Company's sole discretion which you have no entitlement to and which does not constitute an entitlement for a grant of further Awards or other equities in the future.

Vesting. You acknowledge and confirm that the Award is fully discretionary and that before the Restricted Stock Units have vested you shall not have any right in regard to such Restricted Stock Units.

Disability. For the avoidance of any doubt, "***Disability***" shall include, but not be limited to, any permanent disability as per the social security laws of Switzerland.

Social Security and Tax. You herewith directly authorize your employer to make all (if any) applicable social security, insurance and tax deductions resulting from the grant and/or vesting of the Award or the sale of shares of Common Stock from any compensation owed to you by your employer, subject to any statutory limitations. If your compensation shall not be sufficient to cover such social security, insurance and tax liabilities, you will indemnify the employer upon first demand.

Cause. "***Cause***" shall include, but not be limited to, all reasons entitling to a summary dismissal pursuant to article 337 of the Swiss Code of Obligations (CO) and all justified reasons pursuant to article 340c para. 2 CO, without limiting the definition of Cause as outlined in the Plan. You expressly acknowledge that the definition of Cause as per the Plan shall include any crime or felony under Swiss laws and any breaches against your duties and in respect of the employer, and not only in respect of the Company.

Language Acknowledgement. You confirm that you have read and understood the documents relating to the Plan, including the Agreement, with all terms and conditions included therein, which were provided in the English language only. You confirm that you have sufficient language capabilities to understand these terms and conditions in full.

Sie bestätigen, dass Sie den Plan sowie die dazugehörigen Dokumente, inklusive der Vereinbarung, mit all den darin enthaltenen Bedingungen und Voraussetzungen, welche in englischer Sprache verfasst sind, gelesen und verstanden haben. Sie bestätigen, dass Ihre Sprachkenntnisse genügend sind, um die Bedingungen und Voraussetzungen zu verstehen.

Vous confirmez que vous avez lu et compris les documents relatifs au plan, y compris la convention d'attribution, avec toutes les conditions qui y sont incluses, qui ont été fournies en langue anglaise uniquement. Vous confirmez que vous avez des capacités linguistiques suffisantes pour comprendre ces termes et conditions dans leur intégralité.

No Right against Employer. You expressly acknowledge that you shall not have any right or claim under the Restricted Stock Units, the Award, the Plan or the Agreement against your employer. You expressly acknowledge and agree that you only have any right and claim against the Company as set out under the Plan and the Agreement.

Governing Law and Jurisdiction. You expressly acknowledge and agree to the Choice of Law clause in the Plan and the Agreement and accept that Swiss law does not apply and that Swiss courts do not have any jurisdiction in regard to any claims under the Plan or the Agreement.

UNITED KINGDOM

No Cash Settlement. Notwithstanding any provision of the Plan or the Agreement, the Award may not be settled in cash.

Award Not a Service Contract. The following supplements Section 10 of the Agreement:

You waive all rights to compensation or damages in consequence of the termination of your office or employment with the Company or any Affiliate for any reason whatsoever (whether lawful or unlawful and including, without prejudice to the foregoing, in circumstances giving rise to a claim for wrongful dismissal) in so far as those rights arise or may arise from you ceasing to hold or being able to vest your Award, or from the loss or diminution in value of any rights or entitlements in connection with the Plan.

Withholding Obligations. The following supplements Section 11 of the Agreement:

As a condition of the vesting of your Award, you unconditionally and irrevocably agree:

(i) to place the Company in funds and indemnify the Company in respect of: (1) all liability to UK income tax which the Company is liable to account for on your behalf directly to HM Revenue & Customs; (2) all liability to national insurance contributions which the Company is liable to account for on your behalf to HM Revenue & Customs (including, to the extent permitted by law, secondary class 1 (employer's) national insurance contributions for which you are liable and hereby agree to bear); and (3) all liability to national insurance contributions for which the Company is liable and which are formally transferred to you, which arises as a consequence of or in connection with your Award (the "***UK Tax Liability***"); or

(ii) to permit the Company to sell at the best price which it can reasonably obtain such number of shares of Common Stock allocated or allotted to you following vesting as will provide the Company with an amount equal to the UK Tax Liability; and to permit the Company to withhold an amount not exceeding the UK Tax Liability from any payment made to you (including, but not limited to salary); and

(iii) if so required by the Company, and, to the extent permitted by law, to enter into a joint election or other arrangements under which the liability for all or part of such employer's national insurance contributions liability is transferred to you; and

(iv) if so required by the Company, to enter into a joint election within Section 431 of (UK) Income Tax (Earnings and Pensions) Act 2003 ("***ITEPA***") in respect of computing any tax charge on the acquisition of "restricted securities" (as defined in Section 423 and 424 of ITEPA); and

(v) to sign, promptly, all documents required by the Company to effect the terms of this provision, and references in this provision to "the Company" shall, if applicable, be construed as also referring to any Affiliate.

Clawback/Recovery. By executing the Agreement, you expressly consent in writing to the application of the right of recoupment to your Award in accordance with the terms of Section 8(l) of the Plan and Section 17 of the Agreement.

ATTACHMENT II

2020 INDUCEMENT PLAN

Exhibit 10.35

MIRUM PHARMACEUTICALS, INC.

NON-EMPLOYEE DIRECTOR COMPENSATION POLICY

Each member of the Board of Directors (the "***Board***") who is not also serving as an employee of or consultant to Mirum Pharmaceuticals, Inc. (the "***Company***") or any of its subsidiaries (each such member, an "***Eligible Director***") will receive the compensation described in this Non-Employee Director Compensation Policy for his or her Board service upon and following the date of the underwriting agreement between the Company and the underwriters managing the initial public offering of the Company's common stock (the "***Common Stock***"), pursuant to which the Common Stock is priced in such initial public offering (the "***Effective Date***"). An Eligible Director may decline all or any portion of his or her compensation by giving notice to the Company prior to the date cash may be paid or equity awards are to be granted, as the case may be. This policy is effective as of the Effective Date and may be amended at any time in the sole discretion of the Board.

Annual Cash Compensation

The annual cash compensation amount set forth below is payable to Eligible Directors in equal quarterly installments, payable in arrears on the last day of each fiscal quarter in which the service occurred. If an Eligible Director joins the Board or a committee of the Board at a time other than effective as of the first day of a fiscal quarter, each annual retainer set forth below will be pro-rated based on days served in the applicable fiscal year, with the pro-rated amount paid for the first fiscal quarter in which the Eligible Director provides the service and regular full quarterly payments thereafter. All annual cash fees are vested upon payment.

1. Annual Board Service Retainer:
    a. All Eligible Directors: $40,000
    b. Chairman of the Board Service Retainer (in addition to Eligible Director Service Retainer): $60,000
    c. Lead Independent Director Service Retainer (in addition to Eligible Director Service Retainer), if applicable: $10,000

2. Annual Committee Chair Service Retainer (in addition to Committee Member Service Retainer):
    a. Chair of the Audit Committee: $10,000
    b. Chair of the Compensation Committee: $7,500
    c. Chair of the Nominating and Corporate Governance Committee: $4,000

3. Annual Committee Member Service Retainer:
    a. Member of the Audit Committee: $10,000
    b. Member of the Compensation Committee: $7,500
    c. Member of the Nominating and Corporate Governance Committee: $4,000

Equity Compensation

The equity compensation set forth below will be granted under the Company's 2019 Equity Incentive Plan (the "***Plan***"), subject to the approval of the Plan by the Company's stockholders. All stock options granted under this policy will be nonstatutory stock options, with an exercise price per share equal to 100% of the Fair Market Value (as defined in the Plan) of the underlying Common Stock on the date of grant, and a term of ten years from the date of grant (subject to earlier termination in connection with a termination of service as provided in the Plan, provided that upon a termination of service other than for death, disability or cause, the post-termination exercise period will be three months from the date of termination).

1. Initial Grant: For each Eligible Director who is first elected or appointed to the Board following the Effective Date, on the date of such Eligible Director's initial election or appointment to the Board (or, if such date is not a market trading day, the first market trading day thereafter), the Eligible Director will be automatically, and without further action by the Board, granted a stock option to purchase 34,000 shares of Common Stock (the "***Initial Grant***"). Each person who, after the Effective Date, served as an employee of, or consultant to, the Company and as a member of the Board, but who later ceases to provide such employment or consulting services shall not be entitled to an Initial Grant. The shares subject to each Initial Grant will vest in equal annual installments over a three year period such that the option is fully vested on the third anniversary of the date of grant, subject to the Eligible Director's Continuous Service (as defined in the Plan) through each such vesting date and will vest in full upon a Change in Control (as defined in the Plan).

2. Annual Grant: On the date of each annual stockholder meeting of the Company held after the Effective Date, each Eligible Director who continues to serve as a non-employee member of the Board following such stockholder meeting will be automatically, and without further action by the Board, granted a stock option to purchase 17,000 shares of Common Stock (the "***Annual Grant***"); *provided, however*, that if the Eligible Director has not served as member of the Board for 12 months prior to the applicable annual stockholder meeting, the number of shares subject to such individual's Annual Grant will be pro-rated based on the number of full months served on the Board, rounded to the nearest whole share. The shares subject to the Annual Grant will vest on the first anniversary of the date of grant, provided that the Annual Grant will in any case be fully vested on the date of Company's next annual stockholder meeting, subject to the Eligible Director's Continuous Service (as defined in the Plan) through such vesting date and will vest in full upon a Change in Control (as defined in the Plan).

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the following Registration Statements:

(1)Registration Statement (Form S-3 No. 333-240290) of Mirum Pharmaceuticals, Inc.;
(2)Registration Statement (Form S-8 No. 333-233502) pertaining to the 2018 Equity Incentive Plan, 2019 Equity Incentive Plan, and 2019 Employee Stock Purchase Plan of Mirum Pharmaceuticals, Inc.; and
(3)Registration Statement (Form S-8 No. 333-238086) pertaining to the 2019 Equity Incentive Plan, 2019 Employee Stock Purchase Plan, and 2020 Inducement Plan of Mirum Pharmaceuticals, Inc.

of our report dated March 9, 2022, with respect to the consolidated financial statements of Mirum Pharmaceuticals, Inc. included in this Annual Report (Form 10-K) for the year ended December 31, 2021.

/s/ ERNST & YOUNG LLP

Irvine, California
March 9, 2022

Exhibit 31.1

CERTIFICATION 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

I, Christopher Peetz, certify that:

1.I have reviewed this Annual Report on Form 10-K of Mirum Pharmaceuticals, Inc.;

2.Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3.Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

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

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

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

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

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

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

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

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

Date: March 9, 2022

By: /s/ Christopher Peetz
Christopher Peetz
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION 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

I, Ian Clements, Ph.D., certify that:

1.I have reviewed this Annual Report on Form 10-K of Mirum Pharmaceuticals, Inc.;

2.Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3.Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

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

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

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

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

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

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

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

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

Date: March 9, 2022

By: /s/ Ian Clements, Ph.D.
Ian Clements, Ph.D.
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report of Mirum Pharmaceuticals, Inc. (the "Company") on Form 10-K for the year ended December 31, 2021 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

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

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

Date: March 9, 2022

By: /s/ Christopher Peetz
Christopher Peetz
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report of Mirum Pharmaceuticals, Inc. (the "Company") on Form 10-K for the year ended December 31, 2021 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that:

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

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

Date: March 9, 2022

By: /s/ Ian Clements, Ph.D.
Ian Clements, Ph.D.
Chief Financial Officer
(Principal Financial and Accounting Officer)